KORRO BIO

2023 Annual Report

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2023

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 FOR THE TRANSITION PERIOD FROM TO**

Commission file number 001-39062

Korro Bio, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**47-2324450**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
One Kendall Square, Building 600-700, Suite 6-401 Cambridge, MA	**02139**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 468-1999

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, par value $0.001 per share	KRRO	The Nasdaq Capital Market

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive o□cers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant, based on the closing price of the shares of common stock on The Nasdaq Capital Market on June 30, 2023, was $12,148,224.

The number of shares of registrant's Common Stock outstanding as of March 21, 2024 was 8,021,946.

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On November 3, 2023, or the Closing Date, we consummated the previously announced business combination, or the Merger, pursuant to that certain Agreement and Plan of Merger, or the Merger Agreement, dated July 14, 2023, by and among our company (formerly known as Frequency Therapeutics, Inc., or Frequency), Frequency Merger Sub, Inc., or Merger Sub, and Korro Bio Inc., or Legacy Korro.

Pursuant to the terms of the Merger Agreement, a business combination between Frequency and Legacy Korro was effected through the merger of Merger Sub with and into Legacy Korro, with Legacy Korro surviving as a wholly owned subsidiary of Frequency. On the Closing Date, Frequency changed its name to Korro Bio, Inc.

Unless the context otherwise indicates, references in this Annual Report on 10-K to the "Company," "we," "our" and "us" refer, collectively, to Korro Bio, Inc., a Delaware corporation, and its consolidated subsidiaries (including Legacy Korro).

We use various trademarks and trade names in our business, including without limitation our corporate name and logo. All other trademarks or trade names referred to in this Annual Report on Form 10-K are the property of their respective owners. Solely for convenience, the trademarks and trade names in this Annual Report on Form 10-K may be referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K includes statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Our forward-looking statements include, but are not limited to, statements regarding our or our management team's expectations, hopes, beliefs, intentions or strategies regarding the future. In addition, any statements that refer to projections, forecasts or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking statements. The words "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intends," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "will," "would" and similar expressions may identify forward-looking statements, but the absence of these words does not mean that a statement is not forward-looking. Forward-looking statements in this Annual Report on Form 10-K may include, for example, statements about:

- our ability to recognize the benefits of the Merger;
- the initiation, timing, progress, results, and cost of our research and development programs and our current and future preclinical studies and clinical trials, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available, and our research and development programs;
- our strategy;
- our cash runway and ability to reach data inflection points;
- the therapeutic and commercial potential of our product candidates;
- our research and development and other expenses;
- our ability to comply with, and the impact of, regulatory requirements, obligations and restrictions on our business and operations;
- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others, including our ability to obtain and maintain rights to the technologies required to develop and commercialize our product candidates;
- competitive developments, including the impact on our competitive position of rival products and product candidates and our ability to meet such competition; and
- our ability to manage the growth of our business.

These forward-looking statements are subject to known and unknown risks, uncertainties and assumptions about us that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by such forward-looking statements, including those set forth under the heading "*Risk Factors*" in Part I, Item 1A of this Annual Report on Form 10-K. Given these risks and uncertainties, you should not place undue reliance on these forward-looking statements. Should one or more of the risks or uncertainties described in this Annual Report on Form 10-K, or should underlying assumptions prove incorrect, actual results and plans could differ materially from those expressed in any forward-looking statements. Except as otherwise required by applicable law, we disclaim any duty to update any forward-looking statements, all of which are expressly qualified by the statements in this section, to reflect events or circumstances after the date of this annual report. We qualify all of our forward-looking statements by these cautionary statements.

SUMMARY OF RISK FACTORS

Our business involves significant risks. Below is a summary of the material risks that our business faces, which makes an investment in our securities speculative and risky. This summary does not address all these risks. These risks are more fully described below under the heading "*Risk Factors*" in Part I, Item 1A of this Annual Report on Form 10-K. Before making investment decisions regarding our securities, you should carefully consider these risks. The occurrence of any of the events or developments described below could have a material adverse effect on our business, results of operations, financial condition, prospects and stock price. In such event, the market price of our securities could decline, and you could lose all or part of your investment. Further, there are additional risks not described below that are either not currently known to us or that we currently deem immaterial, and these additional risks could also materially impair our business, operations or market price of our securities.

- We have incurred significant losses since inception and expect to incur losses for the foreseeable future and may never achieve or maintain profitability.
- Expectations regarding our cash runway and ability to reach data inflection points are based on numerous assumptions that may prove to be untrue.
- We may be required to raise capital sooner than anticipated and our exposure to certain contingent liabilities and contractual obligations may be greater than anticipated.
- We have never generated revenue from product sales and may never become profitable.
- We will need substantial additional funding. If we are unable to raise capital when needed, we will be forced to delay, reduce, eliminate or prioritize among our research and development programs or future commercialization efforts.
- The gene editing field and RNA editing in particular is relatively new and is evolving rapidly. We are very early in our research and development efforts and may not be successful in identifying and developing product candidates. It will be many years before we or any potential future collaborators commercialize a product candidate or generate any revenues, if ever. Additionally, other gene editing technologies may be discovered that provide significant advantages over RNA editing, which could materially harm our business.
- RNA editing is a novel technology that is not yet clinically validated for human therapeutic use. The approaches we take to discover and develop novel therapeutics are unproven and may never lead to marketable products.
- We are very early in our research and development efforts, and our preclinical studies and clinical trials may not be successful. If we are unable to commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.
- Any product candidates we develop may fail in preclinical or clinical development or be delayed to a point where they do not become commercially viable.
- If we are not able to obtain or protect intellectual property rights related to any of our product candidates, development and commercialization of our product candidates may be adversely affected.
- The market price of our common stock is expected to be volatile, the market price of the common stock may drop following the Merger and an active trading market for our common stock may not develop and our stockholders may not be able to resell their shares of common stock for a profit, if at all.
- Provisions in our charter documents and under Delaware law could make an acquisition of our company more difficult and may discourage any takeover attempts that stockholders may consider favorable, and may lead to entrenchment of management.
- Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.

PART I

Item 1. Business.

Overview

We are a biopharmaceutical company with a mission to discover, develop and commercialize a new class of genetic medicines based on editing RNA, enabling the treatment of both rare and highly prevalent diseases.

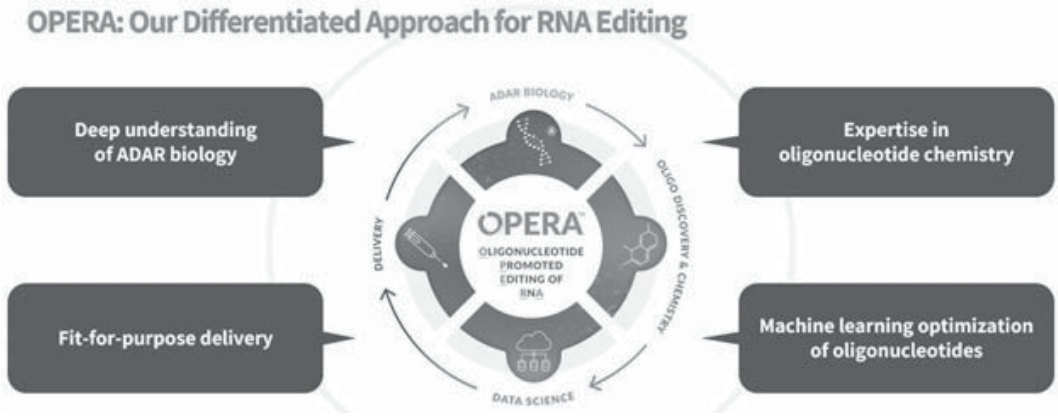
We are generating a portfolio of differentiated programs that are designed to harness the body's natural RNA editing process to effect a precise yet transient single base edit. By editing RNA instead of DNA, we are expanding the reach of genetic medicines by delivering additional precision and tunability, which has the potential for increased specificity and improved long-term tolerability. Using an oligonucleotide-based approach, we expect to bring our medicines to patients by leveraging our proprietary platform with precedented delivery modalities, manufacturing know-how, and established regulatory pathways of approved oligonucleotide drugs. However, the scientific evidence to support the feasibility of developing product candidates using our RNA editing technology is both preliminary and limited. Moreover, regulators have not yet established any definitive guidelines related to overall development considerations for RNA editing therapies and no clinical data has been generated to date.

The advent of large-scale genome sequencing has progressively revealed causal genetic variation underlying several human diseases, both rare and highly prevalent. Genetic mutations, including single nucleotide variants, or SNVs, implicated in disease have been found to be diverse in nature and can affect the function of genes and their associated downstream biochemical pathways. Data correlating DNA to RNA to disease phenotype have demonstrated that SNVs lead to a loss-of-function or a gain-of-function of the gene. In addition, the majority of SNVs implicated in complex diseases are due to modulation of gene function. By editing SNVs on RNA, we believe we will be able to address unmet patient need by transiently modifying gene function.

As our understanding of genetic drivers of disease has increased, significant advances have been made in technologies designed to introduce specific yet permanent changes at the DNA level to treat diseases. While these DNA editing approaches offer great promise for the treatment of certain rare diseases, they present significant risks from potential permanent adverse "off-target" edits. Additionally, the complex nature of DNA editing drug products presents multiple challenges including lack of efficient delivery to target cells and scalable manufacturing, impeding their application to treat complex highly prevalent diseases of larger patient populations. These potential limitations have spurred exploration of alternative approaches to genetic medicine development, such as RNA editing.

Mammals and other lower species like cephalopods have an endogenous process of modifying single bases on RNA, referred to as RNA editing. RNA editing is a natural physiological process, similar to RNA interference, or RNAi, that occurs in cells, including a mechanism mediated by an enzyme called Adenosine Deaminase Acting on RNA, or ADAR. Our RNA editing approach involves co-opting this endogenous editing system via a proprietary engineered oligonucleotide to introduce precise edits to RNA. We iteratively optimize the editing efficiency of our product candidates using a combination of ADAR biology, chemistry and machine learning expertise. Using this approach, we can edit the transcriptome with high efficiency and specificity. The application of such an approach can provide the ability to alter a SNV and affect biology in meaningful ways.

We have assembled a suite of technologies and capabilities to build our RNA editing platform, Oligonucleotide Promoted Editing of RNA, or OPERA.



OPERA integrates a deep understanding of ADAR biology with expertise in oligonucleotide chemistry, machine learning optimization of oligonucleotides and fit-for-purpose delivery, all of which are expected to enable rapid iteration of our product candidates across therapeutic targets. OPERA relies on the following key components that enable us to generate the proprietary RNA editing oligonucleotides that form the basis of our differentiated product candidates:

- Deep understanding from ADAR biology, supported by extensive preclinical research using *in vitro* assays and proprietary mouse models as well as the fundamental work of our scientific advisors and founders to elucidate key insights and know-how of ADAR biology. This enables an understanding of ADAR activity in different species and disease states, allowing us to develop novel product candidates.
- Expertise in oligonucleotide chemistry, enabled by the ability to identify and incorporate chemical modifications to generate a fully modified synthetic oligonucleotide. This increases our ability to generate oligonucleotides with drug-like properties, thereby increasing the editing and translational efficiency of our product candidates.
- Machine learning optimization of oligonucleotides, driven by data science and computational capabilities for rapid design and iteration resulting in optimal product candidates for each disease being pursued.
- Fit-for-purpose delivery, made possible by tissue-specific delivery technologies that can enhance biodistribution, specificity, durability and editing efficiency of product candidates for each given disease.

Such product candidates include Customized High-fidelity Oligonucleotides for RNA Deamination, or CHORDs™. CHORDs are single stranded, anti-sense oligonucleotides designed to have high target efficiency and specificity by leveraging the pillars of OPERA.

The versatility of RNA editing combined with our OPERA platform broadens the therapeutic target space significantly. While our approach can be used to repair pathogenic SNVs, as demonstrated by our most advanced program, our Alpha-1 Antitrypsin Deficiency, or AATD, product candidate, KRRO-110, we can also engineer *de novo* SNVs and change amino acids on proteins to endow them with desired properties while preserving their broader functional capabilities as exemplified by three of our other programs (sAH, ALS, Pain). In preclinical studies, we have demonstrated that single RNA changes can disrupt protein-protein interactions, prevent protein aggregation, selectively modulate ion channels and activate kinases. These modification approaches can unlock validated target classes that have historically been difficult to drug, enabling us to pursue a broad range of diseases with potentially large addressable patient populations traditionally out-of-scope for other genetic medicine approaches and current traditional drug modalities.

Each of our programs demonstrates the versatility of the oligonucleotide-based ADAR-mediated RNA editing approach to bring additional precision and tunability to address a broad range of rare and highly prevalent diseases.

- Repairing pathogenic variants: A SNV that is a G to A mutation on DNA, leading to an aberrant amino acid on a protein can be repaired using RNA editing. Such an approach is relevant when the patient population has a spectrum of disease manifestations from mild-to-severe.
- Disrupting protein-protein interactions: A single SNV observed in human genetic association studies has the potential to inform how to transiently activate a protein pathway. We can generate this protein variant transiently using our RNA editing product candidates, thereby engineering a *de novo* SNV.
- Other target classes: There are multiple other target classes that can be addressed such as preventing protein aggregation selectively modulating ion channels and activating kinases that have been traditionally hard to leverage for developing medicines.

The pipeline chart below demonstrates the breadth of indications and applications enabled by our OPERA platform, with an initial focus on four programs that are all wholly-owned. In addition, we have two other wholly-owned programs not reflected in

the pipeline chart below: one for an undisclosed target for sAH, and one for a kinase target for cardiometabolic disease. All of our programs are still in the research or preclinical stage of development and their risk of failure is high.

[1] Subject to submission of regulatory filing and authorization to proceed

We continue to make meaningful advancements across our programs, including advancing KRRO-110 towards clinical development. Specifically, KRRO-110 is our first development candidate for the treatment for AATD that, using our proprietary RNA editing approach, repairs the amino acid codon. This repair of the endogenous protein has the potential to clear protein aggregates from within liver cells to create a potentially disease-modifying clinically differentiated benefit for liver function and to preserve lung function by providing an adequate amount of normal AAT protein. AATD is an inherited genetic disorder that can cause severe progressive lung and liver disease due to a lack of normal alpha-1 antitrypsin protein, or AAT, caused by SNV mutations in the SERPINA1 gene. There are an estimated 3.4 million individuals with deficiency allele combinations worldwide. Despite being minimally effective and not fully addressing the needs of many AATD patients, augmentation therapy currently represents approximately $1.4 billion in annual sales worldwide. KRRO-110 has the potential to elevate the standard of care and expand the number of patients on treatment and potential to be a leader with a large market opportunity worldwide. However, we have yet to conduct any human clinical trials, our product candidate is in early stages of development and there is no guarantee we will be successful.

KRRO-110 is a proprietary oligonucleotide that utilizes a proven lipid nanoparticle, or LNP, based delivery system administered intravenously to transiently restore production of normal AAT in liver hepatocytes. By correcting the pathogenic G to A SNV in the SERPINA1 gene, we aim to bring individuals with the Z mutation to a phenotype where over 50% of RNA has been corrected to produce normal AAT protein, preserving lung and liver function and preventing further damage. However, this delivery system has not yet been finalized and, while LNPs have been validated clinically to deliver oligonucleotides, such as siRNA, they have not been clinically proven to deliver oligonucleotides for RNA editing, such as our product candidates.

We believe our approach for treating AATD has multiple potential advantages:

- Provides a disease modifying therapy for both lung and liver manifestations by transiently editing over 50% of RNA transcripts in hepatocytes to restore normal AAT protein
- Provides a treatment option that can be tailored to address the broad spectrum of severity within the AATD population
- Potential to enable physiologic regulation of AAT using endogenous ADAR, thereby increasing normal AAT production during inflammation
- Fit-for-purpose delivery using a proven LNP to maximize editing efficiency, leading to greater potential clinical benefit

We have generated compelling preclinical data demonstrating proof of concept across multiple RNA editing oligonucleotides targeting the SERPINA1 gene, including KRRO-110.

- KRRO-110 has achieved high editing efficiency *in vitro* (>50% editing in hepatocytes).
- KRRO-110 demonstrated high specificity with no bystander effects in MZ human primary hepatocytes.
- Intravenous administration of KRRO-110 at 2mg/kg in a human transgenic PiZ mouse model resulted in secretion of approximately 50uM total AAT protein as early as seven days post-single dose.
- KRRO-110 increases the expression of normal AAT protein to >70% of total AAT protein in circulation *in vivo*.

- An increase in functional AAT protein with the inhibition of elastase activity was sustained through week nine when KRRO-110 was dosed every two weeks, demonstrating durability in mice.
- Engineered surrogate oligonucleotides that edit RNA at an adjacent site on the SERPINA1 gene relative to KRRO-110 have high translation of RNA editing efficiency from mice to non-human primates, or NHPs, demonstrating the potential applicability of our approach in humans.

While we believe we can demonstrate many of the key advantages of RNA editing, we are very early in our development efforts and not yet certain of the results we may achieve. Such uncertainties include, but are not limited to, the level of editing efficiency needed in a target tissue type to achieve a clinical benefit, and associated safety of our edits in humans. Preclinical development of KRRO-110 is ongoing in preparation for a potential regulatory filing in the second half of 2024, and an anticipated interim clinical readout in the second half of 2025.

Our Team

We were launched in 2018 and were co-founded by Nessan Bermingham, Jean-Francois Formela, Joshua Rosenthal and Andrew Fraley. Our science was based on pioneering research from the laboratory of Joshua Rosenthal, Ph.D., at the Marine Biological Laboratory, or MBL in Woods Hole, Massachusetts. Dr. Rosenthal's work includes landmark discoveries in RNA editing based on adenosine deamination.

We are led by an experienced team with deep expertise in genetic medicines, development of oligonucleotide-based therapeutics, building novel therapeutic platforms, and bringing multiple therapeutics to market. In addition, our executive leadership team has a successful track record of company building and leading biotech companies, including Ram Aiyar, Ph.D., President and Chief Executive Officer, an experienced executive and company builder with 20 years of diverse industry experience including research, business and strategy; Steve Colletti, Ph.D., Chief Scientific Officer, who brings nearly 30 years of drug discovery and development experience covering a broad range of therapeutic areas and modalities; and Vineet Agarwal, Chief Financial Officer, who brings more than 14 years of financial and industry experience as a biotech investment banker with J.P. Morgan Chase & Co. We also have an accomplished scientific advisory board comprised of leading experts in the fields of ADAR biology, chemistry, translation medicine, and nucleic acid therapeutics.

We are a mission-driven organization and thrive through a strong culture of scientific innovation and behavior that embodies our core values and principles. We are actively working to rewrite the future of medicine by remaining on the cutting edge of science and research. We believe our success is enabled by working better together and embracing diversity, leading us to employ a dynamic team with varied expertise, enabled by kindness and integrity.

We have attracted a talented team of industry experts and experienced scientists as part of a high-performing, nimble organization. Our research and development organization is comprised of individuals with expertise in DNA editing technologies, liver biology, CNS biology, medicinal chemistry, biochemistry and drug delivery, translational medicine and conducting preclinical studies.

Since inception, we have raised $343 million in capital, including $117 million as part of the pre-closing financing that closed immediately prior to the Merger, or the Pre-Closing Financing, from premier venture capital funds, healthcare-dedicated funds, major mutual funds and other leading investors that share our vision of creating transformative genetic medicines for diseases with high prevalence.

Our Strategy

Our mission is to discover, develop and commercialize a new class of RNA editing therapies capable of improving the lives of patients with rare and highly prevalent diseases. We do this by applying our proprietary RNA editing platform, OPERA, which combines our unique expertise in ADAR biology and oligonucleotide chemistry with machine learning-driven optimization and fit-for-purpose delivery. Our RNA editing product candidates are designed to harness the body's natural RNA editing processes to make a precise single base edit. However, this has only been observed in preclinical studies as we have yet to submit an investigational new drug application, or IND, to the U.S. Food and Drug Administration, or FDA, or commence a clinical trial. Our goal is to develop a portfolio of RNA editing product candidates with best-in-class properties across a range of diseases by executing on the following key pillars of our strategy:

- **Develop a novel class of RNA editing therapies using learnings from a combination of genetics and approved medicines.** We are leveraging significant advances in the understanding of the correlation between DNA, RNA and disease phenotypes to develop novel therapeutic approaches across a range of validated biological targets. This novel class of RNA editing therapeutics combines the precision of genomic therapies with the properties associated with traditional approved drugs, such as titratability and ability to re-dose. In addition, our RNA editing product

candidates are structurally similar to other clinically and commercially validated drug modalities such as antisense oligonucleotides, or ASOs, and small-interfering siRNAs, conferring potential advantages in manufacturing, regulatory review and clinical adoption.

- **Develop and advance into the clinic a differentiated disease-modifying therapy for patients with AATD.** Our first development candidate, KRRO-110, has the potential to provide a differentiated therapeutic option for AATD by addressing both the liver and lung pathologies. Our RNA editing oligonucleotide product candidates, including KRRO-110, have generated compelling preclinical data that demonstrates restoration of normal AAT protein while preventing the aggregation of dysfunctional AAT in the liver. Our preclinical *in vivo* data has demonstrated durability and high editing efficiency in both mice and NHPs, illustrating the potential applicability in humans. Preclinical development of KRRO-110 is ongoing in preparation for a potential regulatory filing in the second half of 2024, and an anticipated interim clinical readout in the second half of 2025. Depending on the evidence of efficacy and tolerability, we intend to pursue expedited regulatory pathways. However, regulators have not yet established any definitive guidelines related to overall development considerations for RNA editing therapies and no clinical data has been generated to date.
- **Deploy our versatile OPERA platform to develop a portfolio of programs that modify proteins transiently to expand into highly prevalent diseases.** OPERA has the ability to generate unique RNA editing therapies that can modify protein function or endow proteins with engineered changes that will potentially result in desirable properties to treat disease. In preclinical studies, we have demonstrated that single RNA changes can disrupt a protein-protein interaction, prevent protein aggregation, selectively modulate an ion channel and selectively activate a kinase. These modification approaches can unlock validated target classes that have historically been deemed undruggable, enabling us to pursue a broad range of diseases, including those with high prevalence. We are evaluating potential applications of our OPERA platform for use in other highly prevalent indications including the central nervous system, or CNS, liver, and cardiometabolic therapeutic areas.
- **Continue to optimize and enhance our OPERA platform.** We believe we have built a leading RNA editing company through a combination of our OPERA platform, intellectual property strategy and human capital. Our computationally driven approach enables rapid design and optimization of potential oligonucleotide product candidates. In development of our AATD program, we were able to go from "design-to-data" in 5-6 weeks. We intend to continue to incorporate new data into these machine learning models to improve their ability to predict editing efficiency and to more expeditiously optimize and nominate new product candidates, although there is no guarantee that this will result in an accelerated development or approval timeline, if at all.
- **Maximize the potential of our OPERA platform through collaborations and strategic partnerships.** We currently retain worldwide development and commercialization rights to our programs and platform. We actively collaborate with clinical leaders, academic medical centers of excellence, and patient advocacy groups to continue to enhance our expertise in our focus therapeutic areas. Given the versatility and broad potential of our OPERA platform across therapeutic areas, especially in diseases with high prevalence, we may enter into strategic partnerships with external parties that have complementary capabilities to broaden and accelerate access to our RNA editing therapies.
- **Invest in human capital and encourage innovation to maintain a leading position and advance the frontiers of genetic medicines.** We are a mission-driven organization, and we thrive through a strong culture that embodies our core values. We are actively working to rewrite the future of medicine and remain on the cutting edge of science and research by working better together and embracing diversity in employing a dynamic team with varied expertise, enabled by kindness and integrity. We have attracted a talented team of industry experts and experienced scientists as part of a high-performing and nimble organization. Our research and development organization is comprised of individuals with expertise in editing technologies, RNA biology, liver biology, CNS biology, medicinal chemistry, biochemistry and delivery, translational medicine, preclinical and clinical development.

Expanding the Frontiers of Genetic Medicines: RNA Editing

The advent of large-scale genome sequencing has progressively revealed causal genetic variation underlying several human diseases, both rare and highly prevalent. Genetic mutations, including SNVs, implicated in disease have been found to be diverse in nature and can affect the function of genes and their associated downstream biochemical pathways. Natural genetic variations, revealed by population-level genomic studies, have also been shown to protect against or to increase the risk of disease. Beyond these developments, groundbreaking advances in gene therapy, cell therapy and RNA therapeutics have resulted in several approvals that have transformed the treatment of certain genetic diseases and cancers as well as the prevention of infectious diseases, such as COVID-19. In addition, various DNA editing approaches have been developed that introduce specific genetic changes to DNA to treat diseases. First generation CRISPR-Cas9 DNA editing has demonstrated the potential to knockout

pathogenic mutations at the single gene level with several programs in clinical development and the first *ex vivo* DNA editing therapeutic for a rare hematological condition on file at the FDA. Next generation DNA editing approaches have recently entered the clinic and hold the promise to edit DNA at the single nucleotide level.

Despite these advances, significant risks exist with DNA editing approaches. A key concern is the introduction of unwanted DNA modifications ("off-target" edits) which could have permanent adverse effects such as chromosomal integration and non-specific insertions, deletions and substitutions. Additionally, due to the complexity of a multicomponent DNA editing product, delivery to target cells can be challenging and even more so if there is a need to edit multiple genetic loci. Furthermore, manufacturing is highly complex and expanding to commercial scale remains challenging, specifically for a highly prevalent indication. Given these challenges, DNA editing approaches will likely remain a focus for certain rare diseases, while its ability to treat diseases of high prevalence continues to be limited.

ADAR-mediated RNA editing

RNA editing involves altering a sequence of RNA which intrinsically has the potential to address some of the limitations of DNA editing. RNA editing mediated by adenosine deaminase acting on RNA, or "ADAR-mediated" RNA editing, has recently emerged as a differentiated approach that can generate product candidates having features that combine the precision of genomic therapies with the properties commonly associated with current approved drugs such as titratability and ability to re-dose. Importantly, these drug-like characteristics enable ADAR-mediated RNA editing candidates to be potentially safer and target diseases with high prevalence that would be difficult for DNA editing approaches to address.

ADARs are a family of enzymes present inside a cell, that bind RNA. ADARs bind double-stranded RNA structures, and convert a single base of adenosine (A) on RNA, into an inosine (I) that is typically translated as a guanosine (G), using an enzymatic process. ADAR mediated editing is found at high levels in cephalopods both on the coding and non-coding regions of the RNA. In humans, there are fewer recoding events, and most of the endogenous editing events occur in non-coding regions.

Humans have two known active endogenous ADAR enzymes, ADAR1 and ADAR2. ADAR1 is constitutively expressed and is present in most tissues within the body, whereas ADAR2 is more highly expressed in tissues such as the brain. The ADARs are essential enzymes for normal physiologic function. ADAR-driven RNA editing has been found to be critical for the function of a number of proteins, such as the glutamate ionotropic receptor, which has been found to be almost always RNA-edited in humans. Given ADARs' natural function to catalyze A-to-I edits, this endogenous editing system can be leveraged to make programmed edits to RNA. This ability to introduce programmed highly targeted edits into RNA has the potential to expand the reach of genetic medicines with an ability to modify proteins to achieve a desired function.

Oligonucleotide-based ADAR-mediated RNA Editing

There are multiple therapeutic approaches to utilize ADAR-mediated RNA editing, including synthetic oligonucleotides, engineered ADARs, and Cas-based editing approaches. Our therapeutic approach delivers oligonucleotides to target tissues and cells to introduce precise edits to RNA through recruitment of endogenous ADAR.

Normally, ADARs are recruited to target RNA editing sites through recognition of specific double-stranded RNA structures such as naturally occurring hairpins or loops in endogenous transcripts. Importantly, one can mimic these double-stranded RNA structures by introducing complementary synthetic oligonucleotides into cells. An oligonucleotide can be engineered to mimic the double-stranded RNA structure such that endogenous ADAR is recruited. Using this targeted approach, a site directed specific A-to-I edit can be introduced.

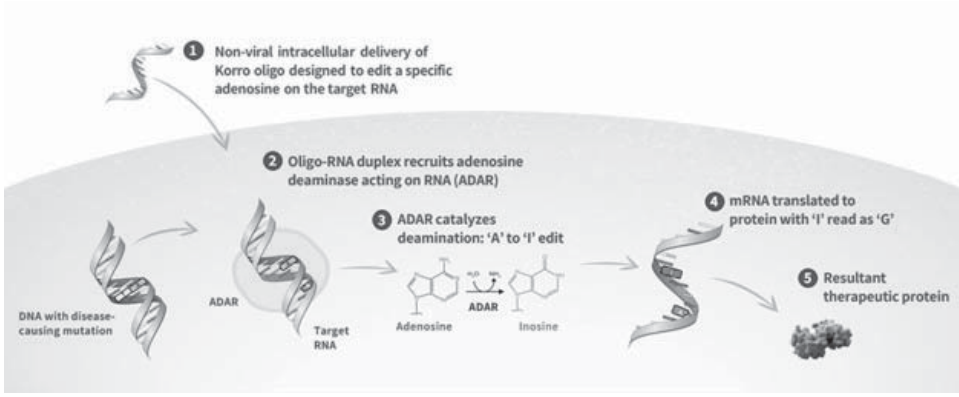
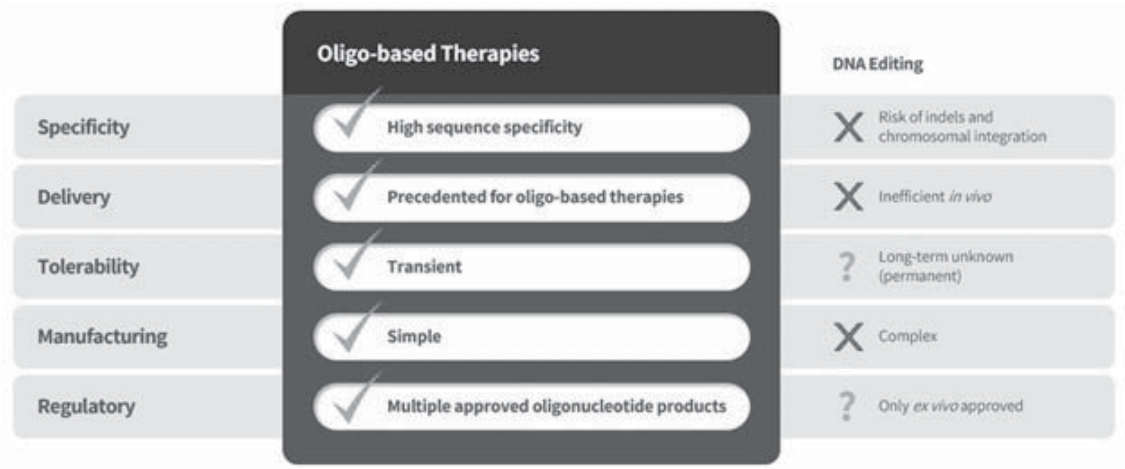


Figure 1: Mechanism of RNA editing using our proprietary platform

Key Advantages of Oligonucleotide-Based ADAR-Mediated RNA Editing as a Therapeutic Modality

We believe that oligonucleotide-based ADAR-mediated RNA editing is a groundbreaking technology that is ideally suited to expand the application of genetic medicines for indications that DNA editing is unable to address. Over the last two decades, there has been significant research around and development of oligonucleotide-based therapeutics, including modalities such as siRNA and ASOs, that has led to the approval of multiple drugs. Specifically, developments in oligonucleotide chemistry, delivery technologies, tolerability, and manufacturing, combined with better defined regulatory pathways, have led to the approval of oligonucleotide-based therapeutics specific for multiple different tissue types. We differentiate our approach from DNA-editing by leveraging the know-how from approved oligonucleotide therapies in development of our product candidates.



While we believe we can demonstrate many of the key advantages of RNA editing, we are very early in our development efforts and not yet certain of the results we may achieve. Such uncertainties include, but are not limited to the level of editing efficiency needed in a target tissue type to achieve a clinical benefit, and associated safety of our edits in humans.

- Specificity: Oligonucleotide-based ADAR-mediated RNA editing enables highly precise edits at the target single nucleotide level on the RNA with low risk of off-target or bystander edits, addressing a key safety concern associated with other DNA editing approaches that carry the risk of permanent insertions and deletions as well as chromosomal integration. Using synthetic oligonucleotides, appropriate chemical modifications can be introduced to increase the overall specificity and targeting efficiency for the site directed RNA editing. The OPERA oligonucleotides are designed to be highly site selective with minimal to no bystander effects or halo effects. To assess global off-target editing, we use a bulk RNA-seq approach to detect base frequency changes at potential off target sites between control and treated samples. We sequence target amplicons via NGS and assess potential A to G editing at all sites across the transcript. In preclinical *in vivo* studies, we have shown that off-target RNA editing using our technology is negligible and transient.

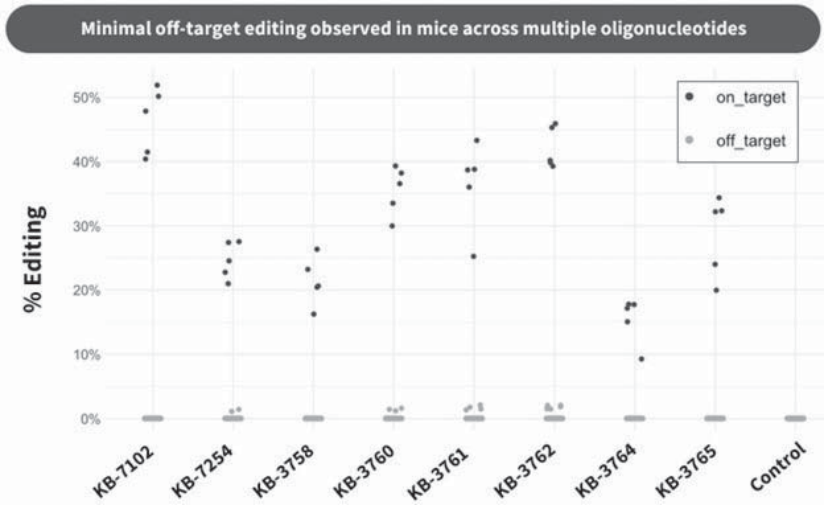


Figure 2. Overview of off-target editing observed in mice across multiple oligonucleotides

- Delivery: Oligonucleotide-based ADAR-mediated RNA leverages well established, clinically precedented delivery approaches used in other approved products, such as LNPs and ligand-based approaches. LNP-based delivery of oligonucleotides is a well established and clinically validated delivery approach that provides sustained targeted delivery and editing efficiency, resulting in infrequent dosing and an acceptable tolerability profile. Additionally, LNP delivery of RNA-editing oligonucleotides enhances optimal distribution to targeted cells. One example of a well-established and clinically validated ligand-based delivery approach is GalNAc delivery of oligonucleotides, which provides highly specific and effective delivery to hepatocytes with improved durability.
- Tolerability: ADAR-mediated RNA editing has a low risk of immunogenicity and can potentially lower off-target editing events resulting in an improved tolerability profile compared to DNA editing approaches. The lower risk of immunogenicity enables the ability to re-dose patients if required, a significant limitation of editing approaches that utilize viral vectors and bacterial Cas systems that carry a higher risk of immunogenicity. The transient and reversible nature of ADAR-based editing confers an ability to modify or cease dosing as needed.
- Manufacturing: Reliance on endogenous ADAR enzymes and the simple drug constructs of oligonucleotide-based therapies has significant advantages over the complexities associated with the manufacturing and delivery of multi-component exogenous complexes used in DNA editing. Manufacturing processes for oligonucleotide-based therapies are well established, cost efficient and scalable to effectively address highly prevalent indications.
- Regulatory: Precedence of marketed oligonucleotide drugs with similar size and types of chemical modifications that therapeutic RNA editing product candidates exhibit. Guidance for the development of oligonucleotide therapeutics by global agencies, including the FDA, provides for an established pathway for the approval of this class of therapeutics. However, regulators have not yet established any definitive guidelines related to overall development considerations for RNA editing therapies and on clinical data has been generated to date.

Our OPERA – Oligonucleotide Promoted Editing of RNA – Platform

We believe we are the leading RNA editing company and have assembled a suite of technologies and capabilities called OPERA, Oligonucleotide Promoted Editing of RNA, to generate differentiated RNA editing product candidates, or CHORDs. A key challenge in developing a therapeutic approach for site-directed RNA editing is to design and optimize oligonucleotides that can drive high-efficiency. This efficiency is facilitated both by the ability to repurpose and optimize oligonucleotide constructs based on existing methods as well as utilizing computational methods to innovate on chemistry and design of the constructs. Our RNA editing product candidates are oligonucleotides capable of forming Watson-Crick base pairing with the target RNA and efficiently inducing the deamination reaction by endogenously recruiting ADAR enzymes.

We have assembled a suite of technologies and capabilities to build our RNA editing platform, Oligonucleotide Promoted Editing of RNA, or OPERA.

OPERA relies on the following key components that enable us to generate our differentiated RNA editing product candidates:

- Deep understanding from ADAR biology: Our insights and know-how of ADAR biology allow us to design oligonucleotides that efficiently recruit ADARs and promote deamination while maintaining selectivity and stability. RNA editing is dependent on endogenous ADAR expression levels and requires a deep understanding of the physiological role of ADAR, its cell and tissue distribution, the factors that lead to efficient recruitment of ADAR to targeted sites and any consequences that may arise from co-opting ADAR from its normal function. We have developed a cell-free *in vitro* RNA editing assay with purified human ADAR1 and ADAR2 that predicts the RNA editing activity with our oligonucleotides. With our cell free assay capability, we are able to limit the number of components implicated in screening (target gene, RNA editing product candidates and ADAR), allowing us to measure the kinetics of RNA editing more accurately. Using only three components including the target RNA, oligonucleotide, and ADAR, we have shown that target editing efficiency is correlated with the chemical modification pattern of an oligonucleotide. Furthermore, we have found that this activity predicts the rank order of oligonucleotides in both *in vitro* cell-based systems and *in vivo* in rodents. This assay capability supports our understanding of the key steps required to edit RNA at a level of detail that is not possible in cells. These platform biology assay capabilities have enabled detailed mechanistic studies into the tissue distribution and subcellular localization of ADAR proteins and our RNA editing product candidates.
- We have found no evidence that our RNA editing oligonucleotides interfere with endogenous RNA editing occurring naturally in a cell. ADAR naturally edits thousands of targets for a variety of reasons. We have looked at natural editing sites and chose AJUBA, COG and COPA as they have shown to be edited by ADAR to different degrees. In this experiment outlined in Figure 3, ZZ HLC cell lines were transfected with RNA editing product candidates targeting two different genes. The assays were evaluated for % editing for Target A and Target B sites as well as natural editing sites in COG, COPA and AJUBA. As shown below, natural editing sites remained unaffected

compared to the control group, demonstrating that our RNA editing product candidates are not likely to have any effect on the degree of editing of native RNA molecules.

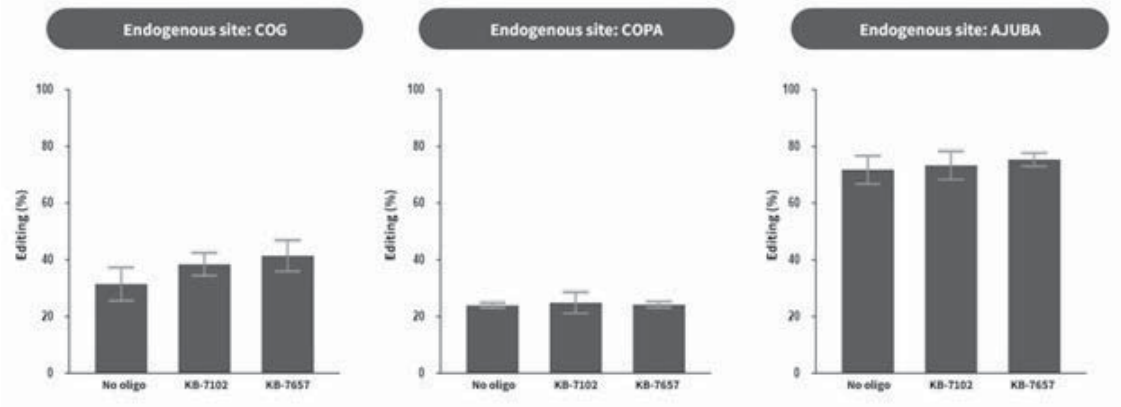


Figure 3. Our RNA editing product candidates show no evidence of interference with endogenous ADAR editing as demonstrated at the above endogenous sites

- Expertise in oligonucleotide chemistry: We have a differentiated ability to create oligonucleotide designs capable of efficiently recruiting endogenous ADAR with chemical modifications that direct high specificity editing. Our oligonucleotides increase the potency and durability of ADAR activation, thereby increasing the editing efficiency and translational efficacy of our product candidates. We have identified critical structural, sequence, and chemistry requirements for our product candidates that drive efficient recruitment of ADARs and subsequent A-to-I editing. Examples of differentiation include oligonucleotide length for efficient ADAR recruitment, use of precedented and proprietary chemistries within the oligonucleotide, as well as backbone chemistries that provide improved metabolic stability. Additionally, we combine this with 2' modification chemistries that, together, create oligonucleotides with improved editing efficiency and durability. As RNA editing is an emerging technology, there is a lack of guiding principles to design site-selective RNA editing oligonucleotide product candidates. To address this knowledge gap, we developed a robust in-house process using our high-throughput cell-based assay and machine learning capabilities to design and synthesize up to approximately 1,200 oligonucleotides per month and generate up to 6,000 assay data points for any given target.
- Machine learning optimization of oligonucleotides: We have built data science capabilities and a dedicated team to extract lessons from existing and newly generated experimental data to expeditiously design and optimize RNA editing product candidates. Our proprietary machine learning models have been trained to accurately predict oligonucleotide structure and observed levels of editing. We have been able to demonstrate that these models are able to make accurate editing predictions even for previously unseen chemical modifications demonstrating their generalizability across targets. We have demonstrated the utility of our machine learning models through an increase in overall editing efficiency of new product candidates over the last several quarters. In some cases, we have been able to go from design-to-data in as little as five weeks. However, there is no guarantee that this will result in an accelerated development or approval timeline, if at all.

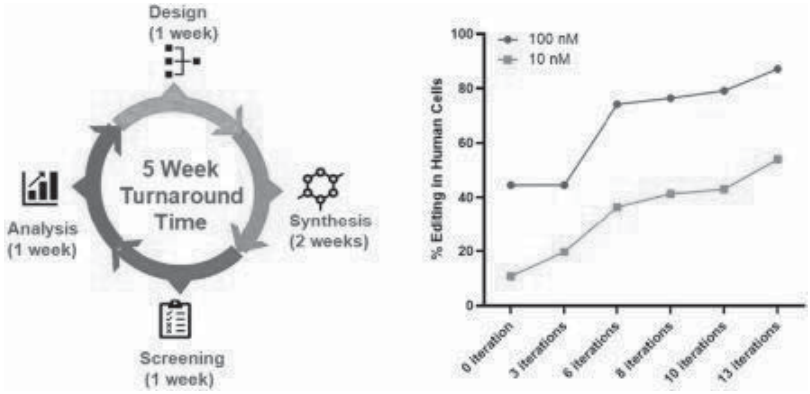


Figure 4. We have shown our ability to rapidly iterate product candidates to maximize editing efficiency

- Structural modeling is another tool that complements our ability to increase the efficiency of our RNA editing candidates. Detailed structural modeling includes shape, size and orientation requirements that can lead to successful deamination at the editing site. These aspects have an important impact on our ability to optimize RNA editing product candidates. As an example, a modification predicted by structural analyses led to a conformational change that was shown to improve editing efficiency in the coding region of the Target A *in vivo*.

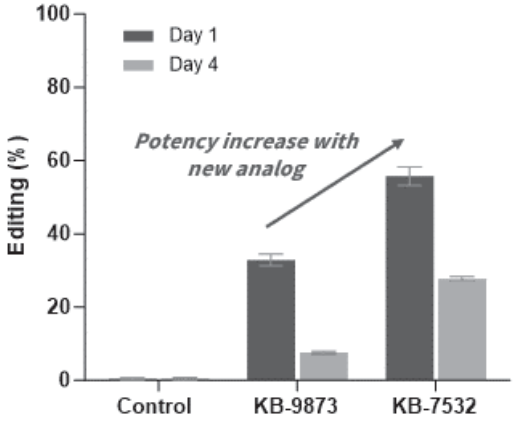


Figure 5. We have a demonstrated ability to improve editing in target coding regions

- Fit-for-purpose delivery: Our product candidates utilize short synthetic oligonucleotides, which we believe can be efficiently delivered using technologies such as LNP or GalNAc. These delivery technologies are well established and clinically validated and have been developed for precedented modalities such as siRNAs and ASOs. Each of these delivery vehicles has optimal characteristics suited for a given therapeutic application. Using CHORDs, we achieved greater than 50% editing in vivo utilizing both a ligand-based GalNAc conjugate and an LNP-based delivery approach (see Figure 6).

Additionally, LNP mediated delivery of RNA editing product candidates provides sustained delivery and an acceptable tolerability profile that have been manufactured at a scale sufficient to serve the target population. In addition to LNP based delivery approach, ligand-based approaches (ex., GalNAc for liver hepatocytes) can also be used for effective delivery and to improve durability with OPERA's RNA editing product candidates, which we have also evaluated in preclinical *in vivo* models. In contrast to treatments targeting liver hepatocytes where there is a need for a delivery system, our RNA editing product candidates have been delivered intrathecally to the central nervous system without a need for any delivery system in preclinical mouse models. Thus, our choice of delivery system is a fit-for-purpose model that is dependent on the oligonucleotide design as well the suitability for the indication and tissue localization of the target. However, this delivery system has not yet been finalized and while LNPs have been validated clinically to deliver oligonucleotides, such as siRNA, they have not been clinically proven to deliver oligonucleotides for RNA editing, such as our product candidates.

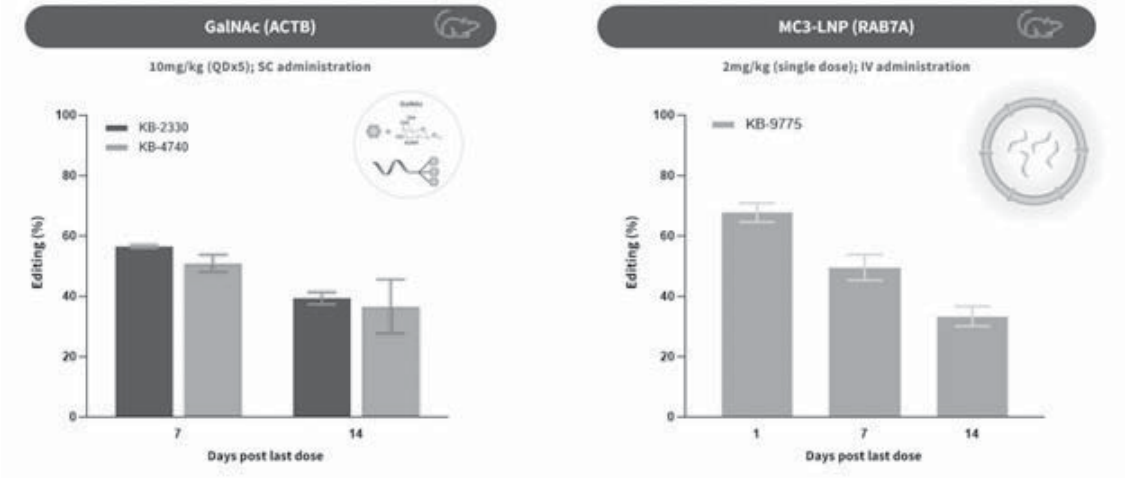
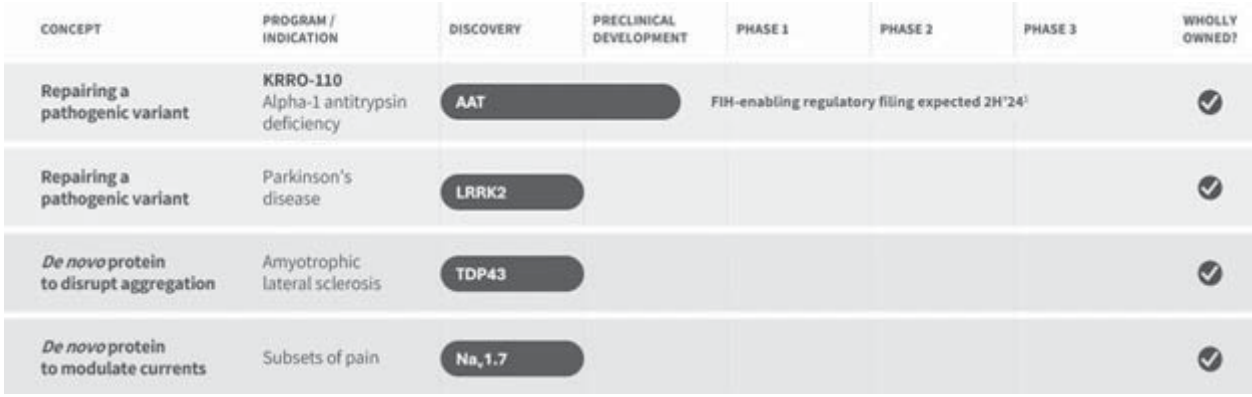


Figure 6. Demonstrated greater than 50% editing using GalNAc conjugates in the liver in a preclinical mouse model.

Our Pipeline Demonstrates the Versatility of the OPERA Platform

We are advancing a broad pipeline of four programs that are wholly owned and demonstrate the versatility of our OPERA platform. In addition, we have two other wholly-owned programs not reflected in the pipeline chart below: one for an undisclosed target for sAH, and one for a kinase target for cardiometabolic disease. All of our programs are still in the research or preclinical stage of development and their risk of failure is high.



[1] Subject to submission of regulatory filing and authorization to proceed

Our proprietary oligonucleotides co-opt endogenous ADAR to perform a single A-to-I base edit on RNA to modify protein function. Delivery of these proprietary oligonucleotides into a cell forms an oligonucleotide-RNA duplex which recruits endogenous ADAR, ultimately editing a target adenosine (A) to inosine (I) on RNA. The location of the edit can lead to a multitude of effects including changes in expression and regulation of mRNA. In the event an edit is made in the coding region of the gene, the mRNA is then translated to a protein with the (I) inosine read as a (G) guanosine, resulting in a modified protein. The resultant therapeutic protein can be applied to go beyond repairing pathogenic SNVs by changing amino acids on proteins implicated in disease biology. Single amino acid changes to non-mutated RNA can create *de novo* modified protein variants with desired altered properties while preserving their broader functional capabilities.

Repairing pathogenic variants: Our OPERA platform enables the development of RNA editing therapies that can repair SNVs on mRNA to express normal proteins through the introduction of precise genetic changes without creating permanent changes to the genome. These normal proteins can be uniquely expressed at desired levels and duration to address both rare and highly prevalent diseases caused by a pathogenic SNV. This approach is especially relevant when the same underlying genetic SNV manifests in a broad disease phenotype from mild to severe forms of the disease.

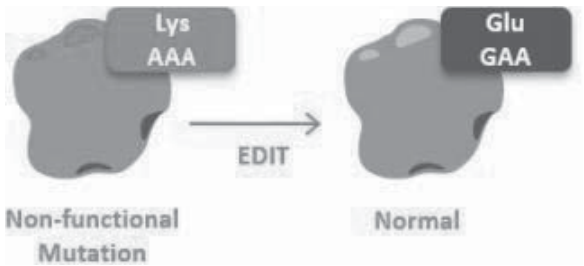


Figure 7. RNA editing of a single nucleotide can restore normal protein expression

Our lead program for AATD addresses a single genetic SNV in the SERPINA1 gene that causes the development of AAT deficiency, which has a high unmet medical need and for which there are no disease modifying treatment options. The disease manifests with a heterogenous population having both liver and lung pathologies. By specifically editing a single nucleotide, the normal synthesis of AAT is restored, resulting in secretion of normal AAT to levels which are predicted to protect the lung from further decline in function. The correction of a subset of AAT produced also prevents aggregation of AAT protein in the liver, thereby potentially alleviating damage to the liver.

Similarly, we are developing a product candidate that addresses a LRRK2 mutation for PD patients. Mutations in LRRK2 that are associated with aberrantly enhanced kinase activity are the most common cause of genetic PD. The G2019S mutation in the LRRK2 protein is the most common pathogenic mutation, accounting for 1–6% of sporadic and 3–19% of familial PD cases. Repairing the G2019S mutated nucleotide can restore the normal LRRK2 protein and return its activity to a physiological state, which we believe may be disease modifying in these patients.

Other protein modifications

Approximately 85% of the human proteome has historically been considered undruggable through traditional therapeutic modalities as many proteins lack defined small molecule binding sites or are inaccessible by biologics. The versatility of RNA editing, combined with our OPERA platform, addresses a meaningful portion of the undruggable human proteome and broadens the target space. Our target identification and selection for programs is based on strong genetic evidence implicating each target in its disease pathology.

Our initial focus is to make edits to the coding region of a transcriptome. Making changes post-transcriptionally, after the mRNA has been created and prior to the protein being translated, provides an exquisite, selective approach for modifying proteins. In preclinical studies, we have demonstrated that single RNA changes can disrupt protein-protein interactions, prevent protein aggregation, selectively modulate an ion channel and selectively activate a kinase. These modification approaches have the potential to unlock validated target classes that have historically been difficult to drug, enabling us to pursue a broad range of diseases, including those with high prevalence and large market opportunities.

Target classes	Examples	
Repairing a pathogenic variant	Repairing aberrant amino acids on proteins implicated in disease biology	Parkinson's disease
Disrupting protein-protein interactions	Activating transcription factor expression by disrupting binding with a negative regulator	Severe alcoholic hepatitis
Preventing protein aggregation	Preventing disease-causing protein aggregation	Amyotrophic lateral sclerosis
Modulating ion channels	Regulating the activation of ion-channels to physiological levels	Subsets of pain

Disrupting Protein-Protein Interaction: Modulating protein-protein interactions provides a novel modality to target intracellular proteins specifically for increasing the activity of the protein. Single amino acid changes to non-mutated RNA can disrupt binding of inhibitors to target proteins, including transcription factors, promoting enhanced biological activity of the target protein. There are two ways this could provide increasing activation, either through a hyperactive protein, or through the presence of a longer half-life or both. Such an approach highlights the broad capabilities of what an RNA editing platform can accomplish in driving biological change.

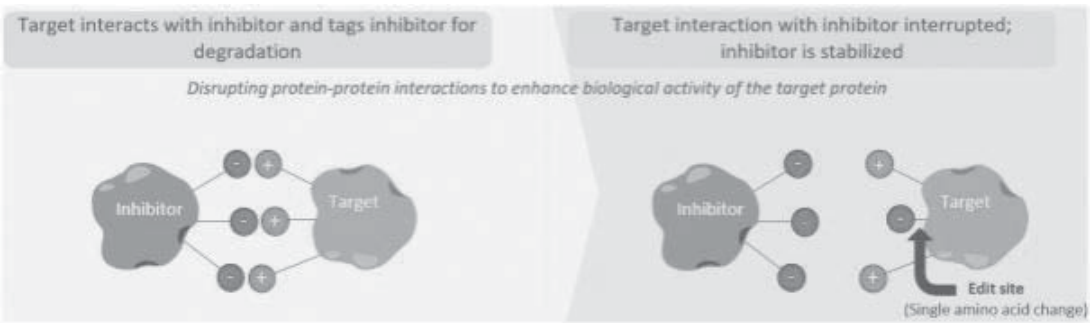


Figure 8. Our oligonucleotides have the ability to disrupt protein-protein interactions using precise edits

One of our programs for disrupting protein-protein interactions is in development for the treatment of severe alcohol-associated hepatitis (sAH). We are selectively modulating a validated transcription factor protein implicated in the disease pathology for sAH. This oligonucleotide product candidate leads to the synthesis of a protein variant that disrupts interaction with its inhibitor and, as a result, increases expression of clinically beneficial downstream target genes. Other approaches have attempted to disrupt the interaction with non-selective small molecules, resulting in unacceptable side effects, or by knocking down the regulatory protein, which is also responsible for regulating other important proteins. In a retrospective analysis of a study looking at sAH patient liver samples, increased expression of these target genes has been shown to have better prognosis. In addition to sAH, this transcription factor is a validated target for other liver, cardiometabolic and inflammatory diseases, which may provide a "pipeline-in-a-product" opportunity.

Other Target Classes: In addition to disrupting protein-protein interactions, we are also advancing product candidates to prevent protein aggregation, selectively modulate ion channels and activate kinases.

Intracellular protein aggregation is a cause of multiple diseases across the body. Specifically in neurodegenerative diseases, accumulation of specific proteins within neurons are pathogenic including Alzheimer's disease, PD, and ALS. Creating a protein variant that can prevent the aggregation, while preserving its intrinsic function, is a therapeutic approach that has the potential to provide a differentiated therapeutic option over knocking down or silencing the protein through alternate mechanisms.

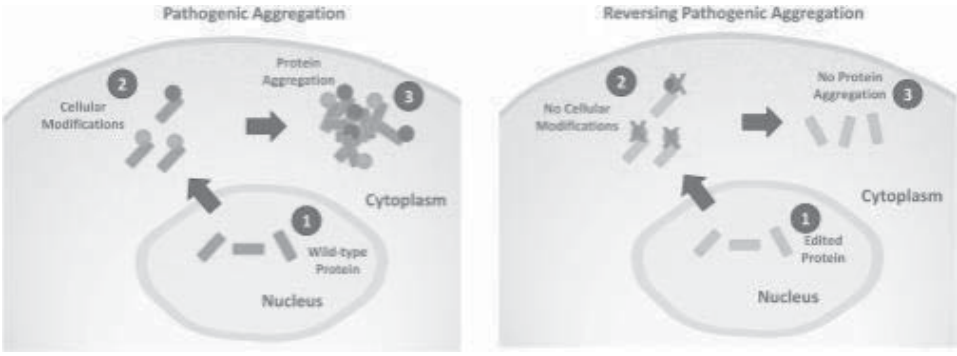


Figure 9. Our product candidates can reduce pathogenic aggregation of undesirable proteins

One of our programs for disrupting protein aggregation is in early-stage discovery for the treatment of ALS. We are selectively modulating TDP-43, an RNA/DNA-binding protein, which carries out a variety of important functions in healthy neurons including initiation of transcription, pre-mRNA splicing, and miRNA processing. In pathological conditions, such as ALS, TDP-43 is depleted from the nucleus and accumulates as protein aggregates in the cytoplasm in hyperphosphorylated, ubiquitinated, and cleaved forms. These aggregates are observed in more than 90% of ALS patients. A single RNA edit to TDP-43 is predicted to lead to the synthesis of a protein variant that does not aggregate and preserves its normal function. Given TDP-43 is essential for neuronal health, knocking down the protein could be detrimental.

We believe that the elegance and versatility of our RNA editing approach will enable a robust pipeline of potentially disease modifying product candidates to treat diseases previously unattainable by genetic medicine approaches. While the above examples demonstrate the breadth of applications enabled by OPERA, we believe our RNA editing approach will bring the first genetic medicine to address the complex genetic underpinnings of highly prevalent diseases.

Our AATD Program: RNA Editing to Repair Pathogenic Missense Variant

Our first development candidate, KRRO-110, is in development as a potential treatment for Alpha-1 Antitrypsin Deficiency, or AATD, that has the potential to be disease-modifying and provide a differentiated therapeutic option. AATD is an inherited genetic disorder that can cause severe progressive lung and liver disease due to a lack of normal alpha-1 antitrypsin protein, or AAT, with varying intensity based on patient genotype and environmental factors. Patients often develop chronic obstructive pulmonary disorder, or COPD, in the lungs and cirrhosis of the liver, which can result in liver failure or death.

There are an estimated 3.4 million individuals with deficiency allele combinations worldwide. There is a single approved modality, a once-a-week infusion of pooled human plasma derived AAT, that does not adequately address the lung or liver manifestations of AATD. Within the United States alone, the opportunity to improve the existing standard of care and expand the treated population represents a large market opportunity.

KRRO-110 is a proprietary RNA editing oligonucleotide that is delivered to liver cells using an established LNP platform to restore production of normal AAT. KRRO-110 is expected to be delivered via intravenous infusion, where it co-opts endogenous ADAR to repair the pathogenic SNV and restore production of normal AAT, creating a clinically differentiated benefit for both liver and lung function in affected individuals.

In addition to the inherent benefits of ADAR-based RNA editing described earlier, we believe our approach has additional potential advantages:

- Provides a disease modifying therapy for both lung and liver manifestations by transiently editing over 50% of RNA transcripts in hepatocytes to restore normal AAT protein
- Provides a treatment option that can be tailored to address the broad spectrum of severity within the AATD population

- Potential to enable physiologic regulation of AAT using endogenous ADAR, thereby increasing normal AAT production during inflammation
- Fit-for-purpose delivery using a proven LNP to maximize editing efficiency, leading to greater potential clinical benefit

We have generated compelling preclinical data demonstrating proof of concept across multiple RNA editing oligonucleotides targeting the SERPINA1 gene, including KRRO-110. KRRO-110 has achieved targeted durability, high editing efficiency (>50% editing in hepatocytes) and increased expression of normal AAT protein (>70% of total AAT protein in circulation) in an *in vivo* mouse model. We have also shown that earlier generation oligonucleotides that target a different site on the SERPINA1 gene than KRRO-110 have high translation of RNA editing efficiency from mice to non-human primates, or NHPs, demonstrating the potential applicability of our approach in humans. With regard to KRRO-110, we have shown high specificity with no bystander effects in MZ human primary hepatocytes. Additionally, we have demonstrated that intravenous administration at 2mg/kg resulted in secretion of approximately 50uM functional AAT as early as seven days post-single dose in a human transgenic PiZ mouse model. An increase in AAT protein and the inhibition of elastase activity were sustained through week nine when dosed every two weeks, demonstrating durability in mice. Finally, we have shown greater than 40% editing in NHPs utilizing an earlier generation oligonucleotide designed to edit a surrogate SERPINA1 RNA target site.

While we believe we can demonstrate many of the key advantages of RNA editing, we are very early in our development efforts and not yet certain of the results we may achieve. Such uncertainties include, but are not limited to, the level of editing efficiency needed in a target tissue type to achieve a clinical benefit, and associated safety of our edits in humans. Preclinical development of KRRO-110 is ongoing in preparation for a potential regulatory filing in the second half of 2024, and an anticipated interim clinical readout in the second half of 2025. Depending on the evidence of efficacy and tolerability for our candidate in our first clinical study, we plan to pursue expedited regulatory pathways, including potentially requesting Fast Track Designation and Breakthrough Therapy Designation.

AATD Overview

AAT function

AAT is a protease inhibitor belonging to the Serpin family. It is produced in the liver and circulates in its native state in human blood at approximately 1.5 g/L, one of the highest concentrations observed for protease inhibitors. The main role of AAT is to protect tissue from proteases released by neutrophils, such as neutrophil elastase. Neutrophil elastase is an enzyme that fights infections in the lungs but can also attack normal lung tissue. If not sufficiently inhibited by AAT, neutrophil elastase destroys elastin in the lung, leading to degradation of lung function. Factors that increase lung inflammation, such as smoking or infections, increase the elastase burden in the lung, leading to severe and potentially life-threatening lung damage in AATD patients.

Genotypes of AATD

AATD is an inherited, autosomal recessive genetic disorder that is most frequently caused by a single nucleotide variant, or SNV, mutation in the SERPINA1 gene. The most common of these SNVs is the "Z" mutation, corresponding to a mutation of glutamate 342 to lysine, or E342K. A healthy individual typically exhibits an "MM" genotype, or PiMM, while an individual with a single Z allele would exhibit a heterozygous PiMZ, genotype and an individual with two Z alleles would exhibit a homozygous, or PiZZ, genotype.

Figure 10. PiMM genotype (normal liver and lung)

Impact of Z mutations on liver and lung function

The presence of a single Z allele can lead to insufficient production of normal AAT protein, as well as the production of dysfunctional AAT protein, causing manifestations of disease in both the lungs and liver. The severity of disease manifestation can vary according to each patient's genotype, as well as environmental factors, such as exposure to inflammatory respiratory agents or other complications.

PiZZ individuals experience greater manifestations of disease as a result of their very low levels of normal AAT (10%—15% of normal levels), which are insufficient to prevent lung damage post an influx of neutrophils. They are also at high risk of developing emphysema or COPD, which can present in individuals as early as in their thirties and forties. PiZZ individuals with additional environmental risk factors such as smoking or infection frequently develop COPD as early adults and develop very severe symptoms.

In addition to lung disease, PiZZ individuals can also manifest with liver disease as a result of dysfunctional AAT aggregating in the liver. In adults, this can cause liver inflammation and cirrhosis, ultimately leading to liver failure or cancer. In addition, as many as 10% of newborns with the PiZZ genotype develop cholestatic hepatitis. A quarter of impacted neonates suffer acute liver failure and require an emergency transplant.

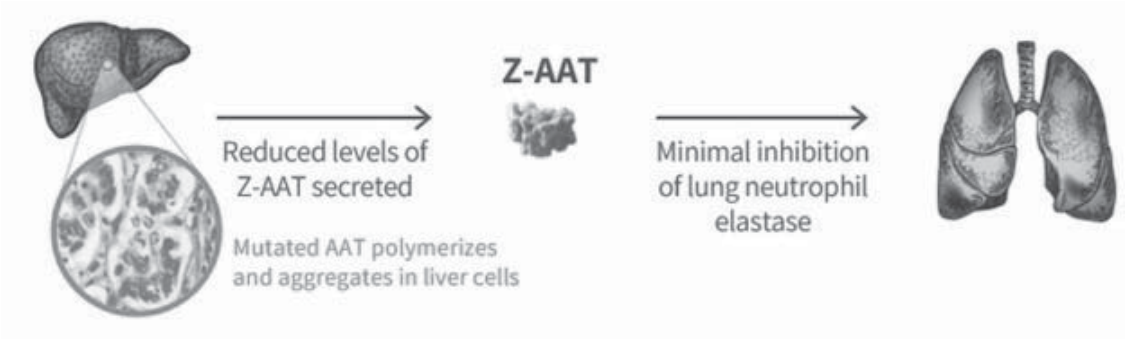


Figure 11. PiZZ genotype that results in fibrotic liver and decreased lung function

Data from the UK Biobank, or UKBB, as well as published literature, have allowed researchers to determine the threshold levels of circulating AAT that are directly linked to the PiMZ and PiZZ genotypes. In Figure 12 below, the range of AAT levels associated with normal individuals (PiMM) is compared with the range of AAT levels observed in mutated PiMZ and PiZZ patients.

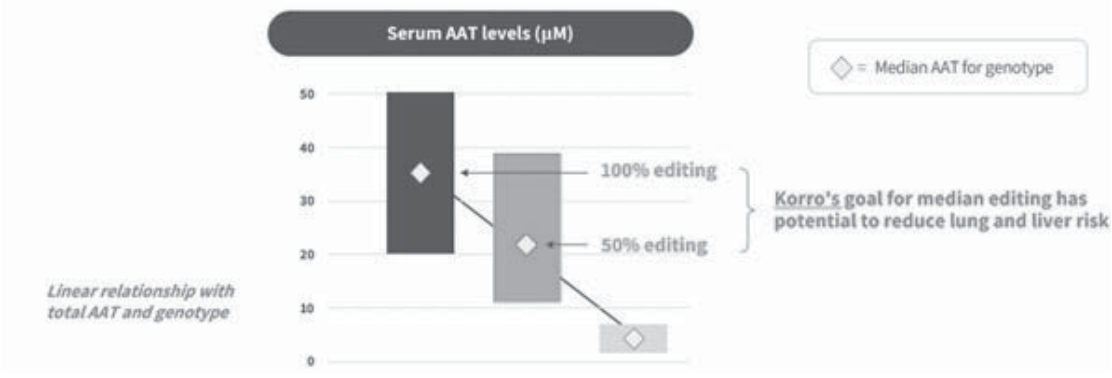


Figure 12. Median Levels of AAT and link to outcomes in liver and lung

In Figure 13 below, the Odds Ratios, or OR, associated with developing COPD and cirrhosis of the liver are compared across the two genotypes, with key findings summarized below:

- COPD: PiMZ individuals have minimal increased risk of developing COPD relative to healthy PiMM individuals, while PiZZ individuals are at very high risk with an OR of 8.8
- Cirrhosis of the liver: PiMZ individuals have mildly elevated risk of developing cirrhosis of the liver with an OR of 1.5, while PiZZ individuals have significantly elevated risk with an OR of 7.8

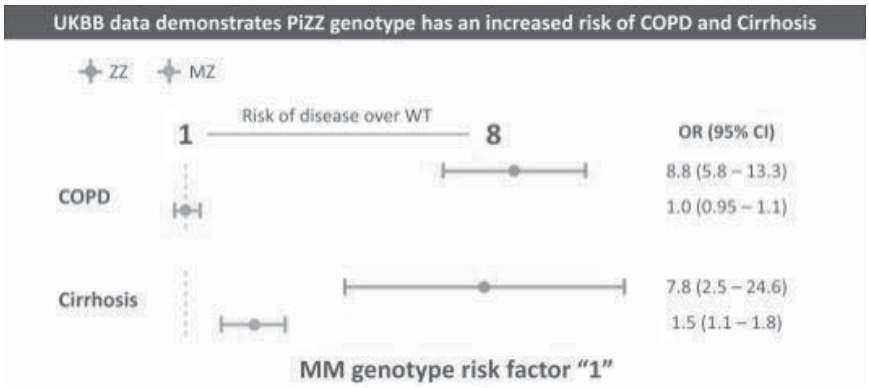


Figure 13. Risk of developing COPD and cirrhosis for different genotypes associated with AATD. Adapted from "The undiagnosed disease burden associated with alpha-1 antitrypsin deficiency genotypes." By Nakanishi T, Forgetta V, Handa T, et al. Eur Respir J 2020; 56:2001441

Based on these findings, we believe that achieving normal AAT protein levels between the ranges of the PiMZ and PiMM genotypes has the potential to alleviate the increased risk of COPD and cirrhosis of the liver, and to meaningfully improve clinical outcomes for PiZZ patients. We further believe that by achieving >50% editing efficiency across cells, we can reach these target levels and modify disease progression.

Prevalence of AATD and limitations of currently approved therapy

AATD is one of the three most common, potentially lethal, rare diseases affecting those of European descent. Worldwide, there are an estimated 3.4 million individuals with deficiency allele combinations. Studies suggest that clinical unawareness of AATD results in a significant number of patients that go undiagnosed or misdiagnosed. There are currently an estimated 100,000 patients in the United States with a PiZZ genotype, and 125,000 patients across the United Kingdom, Germany, France, Spain and Italy. Studies of PiMZ prevalence suggest as many as one in 49 individuals in the United States and one in 58 individuals across Europe.

The only FDA-approved treatment for patients with lung manifestations of AATD (co-indicated with COPD) is augmentation therapy, which utilizes AAT protein purified from pooled human plasma. The purified AAT is administered weekly by intravenous infusion with the goal of maintaining a serum level of AAT above the 11 μM threshold. Even when the serum level can be maintained at or above this threshold, augmentation therapy has not clearly demonstrated its ability to adequately address lung damage nor liver inflammation caused by AAT aggregation. Augmentation therapy is approved in only a few countries due to its limited efficacy. Lung and/or liver transplantation are the only other available treatment options, outside of standard management of the disease manifestations of AATD.

Despite being minimally effective and not fully addressing the needs of many AATD patients, augmentation therapy currently represents approximately $1.4 billion in annual sales worldwide. KRRO-110 has the potential to elevate the standard of care and expand the number of patients on treatment and potential to be a leader with a large market opportunity worldwide.

Limitations of Alternative Treatments in Development for AATD

There are a number of therapies in development to treat AATD. Certain DNA editing approaches attempt to add a normal copy of SERPINA1 gene or permanently correct the mutation within the SERPINA1 gene. DNA editing as a treatment would likely be evaluated on a risk-benefit trade-off relative to the severity of the manifestation of AATD, limiting the applicability of DNA editing approaches to the broader AATD patient population.

Additional approaches outside of DNA editing are also in development. There are approaches which attempt to use siRNA to knock-out the production of dysfunctional AAT protein, which only alleviates the liver manifestation of AATD, while potentially worsening the lung manifestation. Replacing plasma derived protein for augmentation therapy with a fusion protein is another approach in development. This fusion protein aims to introduce AAT on an antibody scaffold to improve upon the existing dosing paradigm and activity levels achieved in augmentation therapy. Fusion proteins do not resolve the liver manifestation and are unable to physiologically regulate AAT levels. Lastly, small molecule correctors attempt to promote proper folding of the Z-AAT protein. To date, small molecule correctors have been unable to achieve normal AAT levels and clinical development is focused only on the liver manifestation of AATD.

We believe many of these approaches have inherent limitations including the following:

- Inability to adequately address the spectrum of clinical pathologies associated with AATD
- Inability to achieve adequate expression of normal AAT to bring patients back to PiMM genotype
- Considerable safety and tolerability concerns
- Potential issues around manufacturability and scalability for the AATD population

Our Approach to Overcome the Limitations: Transiently Correcting the SERPINA1 Variant on RNA

We are developing KRRO-110 to treat patients with AATD. KRRO-110 is designed to leverage endogenous ADAR to make a single base edit in SERPINA1 mRNA, correcting the amino acid codon created by the pathogenic E342K SNV which stems from a single G-to-A mutation. Specifically, KRRO-110 edits the adenosine (A) to an inosine (I), thereby correcting the faulty amino acid and leading to the production of normal AAT protein.

Our goal is to bring individuals with the Z mutation to a phenotype where over 50% of RNA has been corrected to produce normal AAT protein. This would result in levels of AAT consistent with individuals in the upper half of the PiMZ genotype and the fully healthy PiMM genotype. Through human transgenic mouse models, we have shown our ability to drive the required change in RNA sequence with high efficiency, leading to secretion of AAT at target levels.

We believe our approach has multiple potential advantages, in addition to those conferred by the RNA editing modality:

- **Provides a tailored disease modifying treatment option to address the heterogeneity of the AATD population:** We leverage a transient base editing approach leading to restoration of normal AAT. The transient nature of our approach allows us to address a broader AATD patient population, inclusive of PiMZ and PiZZ genotypes. As transient editing is not permanent in nature, we have the ability to adjust dosing and even cease dosing as needed, providing a meaningful benefit in potential safety profile.
- **Provides a disease modifying therapy for both lung and liver manifestations:** By transiently editing over 50% of RNA transcripts in hepatocytes, we believe we can restore levels of normal AAT protein consistent with a PiMZ to PiMM phenotype. These levels of normal AAT have the potential to prevent further lung damage and reduce the risk of dysfunctional AAT aggregating in the liver.
- **Potential to enable physiologic regulation of AAT using endogenous ADAR:** Augmentation therapy and other treatments targeting static thresholds for AAT expression do not address the underlying mechanism of AAT regulation, which is endogenously regulated by inflammation and can sometimes lead to as much as 90uM of AAT in humans. During an inflammatory response, there is a simultaneous increase in ADAR levels. Our ADAR-based therapy has the potential to restore natural physiologic regulation by increasing the prevalence of editing during periods of greater AAT production.
- **Uniform distribution of drug to liver cells to maximize editing:** Unlike other modalities that focus on DNA editing, our product candidates, including KRRO-110, have demonstrated a uniform distribution within the liver, including in NHPs. We believe this will allow our therapy to restore production of normal AAT protein in every cell, reducing reliance on the chance of a survival benefit for edited cells as in DNA editing.
- **Efficient delivery using a proven LNP:** AAT is the fifth most abundant protein in circulation, with a large number of transcripts that require editing to achieve expression of normal protein. LNPs provide a significant benefit to delivery of RNA-editing oligonucleotides by increasing the likelihood of sufficient distribution into liver cells. In March 2023, we entered into an agreement with Genevant Sciences GmbH, or Genevant, a well established leader in the LNP space, to provide access to clinically validated LNP technology to optimize delivery of KRRO-110. Preclinical studies of this LNP delivery technology have shown improved dose-dependent efficacy with reduced clinical chemistry and adverse events. We are pursuing development of KRRO-110 with an optimized LNP from Genevant (GVT-1) for delivery.

KRRO-110

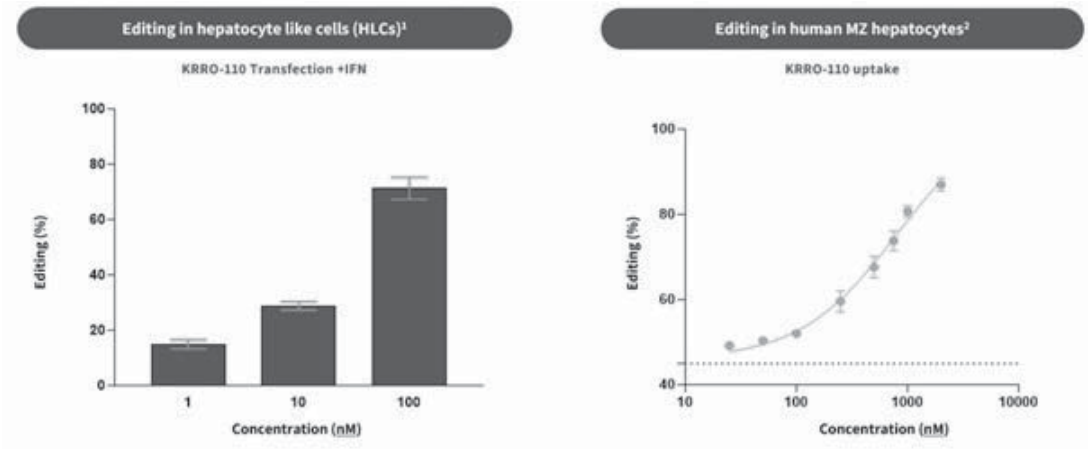
Summary of our preclinical studies and data generated to date leading to KRRO-110 nomination

We have generated highly compelling preclinical data that forms the basis for our proof of mechanism. We have affirmed that multiple disease modifying early generation product candidates have demonstrated proof-of-concept in *in vivo* studies leading to the nomination of KRRO-110 as a development candidate.

Earlier generation product candidates have independently achieved clinically meaningful expression of normal AAT protein consistent with a PiMZ genotype within preclinical *in vivo* animal models while using clinically relevant doses administered on a weekly or biweekly basis. Given that human protein half-life is much longer than other species, we believe our therapies will support a longer dose interval in the clinic. We have initiated preclinical dose-limiting toxicity safety studies and nominated our first development candidate, KRRO-110, in December 2023.

In vitro activity in human cells: Our initial design iterations for RNA-editing oligonucleotides were conducted in *in vitro* human systems, specifically containing human ADAR and human SERPINA1 genes with a Z allele. By leveraging the capabilities of our OPERA platform, we have generated multiple product candidates that achieved increased editing activity in human *in vitro* systems.

In Figure 14 below, we were able to demonstrate greater than 50% editing efficiency at the E342K mutation within stem cell derived hepatocyte like cells, or HLCs, with KRRO-110. HLCs harbor both alleles, an important quality that has the potential to be predictive of function *in vivo*. We also demonstrated editing in MZ primary human hepatocytes, or PHH, which harbor a single Z allele.



1 HLCs derived from ZZ patient, transfected with RNAiMAX with 1U/uL of IFN, editing measured 48-hours post transfection via amplicon-seq
2 Primary hepatocytes from MZ donor, free-uptake of LNP, editing measured 48-hours post uptake via amplicon-seq

Figure 14. KRRO-110 demonstrated >50% editing in in vitro systems with the Z genotype

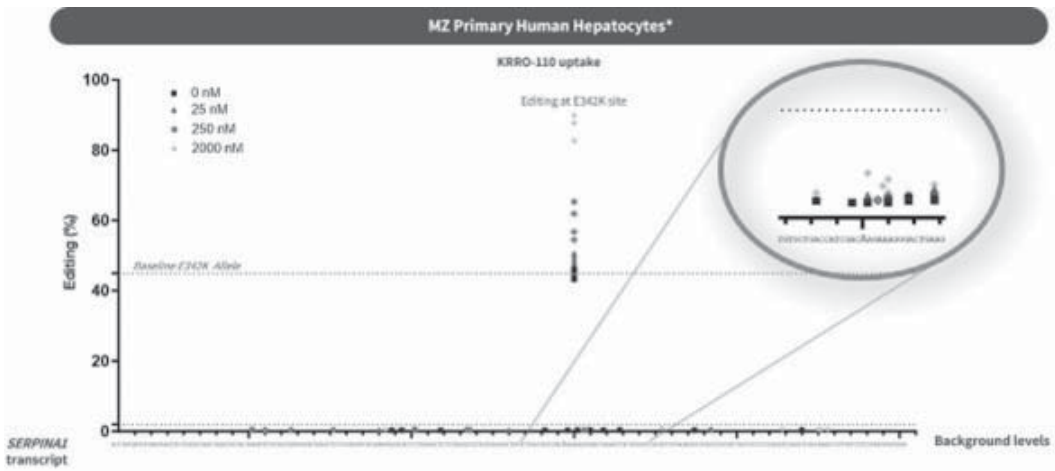


Figure 15. Negligible in vitro cis off-target editing observed for KRRO-110 in MZ hepatocytes

KRRO-110 demonstrates high selectivity for editing at the E342K RNA site, as shown in Figure 15. No off-target editing was observed in MZ primary human hepatocytes assessed at 100 base sites above and below the E342K site, against background levels. A concentration gradient is observed with a dose-response at the E342K edit site.

Preclinical in vivo activity in mice with KRRO-110: To assess and differentiate our development candidate, KRRO-110, in a mouse model used widely in the AATD field, we used a human transgenic NSG-PiZ mouse. The NSG-PiZ transgenic mouse expresses the human SERPINA1 gene with the Z-mutation. As detailed in Figures 16 and 17 below, intravenous administration of KRRO-110 at 2 mg/kg resulted in >50% editing and secretion of approximately 50µM AAT as early as seven days post-single dose in the NSG-PiZ mouse model. Increase in AAT protein and the inhibition of elastase activity were sustained through week nine when dosed every two weeks, demonstrating durability in mice.

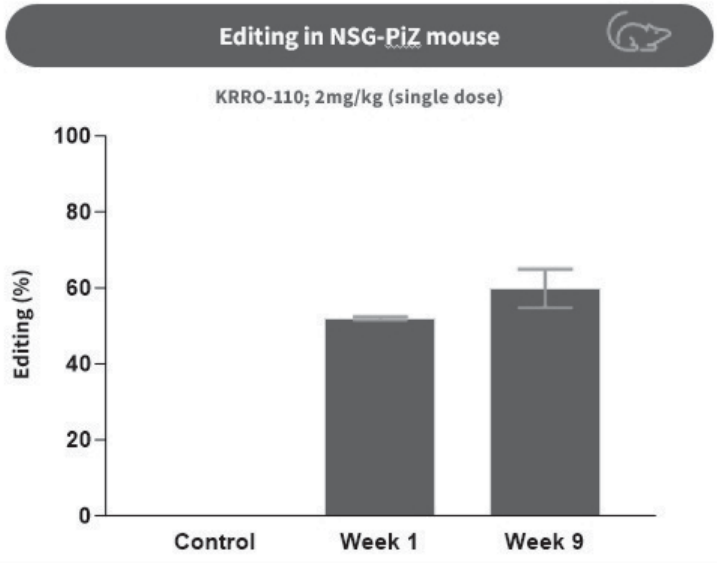


Figure 16. Achieved >50% editing in human transgenic mouse model of Z genotype with a single dose

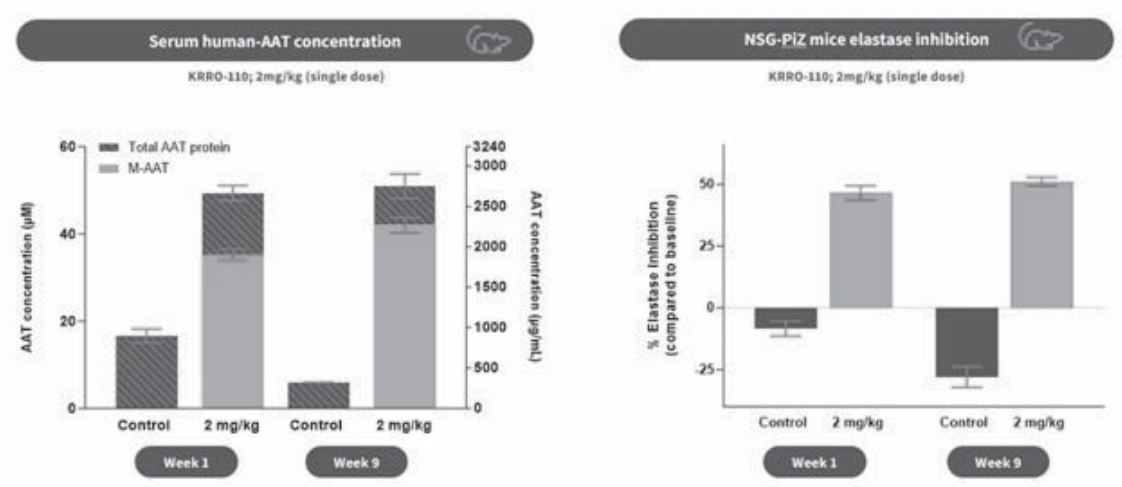


Figure 17. Secretion of functional AAT (approximately 50uM) as early as 7 days post-single dose

Preclinical *in vivo* activity in mice with earlier generation oligonucleotides: Our screening preclinical pharmacology studies have been conducted using a second C57BL/6-PiZ mouse model and licensed by us from Dr. Jeff Teckman's laboratory at St. Louis University School of Medicine. Dr. Teckman is the preeminent expert on the study of AATD. The C57BL/6-PiZ transgenic mouse model also expresses the human SERPINA1 gene containing the Z mutation.

As part of our preclinical studies, we have evaluated multiple oligonucleotides in varying doses in PiZ mice. KB-0794, an earlier generation oligonucleotide, demonstrated editing efficiency as high as 63% on day four following the administration of a single 3mg/kg dose. Normal M-AAT protein was observed at an 18 μM concentration on day four, demonstrating the potential relationship between editing efficiency and secretion of normal AAT. Upon observing the high editing efficiency of KB-0794, we proceeded to conduct an additional preclinical *in vivo* study where four groups of mice were given a lower dose of 2mg/kg and observed in a multi-dose study that lasted up to four weeks. Key takeaways from the multi-dose study included the following:

- Achieved up to 54% editing efficiency with up to 20uM of normal AAT, constituting 72% of total protein in circulation
- Liver polymers associated with the dysfunctional AAT protein were reduced at day 28, pointing to a potential to provide liver benefit through clearing of aggregates

The half-life of AAT in mice and NHPs has been observed to be around 1-2 days and 2-4 days, respectively. Human AAT has a longer half-life of 4-6 days, and when coupled with the optimization of our oligonucleotide drug product and the accumulation of drug product observed in the multi-dose preclinical studies, we believe that we will be able to achieve greater durability and a longer dosing interval in the clinic than the current once weekly or biweekly interval conducted in our preclinical studies.

Our preclinical screening studies to identify product candidates were originally conducted using an LNP delivery vehicle comprising lipids used in the approved product ONPATTRO®. Over the past decade, the field of LNP delivery has made advancements as measured by safety profile and tolerability. Our product candidates, when combined with current generation LNP delivery technology from Genevant, have demonstrated optimized editing efficiency, safety and tolerability.

In a preclinical study to compare the editing efficiency of a previous generation LNP (the comparator LNP) and current generation Genevant LNPs (GVT-1 and GVT-2), we evaluated C57BL/6-PiZ mice after receiving a single 2mg/kg dose of KB-0794 via each of the three delivery vehicles. As detailed below in Figure 18, GVT-1 and GVT-2 achieved comparable or higher editing of 37% and 65%, respectively, as compared to 29% editing for the comparator LNP. Post a single LNP dose, the percentage of normal-AAT increased to 66% for GVT-1 and 85% for GVT-2, compared to 56% for the comparator LNP, showing potential for disease-modifying effects.

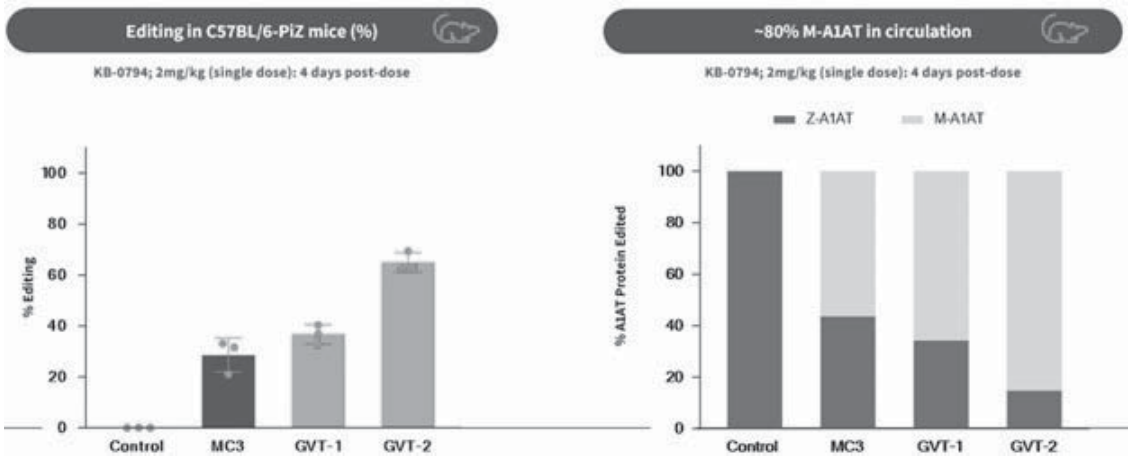


Figure 18. Comparison of editing efficiency and circulating normal protein in C57BL/6-PiZ mice between MC3 LNP and current generation Genevant LNPs (GVT-1 and GVT-2)

Nomination of KRRO-110

The above data demonstrates the highly compelling preclinical data that forms the basis for our proof of mechanism and therapeutic approach. Additionally, we have generated non-GLP toxicology data and affirmed that multiple disease modifying early generation product candidates have demonstrated proof-of-concept in in vivo studies. The complete data set has led to the nomination of KRRO-110 as a development candidate.

Translation to NHPs

In addition to our preclinical in vivo studies conducted using the PiZ mouse models described above, we have also generated a translational model to validate the potential for delivery and ability to edit the SERPINA1 gene in NHPs with an

earlier generation oligonucleotide. Because the human SERPINA1 gene in NHPs does not harbor the E342K mutation, we are demonstrating our oligonucleotide's ability to edit within the coding region of the SERPINA1 gene and the ability to translate that preclinical in vivo editing from the PiZ mouse model to NHPs.

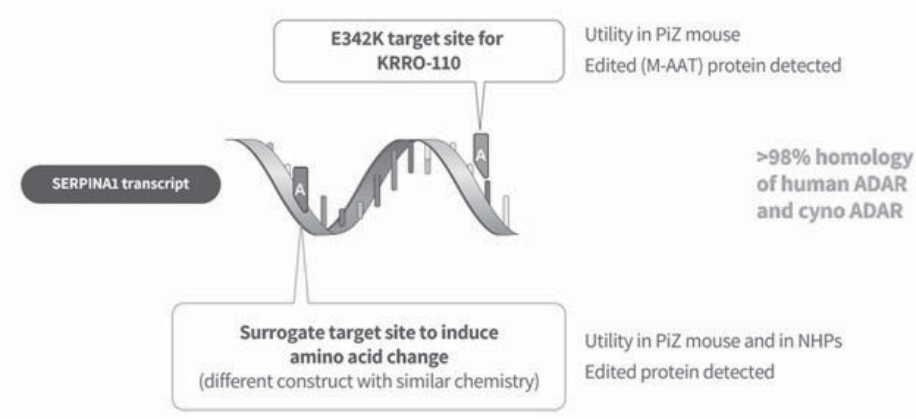


Figure 19. Editing de novo adenosine on cyno SERPINA1 to elucidate editing in higher species

We evaluated the potential editing efficiency of GVT-1 and GVT-2 for the SERPINA1 coding region in NHPs. As detailed in Figure 20 below, the observed editing rate of GVT-2 was meaningfully higher at 34%, relative to 13% in the historical MC3 study. ALT levels, a measure of safety and tolerability, were meaningfully lower in GVT-1 and on par between GVT-2 and the LNP comparator. These results show that our product candidates, when combined with current generation Genevant LNPs, can demonstrate a desirable safety profile while increasing the editing efficiency.

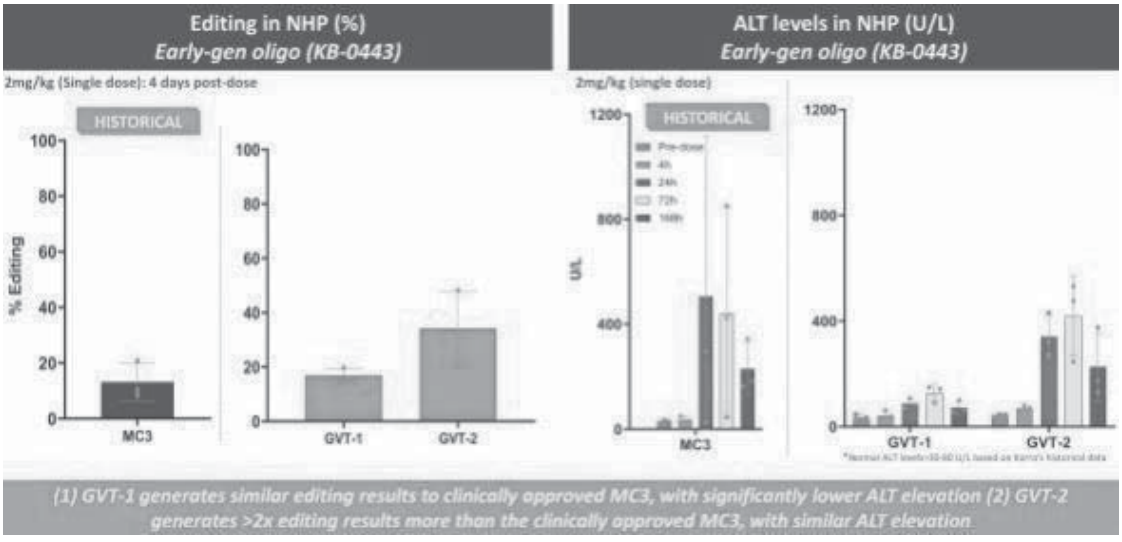


Figure 20. Comparison of editing translation in NHPs between MC3 LNP and current generation Genevant LNPs (GVT-1 and GVT-2)

Optimization of our NHP surrogate led to KB-1494 formulated in the current generation Genevant LNP (GVT-1). Upon receiving a 2mg/kg intravenous dose, liver editing was measured 4-5 days and 14-15 days after the initial dose, in mice and NHPs, respectively. As detailed in Figure 21, the observed editing rate of GVT-1 in NHPs was meaningfully higher at greater than 40%, relative to 26% observed in mice, and sustained for approximately two weeks. These results further illustrate the translation of RNA editing across mouse and NHP preclinical species.

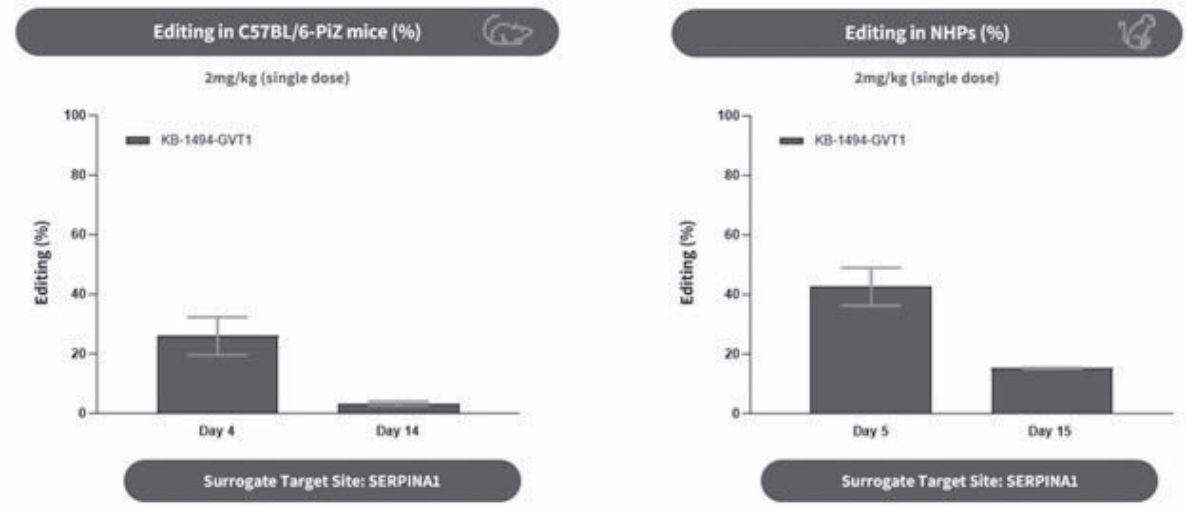


Figure 21. Editing of SERPINA1 coding region in NHPs and C57BL/6-PiZ model showed correlation

Next Steps

Preclinical development of KRRO-110 is ongoing in preparation for a potential regulatory filing in the second half of 2024 to enable the initiation of human clinical studies, with an anticipated interim clinical readout in the second half of 2025.

Our Parkinson's Disease Program: Repairing Pathogenic Variants

We are developing proprietary oligonucleotides that address the leucine-rich repeat kinase 2, or LRRK2. mutation for Parkinson's Disease, or PD, patients. This is the second program that leverages our ability to generate product candidates to repair pathogenic variants, similar to the AATD program.

Parkinson's Disease:

PD is a complex, multifactorial progressive disease that is caused, in part, by the loss of dopaminergic neurons in a structure of the brain called the substantia nigra, which is essential for the proper control of the body's movement. Approximately 10% of PD cases are attributed to inherited genetic mutations, while the remaining cases are considered idiopathic or sporadic. Mutations in *LRRK2* are the most common genetic cause of PD and increasing evidence also provides support for a role of LRRK2 in idiopathic PD. PD is the second most common neurodegenerative disease with approximately 1.0 million people in the United States diagnosed. Despite the large commercial market opportunity, there remains significant unmet need as there is no cure, and current available therapies only relieve the symptoms of PD.

LRRK2 is linked to several cellular processes including mitochondrial function, endocytosis, vesicle trafficking, and the lysosomal autophagy pathway. Additionally, LRRK2 is implicated in regulating cytokine levels and neuroinflammation. There are various mutations in LRRK2 that can result in PD, the most common of which is the pathogenic G2019S mutation that accounts for 1-6% of sporadic and 3-19% of familial PD cases.

Our Differentiated Approach and Results

Our approach is to make a single base edit to repair the protein caused by the G2019S mutation in LRRK2, which is expected to result in returning activity to the normal physiological state. We believe this change may result in disease modification.

As shown in Figure 22 below, our preliminary screening process in heterozygous LRRK2 G2019S patient-derived fibroblasts identified several product candidates that achieved >80% editing in *LRRK2* compared to only 47% in controls. The 47% represents half the transcripts having the mutation at baseline.

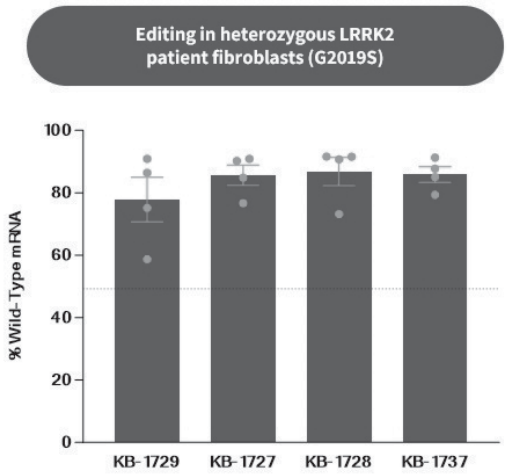


Figure 22. Editing of the G2019S mutation LRRK2 using our product candidates (100 nM)

Next Steps

We are currently screening additional oligonucleotide designs and optimizing *in vitro* assays to correlate LRRK2 editing with downstream functional endpoints. Additionally, we plan to evaluate our product candidates in a LRRK2 G2019S humanized mouse model.

Our Severe Alcohol-Associated Hepatitis Program: Disrupting Protein-Protein Interactions

Another of our programs is focused on the treatment of severe alcohol-associated hepatitis, or sAH, and demonstrates the versatility of our platform to modulate proteins. This program leverages our RNA editing technology to modulate the activity of a naturally occurring protein by disrupting protein-protein interactions. We are selectively modulating a protein transcription factor implicated in the disease pathophysiology for sAH. We believe that this approach enables the synthesis of a protein variant that disrupts interaction with our inhibitor and as a result, will be free to express downstream target genes. sAH patients with higher levels of expression of these downstream target genes have been shown to have better prognosis in a prior study examining liver biopsies at the time of diagnosis.

Severe Alcohol-Associated Hepatitis

The burden of alcohol use and alcohol use disorders contributes significantly to the health care costs for alcohol-related diseases. These patients incur direct costs to the health care system for medical care, and indirect costs to society due to a loss of workforce productivity, absenteeism, injury, early retirement and mortality. Alcohol overconsumption can lead to the development of liver damage that can manifest as fatty liver disease, alcohol-associated hepatitis and cirrhosis. The amount of alcohol intake that puts an individual at risk for alcohol-associated hepatitis is not known, but most patients have a history of heavy alcohol use for two or more decades. It is estimated that two million people die of liver disease each year, and up to half of these cases are due in part to alcohol overconsumption. There are around 300,000 hospitalizations per year for sAH in the United States. sAH is an acute condition with a mortality rate of 25%—45% within 90 days of hospitalization.

There are no FDA-approved treatments for sAH. Prednisolone is used off-label in this setting and is the only treatment available. However, many patients fail to respond to prednisolone or are contraindicated, and studies have failed to show survival benefit at 90 days. Physicians may also prescribe pentoxifylline, an anti-inflammatory, or N-acetyl cysteine, an antioxidant, but the benefit of these drugs for sAH is not well established. Additionally, some sAH patients may be candidates for a liver transplant. It has been documented that survival for sAH patients is mainly driven by liver injury and not significantly impacted by alcohol-relapse. Current strategies to address harmful alcohol use and the development of pharmacotherapies remain largely ineffective, leading to substantial unmet need for advancement of policy efforts and development of novel therapies with effective mechanisms of action.

Our Differentiated Approach and Results

We are developing a product candidate that increases expression of a transcription factor (TFX) implicated in sAH. By selectively modifying a single amino acid, we are able to disrupt interactions between the target protein and our inhibitor, as depicted in Figure 23 below.

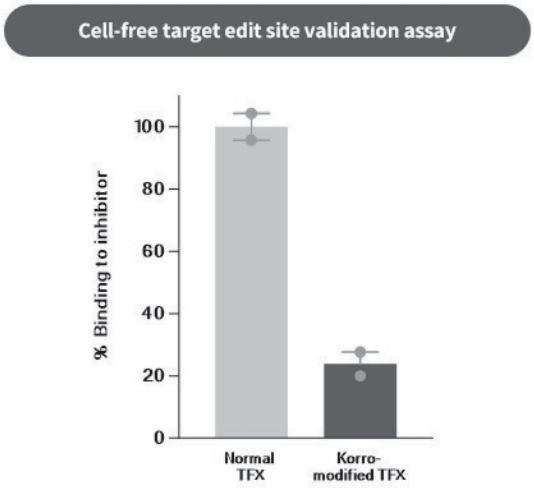
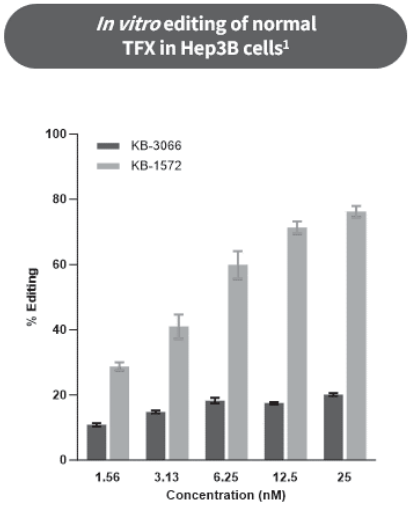


Figure 23. Changing a single amino acid disrupts binding of a transcription factor to its inhibitor

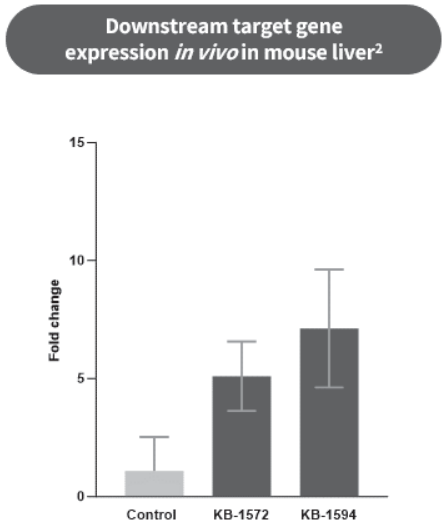
We have generated preclinical data for our sAH product candidates, demonstrating target editing and activation of target transcription factor activity in both preclinical *in vitro* and *in vivo* studies. *In vitro*, our oligonucleotides dose-dependently edited the target gene mRNA in human liver cells reaching > 70% editing efficiency as shown in Figure 24 below.



[1] *Hep3B cells transfected with RNAiMAX at the indicated concentrations with two targeting oligos, editing measured 48-hours post transfection via amplicon-seq*

Figure 24. Our product candidate edits transcription factor RNA at the validated target site in a dose-responsive manner

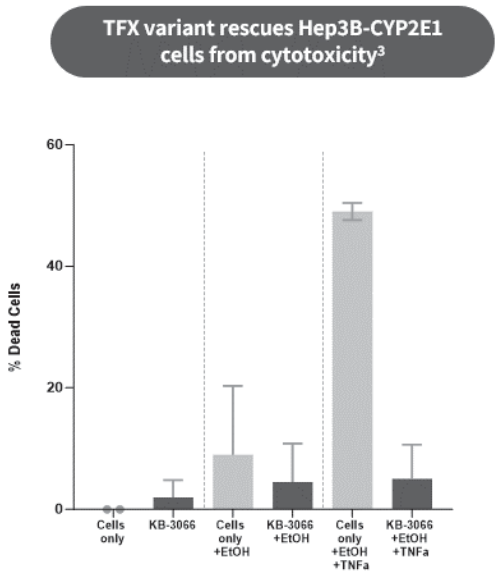
In vivo, our product candidates edited the target mRNA and induced expression of downstream target genes by up to 7x in mouse liver tissue as shown in Figure 25 below.



[2] *Wild type mice dosed with LNP-targeting oligos at a concentration of 3 mg/kg, gene expression measured via quantitative PCR from liver harvested 1 day post dose*

Figure 25. Hyperactive variant of transcription factor increased expression of downstream gene up to 7x in vivo

The product candidate has also demonstrated activity in an *in vitro* model of sAH, showing protection against cytotoxicity induced by alcohol and TNF-alpha in human liver cells overexpressing CYP2E1, the enzyme that metabolizes alcohol as shown in Figure 26 below.



[3] *Hep3B-CYP2E1 cells transfected with RNAiMAX at the indicated concentrations with two targeting oligos, cell viability measured 48-hours post transfection via CellTiter-Fluor Cell Viability Assay from Promega*

Figure 26. RNA editing demonstrates increased viability of liver cells in an in vitro model of sAH

Additionally, as seen in Figure 27 below, the product candidate demonstrated activation of a transcription factor by creating a de novo protein variant resulting in sustained downstream activity in NHPs lasting longer than 21 days, demonstrating potential to preserve transcription factor function, and increase durability of its related protein variant.

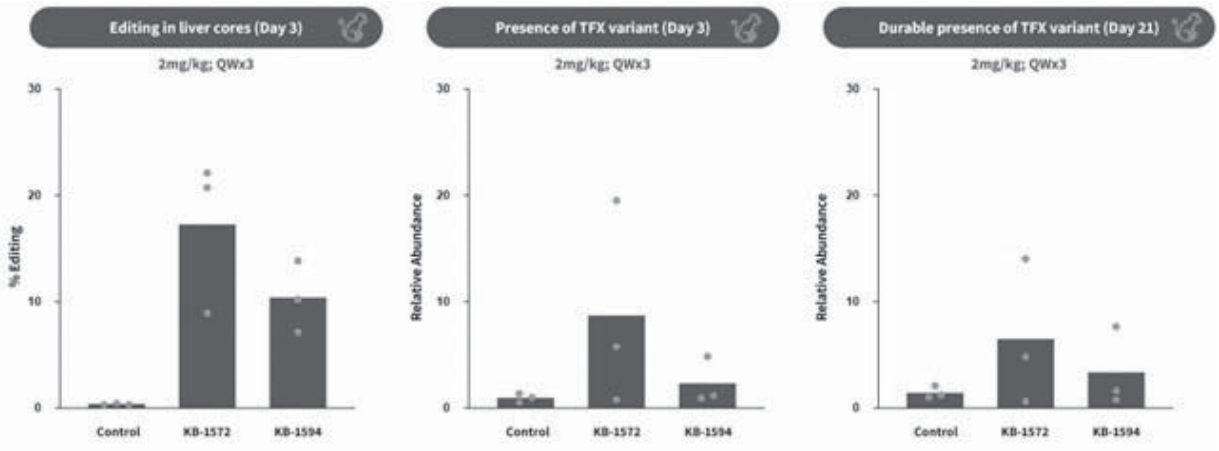


Figure 27. Sustained downstream activity in NHPs lasting longer than 21 days

Next Steps

We are currently evaluating additional oligonucleotide designs and conducting preclinical studies to confirm that our product candidates will ameliorate disease phenotype in models of alcohol-induced hepatitis. We also plan to evaluate our approach in NHP studies.

Our Amyotrophic Lateral Sclerosis Program: Disrupting Protein Aggregation

We are developing proprietary oligonucleotides targeting the mRNA for TAR DNA binding protein 43, or TDP-43, a protein associated with the etiology of amyotrophic lateral sclerosis, or ALS.

Amyotrophic Lateral Sclerosis

ALS is an adult-onset, progressive, and fatal neurodegenerative disorder that causes muscle weakness, paralysis, and ultimately death. The majority of ALS patients die from respiratory failure within three to five years after symptom appearance, with a small percentage of patients surviving beyond 10 years. Despite being classified as a rare disease by the FDA and the EMA, ALS is considered one of the more common neurodegenerative diseases worldwide. Prevalence estimates vary, but it is widely accepted that there are at least an estimated 25,000 ALS patients in the United States. There is currently no cure for ALS, and currently approved therapies either only provide symptomatic relief or slow the overall progression of the disease.

Our Differentiated Approach and Results

Our approach is to selectively modulate TDP-43, an RNA/DNA-binding protein, which carries out a variety of important functions in healthy neurons, including initiation of transcription, pre-mRNA splicing and miRNA processing. Hyper-phosphorylated and ubiquitinated TDP-43 deposits form inclusion bodies in the brain and spinal cord of patients with ALS and frontotemporal dementia, or FTD. The majority of ALS and FTD cases are sporadic, and more than 90% and 45% of ALS and FTD patients, respectively, have TDP-43 aggregations in neurons. Less than 10% of ALS cases are familial, and mutations in *TARDBP*, the gene encoding TDP-43, are responsible for approximately 4% of familial ALS. Given the importance of TDP-43's role in maintaining healthy neurons, the generation of a protein variant with the desired non-aggregating property could potentially have therapeutic benefit for the majority of ALS and FTD patients. We believe that by leveraging the ability of RNA editing to affect a single base edit in *TARDBP*, we can lead to the synthesis of a TDP-43 protein variant that does not aggregate, thereby restoring our normal function.

We have created a series of TDP-43 variants that contain single amino acid changes designed to alter post-translational modification by phosphorylation, ubiquitination, acetylation or cleavage with the intent of reducing the ability to aggregate while maintaining function in RNA metabolism. We believe that modulating TDP-43 through the introduction of specific amino acid changes into TDP-43 mRNA sequence is preferable to other approaches that try to address protein aggregates after they form, to non-specifically prevent stress granule formation, or to target a single TDP-43 downstream target. We have engineered mutations amenable to an RNA edit using our OPERA platform that limit the formation of TDP-43 inclusion bodies *in vitro*. Additionally, we have demonstrated meaningful editing of TDP-43 targets sites with our product candidates in a human neuroblastoma-derived cell line (SK-N-AS) as demonstrated in Figure 28 below.

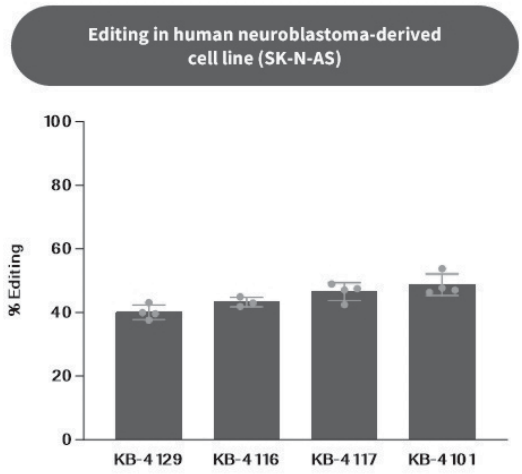


Figure 28. Editing of TDP-43 mRNA using our product candidates (100 nM)

We intend to initially pursue ALS with this approach and has the opportunity to expand our pipeline to other neurodegenerative diseases, such as FTD.

Next Steps

We are continuing to design and screen additional oligonucleotides in SK-N-AS cells to identify proprietary oligonucleotides for further evaluation in aggregation assays. Furthermore, we are identifying and characterizing ALS cell lines including genetic-induced models and patient cell lines, to test the efficacy of TDP-43 protein variants in disease models.

Our Pain Program: Selective Modulation of Ion Channels

We are developing proprietary oligonucleotides that selectively modulate ion channels associated with pain.

Overview of Pain Indications

Pain is a condition that millions of patients experience and is often a component of rare and highly prevalent diseases. Pain can be generally classified as either acute or chronic and can be further segmented into subcategories including nociceptive or neuropathic pain. There are several classes of therapeutics to target the numerous pathways that cause pain, including opioids, nerve growth factors and ion channel blockers. Many of these treatment methods involve non-specific targeting of pathways that lead to off-target effects. Despite a large commercial market opportunity, there remains significant unmet needs for safe and effective pain management, including non-opioid therapeutics.

Several classes of drugs, including local anesthetics, such as lidocaine, are ion channel blockers, although they do not show a high degree of specificity and thus inhibit many types of sodium channels rather than selectively blocking $Na_V1.7$. No highly selective small molecule product candidates have been FDA-approved as therapeutics. One of the challenges in developing a small molecule inhibitor of $Na_V1.7$ is the high degree of homology with other voltage gated sodium channels, inhibition of which has been linked to safety concerns.

Our Differentiated Approach and Results

The introduction of genetic changes into the mRNA encoding $Na_V1.7$ demonstrates the potential of RNA editing to create highly differentiated and selective therapeutics for ion channels. $Na_V1.7$ is a voltage-gated sodium channel that plays a critical

role in the generation and conduction of action potentials and is thus important for electrical signaling in the nervous system. $Na_V1.7$ is highly expressed in the pain sensing dorsal root ganglion neuron. Genetic inactivation of *SCN9A*, the gene encoding $Na_V1.7$, in mice results in the inability to sense pain from inflammatory stimuli. In humans, mutations that lead to inactivation of $Na_V1.7$ function result in a genetic condition known as Channelopathy-associated insensitivity to pain, or CIP. Individuals with CIP have severely diminished ability to sense pain. By contrast, mutations that activate $Na_V1.7$ result in intense pain.

Through our RNA editing technology, we have generated a series of site-specific changes in $Na_V1.7$ that modulate our ion channel function such that it mimics a small molecule sodium channel blocker. We believe that using RNA editing to introduce these changes in patients has the potential to deliver potent analgesic activity without the dose-limiting toxicities that have been observed by other sodium channel blockers.

We have demonstrated that rationally designed single amino acid changes to unique target sites are sufficient to decrease the activity of $Na_V1.7$. Electrophysiology studies performed in CHO cells transfected with plasmids expressing channel variants demonstrated biophysical properties that are associated with a decrease in $Na_V1.7$ channel activity compared to fully functional $Na_V1.7$. Additionally, we have demonstrated meaningful editing for these target sites in SK-N-AS cells as shown in Figure 29 below.

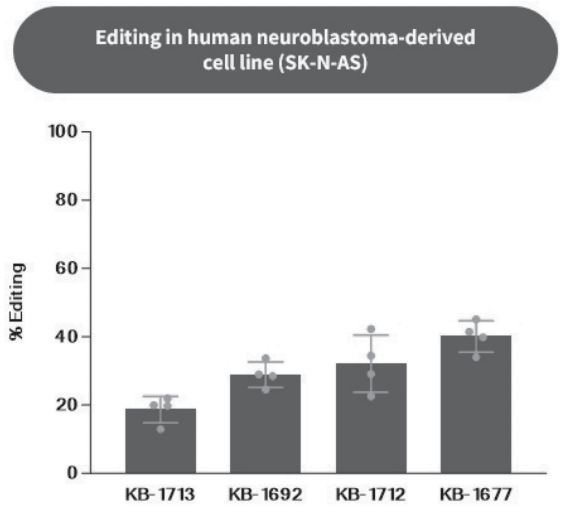


Figure 29. Editing of $Na_V1.7$ using our product candidates (100nM)

Next Steps

We are optimizing and screening our proprietary oligonucleotides in electrophysiology assays. Furthermore, we will perform a high throughput screen of $Na_V1.7$ variants to identify potential novel ion channel variants with enhanced crippling of electrophysiology activity.

Our Cardiometabolic Disease Program: Activating Kinases

We are developing proprietary oligonucleotides that activate a kinase involved in cardiometabolic disease. Cardiometabolic disease encompasses a broad range of complex, multifactorial diseases including cardiovascular disease, diabetes, chronic renal failure and obesity, among others. A number of validated cardiometabolic disease targets such as kinases have been identified but have historically been difficult to drug.

We have generated a site-specific change in a validated kinase that is a central regulator of energy homeostasis. In an *in vitro* mutagenesis study, a site-specific change led to activation of the kinase and an increase in the phosphorylation of its downstream target. We believe that using RNA editing to introduce these changes with an oligonucleotide in patients has the potential to deliver efficacy that has been unattainable when using other modalities.

Pioneering RNA Editing to Deliver the Future of Medicine

Each of our programs demonstrate the versatility of the ADAR-mediated RNA editing approach. Importantly, we are able to not only address classes of diseases caused by deleterious effects of misfolded or misdirected proteins, but we can also potentially utilize genetics to identify highly prevalent diseases where therapeutic benefit can be generated through alteration of

protein function or expression. We will continue to selectively identify and pursue additional targets and indications based on a range of technical, clinical, and commercial factors to build a robust and differentiated pipeline. However, RNA editing is a novel technology that is not yet clinically validated for human therapeutic use. The approaches we take to discover and develop novel therapeutics are unproven and may never lead to marketable products. We are not aware of any clinical trials for safety or efficacy having been completed by any third party using RNA editing and nor are we aware of any RNA editing therapeutic product that has been approved in the United States or Europe. It will be many years before we commercialize a product candidate, if ever.

Manufacturing and Supply Arrangements

We currently have no commercial manufacturing capabilities. For our initial wave of clinical programs, we intend to use qualified third-party CMOs with relevant manufacturing experience in genetic medicines. we plan to partner with suppliers and CMOs to produce or process critical raw materials, bulk compounds, formulated compounds, viral vectors or engineered cells for IND-supporting activities and early-stage clinical trials. At the appropriate time in the product development process, we will determine whether to establish in-house GMP manufacturing capabilities for some core technologies or continue to rely on third parties to manufacture commercial quantities for any products that we may successfully develop.

We also in license technology for our fit-for-purpose delivery systems, including LNP delivery systems. For example, in March 2023, we entered into a collaboration and license agreement with Genevant, a well established leader in the LNP space, to provide access to clinically validated LNP technology to optimize delivery of our AATD product candidate, KRRO-110. Preclinical studies of this LNP delivery technology have shown improved dose-dependent efficacy with reduced clinical chemistry and adverse events. For additional information relating to the financial terms of such agreement, see Note 13 to our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Competition

The pharmaceutical and biotechnology industries, including the gene therapy and gene editing fields, are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property. While we believe that our differentiated technology, scientific expertise, and intellectual property position provide us with competitive advantages, we face potential competition from a variety of companies in these fields. There are several companies using synthetic oligonucleotide or base editing technology, including Beam Therapeutics, Verve Therapeutics, Prime Medicine, ProQR, and Wave Life Sciences. Several additional companies utilize other editing technologies, including Edigene and Shape Therapeutics. In addition, we face competition from companies utilizing gene therapy, oligonucleotides, and DNA editing technologies such as base and prime editing.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates. This may include other types of therapies, such as small molecule, antibody, and/or protein therapies.

In addition, many of our current or potential competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials and approved products than we do today. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. We also compete with these companies in recruiting, hiring and retaining qualified scientific and management talent, establishing clinical trial sites and patient registration for clinical trials, obtaining manufacturing slots at contract manufacturing organizations, and in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, particularly if they represent cures, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. The key competitive factors affecting the success of all of our programs are likely to be their efficacy, safety, convenience, and availability of reimbursement.

Intellectual Property

Overview

We strive to protect the proprietary technology that we believe is important to our business, including seeking and maintaining patent protection in the United States and internationally for our current and future product candidates. We also rely

on trademarks, copyrights, trade secrets, confidentiality procedures, employee disclosure, invention assignment agreements, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position.

We seek to obtain domestic and international patent protection, and endeavors to promptly file patent applications for new commercially valuable inventions. We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

We plan to continue to expand our intellectual property estate by filing patent applications directed to platform technologies and improvements thereof, pharmaceutical compositions, methods of treatment, methods of manufacture or identified from our ongoing development of our product candidates. Our success will depend on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce any patents that we may obtain, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and proprietary rights of third parties.

The patent positions of companies like us are generally uncertain and involve complex legal, scientific and factual questions. In addition, the coverage claimed in a patent may be challenged in courts after issuance. Moreover, many jurisdictions permit third parties to challenge issued patents in administrative proceedings, which may result in further narrowing or even cancellation of patent claims. We cannot guarantee that our pending patent applications, or any patent applications that we may in the future file or license from third parties, will result in the issuance of patents. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or at all, whether the claims of any patent applications, should they issue, will cover our product candidates, or whether the claims of any issued patents will provide sufficient protection from competitors or otherwise provide any competitive advantage. We cannot predict the scope of claims that may be allowed or enforced in our patents. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Consequently, we may not obtain or maintain adequate patent protection for any of our product candidates.

Because patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months or potentially even longer, and because publication of discoveries in the scientific or patent literature often lags behind actual discoveries and patent application filings, we cannot be certain of the priority of inventions covered by pending patent applications. Accordingly, we may not have been the first to invent the subject matter disclosed in some of our patent applications or the first to file patent applications covering such subject matter, and we may have to participate in interference proceedings or derivation proceedings declared by the USPTO to determine priority of invention. For more information regarding the risks related to our intellectual property, see Item 1A "*Risk Factors—Risks Related to Our Business—Risks Related to Intellectual Property*."

Patent Portfolio

We strive to protect our proprietary RNA editing platform OPERA and related technologies and our product candidates, including seeking and maintaining patent protection intended to cover various target-specific editing strategies, the composition of matter of our product candidates, their methods of use, related delivery technologies, and other inventions. The intellectual property that is available to us is critical to our business and we strive to protect it, including by obtaining, maintaining, defending, and enforcing patent protection in the United States and internationally. As of December 31, 2023, our patent portfolio in total consisted of 32 patent families, with two U.S. patents and one patent in foreign jurisdictions (e.g., Canada), including five pending Patent Cooperation Treaty, or PCT, applications, various pending non-provisional applications world-wide (e.g., United States, Australia, Canada, China, Europe, South Korea, and Japan), and nine families with pending provisional patent applications.

We have a patent portfolio that relates to our RNA editing platform OPERA, as well as numerous disease programs listed below, and includes 13 patent families. These families are directed to various oligonucleotide formats, nucleotide compositions, oligonucleotide chemistries, modifications, specific linkage chemistries, oligonucleotides having a specific structures, methods of deaminating an adenosine using such oligonucleotides, methods of oligonucleotide delivery, and methods of treating disease by administering such oligonucleotides. The first patent family is pending in Australia, Canada, China, Europe, Japan, South Korea, Taiwan and the United States, and includes a U.S. patent. The first three patent families are pending in Australia, Canada, China, Europe, Hong Kong, Japan, South Korea, Taiwan and the United States, and include two U.S. patents. U.S. Patent No. 11,479,575 is directed to specific oligonucleotide structures and expires in 2040; U.S. Patent No. 11,453,878 is directed to methods of deamination of an adenosine in an mRNA using oligonucleotide with specific structures and also expires in 2040. Any other patents issuing from applications in these families will expire in 2040, absent any available additional term for patent term extension or patent term adjustment. The fourth patent family is pending in the United States and patents issuing from applications in this family will expire in 2042, absent any available additional term for patent term extension or patent term adjustment. The fifth, sixth, seventh and eighth patent families have been filed as PCT applications, and if issued, patents in these families would expire between 2042 and 2043, absent any available additional term for patent term extension or patent term

adjustment. The ninth, tenth, eleventh, twelfth and thirteenth patent families have been filed as provisional patent applications, and if re-filed as PCT or non-provisional applications, and issued, patents in this family would expire in 2044, absent any available additional term for patent term extension or patent term adjustment.

In addition to the patent families related to our OPERA platform technology described above, our patent portfolio that relates to our AAT program includes two patent families. These patent families are directed to specific oligonucleotides that target SERPINA1 for editing to treat AAT. The first patent family includes pending applications in Australia, Canada, China, Europe, Japan, South Korea and the United States. Patents issuing from applications in this family will expire in 2041, absent any available additional term for patent term extension or patent term adjustment. The second patent family has been filed as a provisional patent application, and if re-filed as PCT or non-provisional applications, and issued, patents in this family would expire in 2044, absent any available additional term for patent term extension or patent term adjustment.

In addition to the patent families related to our OPERA platform technology described above, our patent portfolio that relates to our PD program includes one patent family. This patent family is directed to specific oligonucleotides that target LRRK2 for editing to treat PD, and includes pending applications in Europe, and the United States. Patents issuing from applications in this family will expire in 2041, absent any available additional term for patent term extension or patent term adjustment.

In addition to the patent families related to our OPERA platform technology described above, our patent portfolio that relates to our sAH program includes two patent families. This patent family is directed to specific oligonucleotides that are capable of editing a specific target associated with sAH, and consists of a pending PCT application. Patents issuing from this family of patent applications will expire in 2042, absent any available additional term for patent term extension or patent term adjustment. The second patent family has been filed as a provisional patent application, and is directed to specific oligonucleotides that are capable of editing a specific target associated with sAH. If this application is re-filed as an PCT or non-provisional application, and issued, patents in this family will expire in 2043, absent any available additional term for patent term extension or patent term adjustment.

In addition to the patent families related to our OPERA platform technology described above, our patent portfolio that relates to our ALS includes one patent family, directed to oligonucleotides that edit TDP-43. This patent family consists of two provisional patent applications, and if re-filed as PCT or non-provisional applications, and issued, patents in this family would expire in 2044, absent any available additional term for patent term extension or patent term adjustment.

In addition to the patent families described above, we also have other patent families directed to additional target-specific editing strategies, oligonucleotide compositions and their methods of use, related delivery technologies, and other inventions related to early-stage research and development efforts not reflected in our pipeline. Patents issued from or issuing from applications in these families will expire between 2041 and 2044, absent any available additional term for patent term extension or patent term adjustment, and includes Canadian Patent No. 3,162,416, which will expire in 2042. We also have legacy patents related to our pre-Merger operations.

Patent Term

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, including the United States, the base term is 20 years from the filing date of the earliest-filed non-provisional patent application from which the patent claims priority. The term of a U.S. patent can be lengthened by patent term adjustment, which compensates the owner of the patent for administrative delays at the USPTO. In some cases, the term of a U.S. patent is shortened by terminal disclaimer that reduces its term to that of an earlier-expiring patent. The term of a U.S. patent may be eligible for patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act, to account for at least some of the time the drug is under development and regulatory review after the patent is granted. With regard to a drug for which FDA approval is the first permitted marketing of the active ingredient, the Hatch-Waxman Act allows for extension of the term of one U.S. patent that complies with applicable FDA or other requirements at any time with respect to product development, clinical testing, approval or any other regulatory requirements relating to product manufacture, processing, handling, storage, quality control, safety, marketing, advertising, promotion, packaging, labeling, export, import, distribution, or sale.

We may become subject to administrative or judicial sanctions or other legal consequences. These sanctions or consequences could include, among other things, the FDA's refusal to approve pending applications, issuance of clinical holds for ongoing studies, suspension or revocation of approved at least one claim covering the composition of matter of such an FDA-approved drug, an FDA-approved method of treatment using the drug and/or a method of manufacturing the FDA-approved drug. The extended patent term cannot exceed the shorter of five years beyond the non-extended expiration of the patent or 14 years from the date of the FDA approval of the drug, and a patent cannot be extended more than once or for more than a single product. During the period of extension, if granted, the scope of exclusivity is limited to the approved product for approved uses. Some

foreign jurisdictions, including Europe and Japan, have analogous patent term extension provisions, which allow for extension of the term of a patent that covers a drug approved by the applicable foreign regulatory agency.

In the future, if and when our product candidates receive FDA approval, we expect to apply, if appropriate, for patent term extension on patents directed to those product candidates, their methods of use and/or methods of manufacture. However, there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extensions should be granted, and if granted, the length of such extensions. For more information regarding the risks related to our intellectual property, see Item 1A "*Risk Factors—Risks Related to Our Business—Risks Related to Intellectual Property*."

Reservation of Rights by the U.S. Government

Our in-licensed patent rights may be subject to a reservation of rights by one or more third parties, including the U.S. government. Pursuant to the Bayh-Dole Act of 1980, the U.S. government has certain rights in inventions developed with government funding. These U.S. government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. When new technologies are developed with government funding, in order to secure ownership of patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. government and timely electing title to such inventions. A failure to meet these obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications. In addition, the U.S. government has the right, under certain limited circumstances, to require the licensor to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (1) adequate steps have not been taken to commercialize the invention; (2) government action is necessary to meet public health or safety needs; or (3) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). Recent developments in regarding the Bayh-Dole Act of 1980 indicate that the march-in rights could be exercised to affect pricing.

If the U.S. government exercised its march-in rights in our future intellectual property rights that are generated through the use of U.S. government funding or grants, we could be forced to license or sublicense intellectual property that we license on terms unfavorable to us, and there can be no assurance that we would receive compensation from the U.S. government for the exercise of such rights. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology that was developed using U.S. government funding. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the U.S. government requires that any products embodying any of these inventions or produced through the use of any of these inventions be manufactured substantially in the United States. This preference for U.S. industry may be waived by the federal agency that provided the funding if the owner or assignee of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. industry may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. For more information regarding the risks related to our intellectual property, see Item 1A "*Risk Factors—Risks Related to Our Business—Risks Related to Intellectual Property*."

Trade Secrets

In addition to patents, we rely on trade secrets and know-how to develop and maintain our competitive position. We typically rely on trade secrets to protect aspects of our business that are not amenable to, or that we not consider appropriate for, patent protection. We protect trade secrets and know-how by establishing confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements provide that all confidential information developed or made known during the course of an individual or entities' relationship with us must be kept confidential during and after the relationship. These agreements also provide that all inventions resulting from work performed for us or relating to our business and conceived or completed during the period of employment or assignment, as applicable, shall be our exclusive property. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary information by third parties.

Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. For more information regarding the risks related to our intellectual property, see Item 1A "*Risk Factors—Risks Related to Our Business—Risks Related to Intellectual Property*."

Governmental Regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, clinical trial, testing, manufacture, quality control, import, export, safety, efficacy, labeling, packaging, storage, distribution, recordkeeping, approval, distribution, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of drugs. We, along with our vendors, CROs, clinical investigators and CMOs will be required to navigate the various preclinical, clinical, manufacturing and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval of our product candidates. The process of obtaining regulatory approvals of drugs and ensuring subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources.

Overview of U.S. Drugs Development Process

In the United States, the FDA regulates drug products under the FD&C Act and its implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. If we fail to comply with applicable FDA or other requirements at any time with respect to product development, clinical testing, approval or any other legal requirements relating to product manufacture, processing, handling, storage, quality control, safety, marketing, advertising, promotion, packaging, labeling, export, import, distribution, or sale, we may become subject to administrative or judicial sanctions or other legal consequences. These sanctions or consequences could include, among other things, the FDA's refusal to approve pending applications, warning or untitled letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution.

Our product candidates must be approved for therapeutic indications by the FDA before they may be marketed in the United States. For drug product candidates regulated under the FD&C Act, FDA must approve a NDA. The process generally involves the following:

- completion of extensive preclinical studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practice, or GLP, requirements and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- completion of the manufacture, under cGMP conditions, of the drug substance and drug product that the sponsor intends to use in human clinical trials along with required analytical and stability testing;
- submission to the FDA of an IND which must become effective before clinical trials may begin;
- payment of user fees for FDA review of the NDA;
- approval by an IRB or independent ethics committee at each clinical trial site before each trial may be initiated;
- performance of adequate and well-controlled clinical trials in accordance with applicable IND regulations, GCP requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- preparation and submission to the FDA of an NDA;
- a determination by the FDA within 60 days of its receipt of an NDA to file the application for review;
- satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the drug will be produced to assess compliance with cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the drug product's identity, strength, quality and purity;
- satisfactory completion of potential FDA audit of the preclinical study clinical trial sites that generated the data in support of the NDA; and
- FDA review and approval of the NDA, including, where applicable, consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug in the United States.

Preclinical Studies and Clinical Trials for Drugs

Before testing any drug in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluations of product chemistry, formulation and stability, as well as *in vitro* and animal studies to assess safety and in some cases to establish the rationale for therapeutic use. The conduct of preclinical studies is subject to federal and state regulation and requirements, including GLP requirements for safety/toxicology studies. The results of the preclinical studies, together with manufacturing information and analytical data, must be submitted to the FDA as part of an IND.

An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before clinical trials may begin. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical trials. The IND also includes the results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. Some long-term preclinical testing may continue after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions about the conduct of the clinical trial, including concerns that human research subjects will be exposed to unreasonable health risks, and imposes a full or partial clinical hold. FDA must notify the sponsor of the grounds for the hold and any identified deficiencies must be resolved before the clinical trial can begin. Submission of an IND may result in the FDA not allowing clinical trials to commence or not allowing clinical trials to commence on the terms originally specified in the IND. A clinical hold can also be imposed once a trial has already begun, thereby halting the trial until the deficiencies articulated by FDA are corrected.

The clinical stage of development involves the administration of the product candidate to healthy volunteers or patients under the supervision of qualified investigators, who generally are physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which include the requirements that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters and criteria to be used in monitoring safety and evaluating effectiveness. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trials are minimized and are reasonable compared to the anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. The FDA, the IRB, or the sponsor may suspend or discontinue a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trials to public registries. Information about clinical trials, including results for clinical trials other than Phase 1 investigations, must be submitted within specific timeframes for publication on www.ClinicalTrials.gov, a clinical trials database maintained by the National Institutes of Health.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a clinical trial may move forward at designated check points based on access that only the group maintains to available data from the trial and may recommend halting the clinical trial if it determines that the participants or patients are being exposed to an unacceptable health risk or other grounds, such as no demonstration of efficacy. Other reasons for suspension or termination may be made by us based on evolving business objectives and/or competitive climate.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, FDA will nevertheless accept the results of the study in support of an NDA if the study was well-designed and well-conducted in accordance with GCP requirements, including that the clinical trial was performed by a qualified investigator(s); the data are applicable to the U.S. population and U.S. medical practice; and the FDA is able to validate the data through an onsite inspection if deemed necessary.

Clinical trials to evaluate therapeutic indications to support NDAs for marketing approval are typically conducted in three sequential phases, which may overlap.

- *Phase 1* – Phase 1 clinical trials involve initial introduction of the investigational product in a limited population of healthy human volunteers or patients with the target disease or condition. These studies are typically designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, excretion the side effects associated with increasing doses, and, if possible, to gain early evidence of effectiveness.
- *Phase 2* – Phase 2 clinical trials typically involve administration of the investigational product to a limited patient population with a specified disease or condition to evaluate the drug's potential efficacy, to determine the optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.

- *Phase 3* – Phase 3 clinical trials typically involve administration of the investigational product to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval and physician labeling. Generally, two adequate and well-controlled Phase 3 trials are required by the FDA for approval of an NDA.

Post-approval trials, sometimes referred to as Phase 4 clinical trials or post-marketing studies, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and are commonly intended to generate additional safety data regarding use of the product in a clinical setting. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of NDA approval.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA. Written IND safety reports must be submitted to the FDA and the investigators fifteen days after the trial sponsor determines the information qualifies for reporting for serious and unexpected suspected adverse events, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human volunteers and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must also notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction as soon as possible but in no case later than seven calendar days after the sponsor's initial receipt of the information. During the development of a new drug product, sponsors have the opportunity to meet with the FDA at certain points, including prior to submission of an IND, at the end of Phase 2 and before submission of an NDA. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date and for the FDA to provide advice on the next phase of development.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing the drug product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and manufacturers must develop, among other things, methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Process for Drugs

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. An NDA is a request for approval to market a new drug for one or more specified indications and must contain proof of the drug's safety and efficacy for the requested indications. The marketing application is required to include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational drug, to the satisfaction of the FDA. FDA must approve an NDA before a drug may be marketed in the United States.

The FDA reviews all submitted NDAs to ensure they are sufficiently complete to permit substantive review before it accepts them for filing and may request additional information rather than accepting the NDA for filing. The FDA must make a decision on accepting an NDA for filing within 60 days of receipt, and such decision could include a refusal to file by the FDA. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the NDA. The FDA reviews an NDA to determine, among other things, whether the product is safe and effective for the indications sought and whether the facility in which it is manufactured, processed, packaged or held meets standards, including cGMP requirements, designed to assure and preserve the product's continued identity, strength, quality and purity. Under the goals and polices agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA targets ten months, from the filing date, in which to complete its initial review of a new molecular entity NDA and respond to the applicant, and six months from the filing date of a new molecular entity NDA for priority review. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs, and the review process is often extended by FDA requests for additional information or clarification.

Further, under PDUFA, as amended, each NDA must be accompanied by a substantial user fee. The FDA adjusts the PDUFA user fees on an annual basis. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA also may require submission of a REMS if it believes that a REMS is necessary to ensure that the benefits of the drug outweigh its risks. A REMS can include use of risk evaluation and mitigation strategies like medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, special monitoring or other risk-minimization tools.

The FDA may refer an application for a novel drug to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP and other requirements and the integrity of the clinical data submitted to the FDA.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response Letter without first conducting required inspections, testing submitted product lots, and/or reviewing proposed labeling. In issuing the Complete Response Letter, the FDA may require additional clinical or preclinical testing or recommend other actions, such as requests for additional information or clarification, that the applicant might take in order for the FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications.

Even if the FDA approves a product, depending on the specific risk(s) to be addressed it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug intended to treat a rare disease or condition, which is a disease or condition with either a patient population of fewer than 200,000 individuals in the United States, or a patient population of 200,000 or more individuals in the United States when there is no reasonable expectation that the cost of developing and making the product available in the United States for the disease or condition will be recovered from sales of the product. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, though companies developing orphan products are eligible for certain incentives, including tax credits for qualified clinical testing and user-fee waivers.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity during which the FDA may not approve any other applications to market the same therapeutic agent for the same indication, except in limited circumstances, such as a subsequent product's showing of clinical superiority over the product with orphan exclusivity or where the original applicant cannot produce sufficient quantities of product. Competitors, however, may receive approval of different therapeutic agents for the indication for which the orphan product has exclusivity or obtain approval for the same therapeutic agent for a different indication than that for which the orphan product has exclusivity. Orphan product exclusivity could block the approval of one of our products for seven years if a competitor obtains approval for the same therapeutic agent for the same indication before we do, unless we are able to demonstrate that our product is clinically superior. If an orphan designated product receives

marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity. Further, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

The FDA may further reevaluate its regulations and policies under the Orphan Drug Act. It is unclear as to how, if at all, the FDA may change the orphan drug regulations and policies in the future.

Expedited Development and Review Programs for Drugs

The FDA maintains several programs intended to facilitate and expedite development and review of new drugs to address unmet medical needs in the treatment of serious or life-threatening diseases or conditions. These programs include Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval, and the purpose of these programs is to either expedite the development or review of important new drugs to get them to patients more quickly than standard FDA review timelines typically permit.

A new drug is eligible for Fast Track designation if it is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address unmet medical needs for such disease or condition. Fast track designation applies to the combination of the product candidate and the specific indication for which it is being studied. Fast Track designation provides increased opportunities for sponsor interactions with the FDA during preclinical and clinical development, in addition to the potential for rolling review once a marketing application is filed. Rolling review means that the FDA may review portions of the marketing application before the sponsor submits the complete application.

In addition, a new drug may be eligible for Breakthrough Therapy designation if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug, alone or in combination with one or more other drugs, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Breakthrough Therapy designation provides all the features of Fast Track designation in addition to intensive guidance on an efficient product development program beginning as early as Phase 1, and FDA organizational commitment to expedited development, including involvement of senior managers and experienced review staff in a cross-disciplinary review, where appropriate.

Any product submitted to the FDA for approval, including a product with Fast Track or Breakthrough Therapy designation, may also be eligible for additional FDA programs intended to expedite the review and approval process, including Priority Review designation and Accelerated Approval. A product is eligible for Priority Review, once an NDA is submitted, if the product that is the subject of the marketing application has the potential to provide a significant improvement in safety or effectiveness in the treatment, diagnosis or prevention of a serious disease or condition. Under priority review, the FDA's goal date to take action on the marketing application is six months compared to ten months for a standard review.

Products are eligible for Accelerated Approval if they can be shown to have an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or an effect on a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, which is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Accelerated Approval is usually contingent on a sponsor's agreement to conduct, in a diligent manner, adequate and well-controlled additional post-approval confirmatory studies to verify and describe the product's clinical benefit, and under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA may require, as appropriate, that such trials be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Further, under FDORA, the FDA has increased authority for expedited procedures to withdraw approval of a product or an indication approved under Accelerated Approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, for products being considered for Accelerated Approval, the FDA generally requires, unless otherwise informed by the agency, that all advertising and promotional materials intended for dissemination or publication within 120 days of marketing approval be submitted to the agency for review during the pre-approval review period. After the 120-day period has passed, all advertising and promotional materials must be submitted at least 30 days prior to the intended time of initial dissemination or publication.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or the time period for FDA review or approval may not be shortened. Furthermore, Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval do not change the scientific or medical standards for approval or the quality of evidence necessary to support approval, though they may expedite the development or review process.

U.S. Post-Approval Requirements for Drugs

Drugs manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, reporting of adverse experiences with the product, complying with promotion and advertising requirements, which include restrictions on promoting products for unapproved uses or patient populations (known as "off-label use") and limitations on industry-sponsored scientific and educational activities.

Although physicians may prescribe approved products for off-label uses, manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, including not only by company employees but also by agents of the company or those speaking on the company's behalf, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including investigation by federal and state authorities. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Promotional materials for approved drugs must be submitted to the FDA in conjunction with their first use or first publication. Further, if there are any modifications to the drug, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new NDA or NDA supplement, which may require the development of additional data or preclinical studies and clinical trials.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-market testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. In addition, manufacturers and their subcontractors involved in the manufacture and distribution of approved drugs and those supplying products, ingredients and components of them, are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMPs, which impose certain procedural and documentation requirements on sponsors and their CMOs. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that a sponsor may use. Additionally, manufacturers and other parties involved in the drug supply chain for prescription drugs must also comply with product tracking and tracing requirements and for notifying FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Accordingly, manufacturers must continue to expend time money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance. Failure to comply with statutory and regulatory requirements may subject a manufacturer to possible legal or regulatory action, such as warning letters, suspension of manufacturing, product seizures, injunctions, civil penalties or criminal prosecution. There is also a continuing, annual program user fee for any marketed product.

The FDA may withdraw approval of a product if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, requirements for post-market studies or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs; and
- mandated modification of promotional materials and labeling and issuance of corrective information.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of our future product candidates, some of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit restoration of the patent term of up to five years as compensation for patent term lost during the FDA regulatory review process. Patent term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond a patent's current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA.

Marketing exclusivity provisions under the FDCA also can delay the submission or the approval of certain drug product applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an Abbreviated New Drug Application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Privacy and Cybersecurity

Our operations entail the collection, use, disclosure, transfer, and processing of sensitive and personal information. These operations subject us to privacy and data security laws and regulations in the United States and internationally, including, for example and depending on the particular activity, the EU General Data Protection Regulation, or EU GDPR, as the GDPR as incorporated into the laws the United Kingdom, or UK GDPR, and together with EU GDPR, GDPR, and other laws, rules and regulations designed to regulate the processing of personal information and for example reduce risks of identity theft. The privacy, data protection and data security laws to which we are subject impose obligations with respect to the collection, processing, storage, disposal, use, transfer, retention and disclosure of personal information. In addition, under certain of these laws, we must provide notice to individuals of our policies and practices for sharing personal information with third parties, provide advance notice of any changes to our policies and in some cases give individuals the right to prevent processing of their personal information and disclosure of it to third parties. Our operations extend to commercial partnerships and third-party processors, each of which may be governed by their distinct privacy regulations and data security laws. These laws are constantly evolving and subject to varying interpretations, requiring us to periodically update our policies and measures to maintain compliance.

The GDPR in the EU and the UK, which have been incorporated into their respective laws, impose stringent requirements on the processing of health and other sensitive data. These requirements encompass: (i) providing information to individuals regarding data processing activities; (ii) ensuring a legal basis or condition applies to the processing of personal data and, where applicable, obtaining consent from individuals to whom the data processing relates; (iii) responding to data subject requests; (iv) imposing requirements to notify the competent national data protection authorities and data subjects of personal data breaches; (v) implementing safeguards in connection with the security and confidentiality of the personal data; (vi) accountability requirements; and (vii) taking certain measures when engaging third-party processors. The GDPR is also the regulation that informs our obligations with respect to any clinical trials conducted in the EEA or UK. The GDPR's definition of personal data includes coded data, and it requires changes to informed consent practices and detailed notices for clinical trial subjects and investigators. Failure to comply with the GDPR can result in significant practical, legal, and financial repercussions, including the destruction of improperly gathered or used personal data, substantial fines of up to €20 million (£17.5 million) or 4% of the

company's global annual turnover, mandatory audits, orders to cease or modify data use, and a private right of action enabling data subjects to seek damages. In addition, the GDPR provides that EU member states or the UK may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric, or health data.

Further, the UK has recently introduced a new Data Protection & Digital Information (No. 2) Bill. This development could reshape the UK's data protection landscape, distancing it from the EU's data protection regime. This lack of clarity on future UK laws and regulations and their interaction with those of the EU could add legal risk, uncertainty, complexity, and cost; and any resulting divergence in laws could increase our risk profile and necessitate further compliance measures.

To enable the transfer of personal data outside of the EU or the UK, adequate safeguards must be implemented in compliance with the GDPR. The European Commission has issued standard contractual clauses, or SCCs, for data transfers from controllers or processors in the EU (or otherwise subject to the GDPR) to controllers or processors established outside the EU (and not subject to the GDPR). The UK is not subject to the European Commission's new SCCs, and instead it has published the UK International Data Transfer Agreement, or IDTA, and the International Data Transfer Addendum to the new SCCs, or the Addendum, which enable transfers from the UK. Companies relying on SCCs or the IDTA to govern transfers of personal data to third countries will also need to assess whether the data importer can ensure sufficient guarantees for safeguarding the personal data under GDPR, including an analysis of the laws in the recipient's country. Further, the EU and United States have adopted its adequacy decision for the EU-U.S. Data Privacy Framework, or the Framework, which entered into force on July 11, 2023. The Framework provides that the protection of personal data transferred between the EU and the United States is comparable to that offered in the EU. This provides a further avenue to ensuring transfers to the United States are carried out in line with EU GDPR. There has been an extension to the Framework to cover UK transfers to the United States. The Framework could be challenged like its predecessor frameworks. When conducting restricted data transfers under the EU and UK GDPR, we will need to implement these new safeguards, and doing so will require significant effort and cost.

Failure to implement valid mechanisms for personal data transfers from Europe may result in increased exposure to regulatory actions, substantial fines and injunctions against processing personal data from Europe. Inability to export personal data may also: (i) restrict our activities outside Europe; (ii) limit the ability to collaborate with partners as well as other service providers, contractors and other companies outside of Europe; and/or (iii) require us to increase our processing capabilities within Europe at significant expense or otherwise cause us to change the geographical location or segregation of our relevant systems and operations – any or all of which could adversely affect our operations or financial results.

In the United States, privacy and security of personal information are regulated by various federal and state laws, such as health information privacy laws, security breach notification laws, and consumer protection laws.

Compliance with these multifaceted privacy and data security laws can be time-consuming, and failure to comply with any of these regulations could lead to significant fines and penalties (potentially including criminal prosecution), adversely affecting our reputation, business, financial condition, and operational results. Changes in statutes, regulations, or interpretations of existing regulations could impose additional requirements on our operations, such as modifications to data processing arrangements, changes to privacy policies, recall or discontinuation of certain data processing methods, or additional recordkeeping requirements. These changes could adversely affect the operation of our business.

There is a further risk that we may not be able to adequately protect our information systems from cyberattacks. Such breaches could result in the disclosure of confidential, protected, or personal information, damage our reputation, and expose us to significant financial and legal exposure, including potential civil fines and penalties, litigation, and regulatory investigations or enforcement actions under laws such as HIPAA and the GDPR. Further, all 50 states in the United States have laws including obligations to provide notification of unauthorized acquisition of personal information to affected individuals, state officers and others. Some laws may also impose physical and electronic security requirements regarding the safeguarding of personal information. In order to comply with privacy and information security laws, we have confidentiality and information security standards and procedures in place for our business activities.

In addition to the risks outlined above, the legal or regulatory actions may also divert our management from their primary operations. Prohibitions, restrictions, or allegations of violations of these laws could materially and adversely affect our business. Hence, ensuring consistent compliance with privacy and data security laws and regulations remains a critical operational imperative for us.

Other Regulatory Matters

Manufacturing, labeling, packaging, distribution, sales, promotion and other activities of product candidates following product approval, where applicable, or commercialization are also potentially subject to federal and state consumer protection and unfair competition laws, among other requirements to which we may be subject. Additionally, the activities associated with the commercialization of product candidates are subject to regulation by numerous regulatory authorities in the United States in addition to the FDA, which may include the Centers for Medicare and Medicaid Services, or CMS, other divisions of the U.S.

Department of Health and Human Services, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments and governmental agencies.

The distribution of pharmaceutical drugs is subject to additional requirements and regulations, including extensive recordkeeping, licensing, storage and security requirements intended to prevent the unauthorized sale of such pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements may subject firms to legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, exclusion from federal healthcare programs, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, relabeling or repackaging, or refusal to allow a firm to enter into supply contracts, including government contracts. Any claim or action against us for violation of these laws, even if we successfully defends against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Prohibitions or restrictions on marketing, sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in statutes, regulations, or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling or packaging; (iii) the recall or discontinuation of our products; or (iv) additional recordkeeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Regulation Outside of the United States

In addition to regulations in the United States, we are subject to a variety of regulations in other jurisdictions governing clinical studies, commercial sales, and distribution of our products. Most countries outside of the United States require that clinical trial applications be submitted to and approved by the local regulatory authority for each clinical study. In the EU, for example, an application must be submitted to the national competent authority and an independent ethics committee in each country in which we intend to conduct clinical trials, much like the FDA and IRB, respectively. Under the new CTR (EU) No 536/2014, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022, a single application is now made through the Clinical Trials Information System, or CTIS, for clinical trial authorization in up to 30 EU/EEA countries at the same time and with a single set of documentation.

The assessment of applications for clinical trials is divided into two parts (Part I contains scientific and medicinal product documentation and Part II contains the national and patient-level documentation). Part I is assessed by a coordinated review by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted, or Member States concerned of a draft report prepared by a Reference Member State. Part II is assessed separately by each Member State concerned. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the Member State concerned, however overall related timelines are defined by the CTR. The new CTR also provides for simplified reporting procedures for clinical trial sponsors.

In addition, whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of countries outside the United States before we can commence marketing of the product in those countries. The approval process and requirements vary from country to country, so the number and type of nonclinical, clinical, and manufacturing studies needed may differ, and the time may be longer or shorter than that required for FDA approval.

To obtain regulatory approval of our medicinal products under the EU's regulatory system, we are required to submit a marketing authorization application, or MAA, to be assessed in the centralized procedure. The centralized procedure allows applicants to obtain a marketing authorization, or MA, that is valid throughout the EU, and the additional Member States of the European Economic Area (Iceland, Liechtenstein and Norway), or EEA. It is compulsory for medicinal products manufactured using biotechnological processes, orphan medicinal products, advanced therapy medicinal products (gene-therapy, somatic cell-EU and which is intended for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, auto-immune and other immune dysfunctions, viral diseases or diabetes). The centralized procedure is optional for any other products containing new active substances not authorized in the EU or for products which constitute a significant therapeutic, scientific, or technical innovation or for which a centralized authorization is in the interests of patients at EU level. When a company wishes to place on the market a medicinal product that is eligible for the centralized procedure, it sends an application directly to the European Medicines Agency, or EMA, to be assessed by the Committee for Medicinal Products for Human Use, or CHMP. The CHMP is responsible for conducting the assessment of whether a medicine meets the required quality, safety, and efficacy requirements, and whether the product has a positive risk/benefit profile. Once the CHMP has completed its assessment, the CHMP will give a favorable or unfavorable opinion as to whether to grant the authorization. The time limit for the evaluation procedure is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the

CHMP). The EMA then has fifteen days to forward its opinion to the European Commission, which will make a binding decision on the grant of an MA within 67 days of the receipt of the CHMP opinion.

The criteria for designating an "orphan medicinal product" in the EU are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as an orphan medicinal product if it is intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition that affects no more than five in 10,000 persons in the EU when the application is made. In addition, orphan designation can be granted if the product is intended for a life threatening, seriously debilitating, or serious and chronic condition in the EU and when, without incentives, it is unlikely that sales of the product in the EU would be sufficient to justify the necessary investment in its development. Orphan designation is only available if there is no other satisfactory method approved in the EU of diagnosing, preventing, or treating the applicable orphan condition, or if such a method exists, the proposed orphan medicinal product will be of significant benefit to patients affected by such condition, as defined in Regulation (EC) 847/2000.

Orphan designation provides opportunities for fee reductions, protocol assistance, and access to the centralized procedure. In addition, if a product which has an orphan designation subsequently receives a centralized MA for the indication for which it has such designation, the product is entitled to orphan market exclusivity, which means the EMA may not approve any other application to market a similar medicinal product for the same indication for a period of ten years. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The exclusivity period may be reduced to six years if, at the end of the fifth year, it is shown that the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, an MA may be granted to a similar medicinal product for the same indication at any time if:

- the second applicant can establish that its product, although similar to the authorized product, is safer, more effective or otherwise clinically superior;
- the MA holder of the authorized product consents to a second orphan medicinal product application; or
- the MA holder of the authorized product cannot supply enough orphan medicinal product.

A pediatric investigation plan, or PIP, in the EU is aimed at ensuring that the necessary data are obtained to support the authorization of a medicine for children, through studies in children. All applications for MAs for new medicines have to include the results of studies as described in an agreed PIP, unless the medicine is exempt because of a deferral or waiver. This requirement also applies when an MA holder wants to add a new indication, pharmaceutical form, or route of administration for a medicine that is already authorized and covered by intellectual property rights. Several rewards and incentives for the development of pediatric medicines for children are available in the EU. Medicines authorized across the EU with the results of studies from a PIP included in the product information are eligible for an extension of their supplementary protection certificate, or SPC, by six months (provided an application for such extension is made at the same time as filing the SPC application for the product, or at any point up to two years before the SPC expires). This is the case even when the studies' results are negative. For orphan medicinal products, the incentive is an additional two years of market exclusivity. Scientific advice and protocol assistance at the EMA are free of charge for questions relating to the development of pediatric medicines.

In March 2016, the EMA launched an initiative, the Priority Medicines scheme, or the PRIME scheme, to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIME scheme is intended to encourage development of products in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies on the basis of compelling non-clinical data and tolerability data from initial clinical trials. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and potentially accelerated MAA assessment once a dossier has been submitted. Importantly, once a candidate medicine has been selected for the PRIME scheme, a dedicated contact and rapporteur from the CHMP or from the Committee for Advanced Therapies, or CAT, are appointed early in the PRIME scheme facilitating increased understanding of the product at EMA's committee level. An initial meeting with the CHMP/CAT rapporteur initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies. PRIME eligibility does not change the standards for product approval, and there is no assurance that any such designation or eligibility will result in expedited review or approval. The aforementioned EU rules are generally applicable in the EEA. The United Kingdom left the EU on January 31, 2020.

The United Kingdom have concluded a trade and cooperation agreement, or TCA, which was provisionally applicable since January 1, 2021 and has been formally applicable since May 1, 2021. The TCA includes specific provisions concerning pharmaceuticals, which include the mutual recognition of GMP, inspections of manufacturing facilities for medicinal products and GMP documents issued, but does not provide for wholesale mutual recognition of United Kingdom and EU pharmaceutical regulations. At present, Great Britain has implemented EU legislation on the marketing, promotion and sale of medicinal products

through the Human Medicines Regulations 2012 (as amended). Except in respect of the new EU Clinical Trials Regulation, the regulatory regime in Great Britain therefore largely aligns with current EU medicines regulations, however it is possible that these regimes will diverge more significantly in future now that Great Britain's regulatory system is independent from the EU and the TCA does not provide for mutual recognition of United Kingdom and EU pharmaceutical legislation. However, notwithstanding that there is no wholesale recognition of EU pharmaceutical legislation under the TCA, under a new framework which will be put in place by the Medicines and Healthcare products Regulatory Agency, or MHRA, the United Kingdom's medicines regulator, from January 1, 2024, the MHRA will take into account decisions on the approval of MAs from the EMA (and certain other regulators) when considering an application for a Great Britain MA.

On February 27, 2023, the United Kingdom government and the European Commission announced a political agreement in principle to replace the Northern Ireland Protocol with a new set of arrangements, known as the "Windsor Framework". This new framework fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the United Kingdom. In particular, the MHRA will be responsible for approving all medicinal products destined for the United Kingdom market (i.e., Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. A single United Kingdom-wide MA will be granted by the MHRA for all medicinal products to be sold in the United Kingdom, enabling products to be sold in a single pack and under a single authorization throughout the United Kingdom. On June 9, 2023, the MHRA announced that the medicines aspects of the Windsor Framework will apply after January 1, 2025.

There is now no pre-MA orphan designation in Great Britain. Instead, the MHRA reviews applications for orphan designation in parallel to the corresponding MAA. The criteria are essentially the same, but have been tailored for the Great Britain market, i.e., the prevalence of the condition in Great Britain (rather than the EU) must not be more than five in 10,000. Should an orphan designation be granted, the period or market exclusivity will be set from the date of first approval of the product in Great Britain or the EU, wherever is earliest.

Outside the United States, ensuring coverage and adequate payment for a product also involves challenges. Pricing of prescription pharmaceuticals is subject to government control in many countries. In some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product candidate to currently available therapies. A Member State may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our product candidates. Historically, products launched in the European Union do not follow U.S. price structures and generally prices tend to be significantly lower.

Patients Rely on Insurance Coverage by Third-Party Payors (third-party payors include Medicare and Medicaid (government payors) and commercial insurance companies such as Blue Cross Blue Shield, Humana, Cigna, etc.) to Pay for Products

In the United States and markets in other countries, patients generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Our ability to successfully commercialize our product candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations.

Additionally, the process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which products they will pay for and establish reimbursement levels. Third-party payors are increasingly challenging the prices charged, examining the medical necessity, and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication. Net prices for products may also be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the United States. Increasingly, third-party payors are requiring that pharmaceutical companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained. Even if

coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

No Uniform Policy Exists for Coverage and Reimbursement in the U.S.

There is also significant uncertainty related to the insurance coverage and reimbursement of newly approved products and coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the United States, the principal decisions about reimbursement for new medicines are typically made by the CMS, an agency within the U.S. Department of Health and Human Services that implements the Medicare program. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree.

We currently expect that any drugs we develop may need to be administered under the supervision of a physician on an outpatient basis. Under currently applicable U.S. law, certain drugs that are not usually self-administered (such as most injectable drugs) may be eligible for coverage under the Medicare Part B program if:

- they are incident to a physician's services;
- they are reasonable and necessary for the diagnosis or treatment of the illness or injury for which they are administered according to accepted standards of medical practice; and
- they have been approved by the FDA and meet other requirements of the statute.

Pharmaceutical companies whose products are reimbursed under Medicare Part B must calculate and report certain price reporting metrics to the government, such as average sales price, and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for product candidates may be reduced by mandatory discounts or rebates required by government healthcare programs.

Further, during the COVID-19 pandemic, millions of individuals lost employer-based insurance coverage. It is unclear what effect, if any, the American Rescue Plan will have on the number of covered individuals, which may adversely affect our ability to commercialize our products.

Other Healthcare Laws

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business that may constrain the financial arrangements and relationships through which we research, as well as sell, market and distribute any products for which we obtain marketing authorization. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, and transparency laws and regulations related to drug pricing and payments and other transfers of value made to physicians and other healthcare providers. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of operations, integrity oversight and reporting obligations, exclusion from participation in federal and state healthcare programs and responsible individuals may be subject to imprisonment.

Affordable Care Act and Legislative Reform Measures

Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs and those methods are not always specifically adapted for new technologies such as gene therapy and therapies addressing rare diseases such as those we are developing. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the ACA was enacted, which, among other things, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations; subjected manufacturers to new annual fees and taxes for certain branded prescription drugs; created a Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; and provided incentives to programs that increase the federal government's comparative effectiveness research.

Since its enactment, there have been numerous judicial, administrative, and executive, challenges to certain aspects of the ACA. In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, the American Rescue Plan Act of 2021 eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024. Further, the Budget Control Act of 2011 and subsequent legislation, among other things, created measures for spending reductions by Congress that include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which remain in effect through 2031. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers will be further reduced starting in 2025 absent further legislation. The U.S. American Taxpayer Relief Act of 2012 further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

The Inflation Reduction Act of 2022, or IRA, includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket cap for Medicare Part D beneficiaries to $2,000 starting in 2025; impose new manufacturer financial liability on certain drugs under Medicare Part D; allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs without generic competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the rebate rule that would limit the fees that pharmacy benefit managers can charge. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication is for that disease or condition. If a product receives multiple orphan designations or has multiple approved indications, it may not qualify for the orphan drug exemption. Further, judicial challenges to the IRA may have an impact on the implementation of the IRA's provisions; and the overall effects of the IRA on our business and the healthcare industry in general is not yet known.

These laws and regulations may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Other U.S. Environmental, Health and Safety Laws and Regulations

We may be subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover costs and expenses we may incur due to injuries to our employees as well as insurance for environmental liability, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Employees and Human Capital Resources

As of December 31, 2023, we had 101 full-time employees, including 34 who hold Ph.D. degrees, and one part-time employee; 74 employees are engaged in research and development and 27 employees are engaged in management or general and administrative activities. None of our employees are subject to a collective bargaining agreement or represented by a trade or labor union. We consider our relationship with our employees to be good. We also employ consultants from time to time, including to assist with Merger integration efforts.

Our human capital objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards and cash-based performance bonus awards.

Item 1A. Risk Factors.

You should consider carefully the risks and uncertainties described below, together with all of the other information in this Annual Report on Form 10-K and in our other filings with the Securities and Exchange Commission, or SEC. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including the risks we face as described below and elsewhere in this Annual Report on Form 10-K. See "Cautionary Statement Regarding Forward Looking Statements.".

Risks Related to Our Business

Risks Related to Our Financial Position and Need for Capital

We have incurred significant losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. Our net loss was $81.2 million and $58.0 million for the years ended December 31, 2023 and 2022, respectively. As of December 31, 2023, we had an accumulated deficit of $183.0 million. We have financed our operations primarily through private placements of our preferred stock and more recently, common stock in the pre-closing financing that closed immediately prior to the Merger. Substantially all of our losses have resulted from expenses incurred in connection with our research and development and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses, increasing operating losses, and negative operating cash flows for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- continue current research programs and preclinical development of any product candidates we may identify;
- seek to identify additional research programs and product candidates;
- initiate preclinical studies and clinical trials for any product candidates we may identify;
- further develop Oligonucleotide Promoted Editing of RNA, or OPERA, our RNA editing platform;
- maintain, expand, enforce, defend and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our intellectual property portfolio;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- develop, maintain and enhance a sustainable, scalable, reproducible and transferable manufacturing process for the product candidates we may develop;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any therapies for which we may obtain marketing approval;
- hire additional research and development personnel;
- hire clinical and commercial personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development;
- acquire or in-license product candidates, intellectual property and technologies;
- establish and maintain collaborations;
- should we decide to do so, build and maintain commercial-scale current Good Manufacturing Practices, or cGMP, manufacturing facility;

- experience any delays or interruptions due to global pandemics, such as the recent COVID-19 endemic, or other events unrelated to our business such as the Russian invasion of Ukraine or Israeli-Hamas conflict that could result in delays in preclinical testing and clinical trials or interruptions in the supply chain; and
- operate as a public company.

We have not initiated clinical development of any potential product candidate and expect that it will be many years, if ever, before we have a RNA editing therapy ready for commercialization. To become and remain profitable, we must develop and, either directly or through collaborators, eventually commercialize a therapy or therapies with market potential. This will require us to be successful in a range of challenging activities, including identifying product candidates, completing preclinical studies and clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those therapies for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability.

We have transitioned from discovery, research and development to early preclinical development for our development candidate, KRRO-110. Because of the numerous risks and uncertainties associated with developing oligonucleotide product candidates, we are unable to predict the extent of any future losses or when we will become profitable, if at all. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand business or continue our operations. A decline in our value could also cause you to lose all or part of your investment.

We have never generated revenue from product sales and may never become profitable.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates we may identify for development. We do not anticipate generating revenues from product sales for many years, if ever. Our ability to generate future revenues from product sales depends heavily on our, or our collaborators', ability to successfully:

- identify product candidates and successfully complete research and development of such product candidates;
- seek and obtain regulatory and marketing approvals for any product candidates for which we complete clinical trials;
- launch and commercialize any product candidates for which we may obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure, or alternatively, collaborating with a commercialization partner;
- qualify for adequate coverage and reimbursement by government and third-party payors for any product candidates for which we may obtain regulatory and marketing approval;
- establish and maintain supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for any product candidates for which we obtain regulatory and marketing approval;
- develop, maintain and enhance a sustainable, scalable, reproducible and transferable manufacturing process for the product candidates we may develop;
- address competing technological and market developments;
- negotiate favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- receive market acceptance by physicians, patients, healthcare payors, and others in the medical community;
- maintain, protect, enforce, defend and expand our portfolio of intellectual property and other proprietary rights, including patents, trade secrets and know-how;
- defend against third party intellectual property claims of infringement, misappropriation or other violation; and
- attract top talent and retain qualified personnel.

Our expenses could increase beyond expectations if we are required by the U.S. Food and Drug Administration, or the FDA, the European Medicines Agency, or the EMA, or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if one or more of the product candidates we may develop are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Additionally, such products may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives. Even if we are able to generate revenues from the sale of any approved product candidates, we may not become profitable and may need to obtain additional funding to continue operations.

We will need substantial additional funding. If we are unable to raise capital when needed, we will be forced to delay, reduce, eliminate or prioritize among our research and development programs or future commercialization efforts.

We expect our expenses to continue to increase in connection with our ongoing activities, particularly as we identify, continue the research and development of, initiate preclinical studies and clinical trials of, and seek marketing approval for, product candidates. Because we have limited financial and managerial resources, we have prioritized our research programs and lead optimization efforts in specific indications among many potential options. Specifically, our initial development programs target liver and central nervous systems indications, amongst others. As a result of this prioritization, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater clinical or commercial potential and we may need to reprioritize our focus in the future. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable therapies.

In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a collaborator. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and product development programs or future commercialization efforts.

As of December 31, 2023, our cash and cash equivalents were $166.1 million, excluding restricted cash, or $173.1 million, including restricted cash. We believe our existing cash and cash equivalents will be sufficient to fund our operating expenses and capital expenditure requirements through several value-creating milestones and into 2026. However, our operating plan may change as a result of factors currently unknown, and expectations regarding our cash runway and ability to reach data inflection points are based on numerous assumptions that may prove to be untrue. As a result, we may be required to raise capital sooner than anticipated and our exposure to certain contingent liabilities and contractual obligations may be greater than anticipated. Our future capital requirements will depend on many other factors, including those discussed in the risk factor entitled *"We have incurred significant losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability."*

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize any product candidates we may develop. We cannot be certain that additional funding will be available on acceptable terms or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of any product candidates or other research and development initiatives. We could be required to seek collaborators for potential product candidates earlier than we would otherwise plan or on terms that are less favorable than might otherwise be available. We could also be required to relinquish or license our rights to product candidates on unfavorable terms in certain markets where we otherwise would seek to pursue development or commercialization ourselves.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates we may develop.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our capital needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends and possibly other restrictions. In addition, if we raise funds through additional license and collaboration agreements, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our intellectual property, technologies, future revenue streams, research programs or product candidates we may develop, or we may have to grant licenses on terms that may not be favorable.

Our operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our business has now become that of Legacy Korro, an early-stage company founded in September 2018 and which commenced operations in October 2019. Prior to the Merger, Legacy Korro's operations (which are now ours) were limited to organizing and staffing, business planning, raising capital, acquiring and developing our platform and technology and identifying and beginning to advance preclinical testing of potential product candidates. All of our current programs are still in the research or preclinical stage of development and their risk of failure is high. We have not yet demonstrated an ability to initiate or successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial-scale therapy, arrange for a third party to do so on our behalf or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes about 10 to 15 years to develop a new therapy from the time it is discovered to when it is available for treating patients.

Legacy Korro's limited operating history, particularly in light of the rapidly evolving gene editing field, may make it difficult to evaluate our technology and industry and predict our future performance. Accordingly, any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by very early-stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer.

In addition, as a new business, we may encounter other unforeseen expenses, difficulties, complications, delays, and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Our ability to utilize our net operating loss, or NOL, carryforwards and certain other tax attributes may be limited.

Since our inception, we have incurred losses and we may never achieve profitability. As of December 31, 2023, we had federal and state NOLs of $302.5 million and $266.3 million, respectively. Under current law, our federal NOLs generated in taxable years ending after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOLs is limited to 80% of our taxable income annually for tax years beginning after December 31, 2018. Federal NOLs generated in taxable years ending on or prior to December 31, 2017, however, have a 20-year carryforward period, but are not subject to the 80% limitation.We have federal NOLs of $22.4 million that are subject to expiration between 2036 and 2037 and have $280.1 million of federal NOLs that do not expire. Our state NOLs expire at various dates from 2035 through 2043. As of December 31, 2023, we had federal research and development tax credit carryforwards of $15.6 million that expire at various dates from 2036 through 2043. In addition, as of December 31, 2023, we had state research and development tax credit carryforwards of $8.7 million that expire at various dates from 2032 through 2038 and state investment tax credit carryforwards of $0.2 million that expire at various dates from 2024 through 2025.

Under Sections 382 and 383 of the Code, if a corporation undergoes an "ownership change," generally defined as one or more shareholders or groups of shareholders who own at least 5% of the corporation's equity increasing their equity ownership in the aggregate by more than 50 percentage points (by value) over a rolling three-year period, the corporation's ability to use its pre-change NOLs and other pre-change tax attributes (such as research and development tax credits) to offset its post-change income or taxes may be limited. Similar rules may apply under state tax laws. Our prior equity offerings and other changes in our stock ownership may have resulted in such ownership changes in the past. We have not conducted a formal study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception. In addition, we may experience ownership changes in the future as a result of future securities offering or subsequent shifts in our stock ownership, some of which are outside of our control. In particular, if the Merger or the Pre-Closing Financing constitutes an ownership change within the meaning of Section 382 of the Code, we could lose or otherwise be substantially limited in our ability to use our NOLs and tax credit carryforwards. As a result, if we earn net taxable income in the future, our ability to use our pre-change NOLs or other pre-change tax attributes to offset U.S. federal taxable income or income taxes may be subject to limitations, which could potentially result in increased future tax liability to us. There is a risk that due to changes under the tax law, regulatory changes or other unforeseen reasons, our existing NOLs or business tax credits could expire or otherwise be unavailable to offset future income tax liabilities. At the state level, there may also be periods during which the use of NOLs or business tax credits is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed by us. For these reasons, we may not be able to realize a tax benefit from the use of our NOLs or tax credits, even if we attain profitability.

Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect our business and financial condition. In recent years, many such changes have been made and changes are likely to continue to occur in the future. We cannot predict whether, when, in what form or with

what effective dates, tax laws, regulations and rulings may be enacted, promulgated or decided or whether they could increase our tax liability or require changes in the manner in which we operate in order to minimize increases in our tax liability.

Risks Related to Discovery, Development and Commercialization

The gene editing field and RNA editing in particular is relatively new and is evolving rapidly. We are very early in our development efforts and may not be successful in identifying and developing product candidates. It will be many years before we or our collaborators commercialize a product candidate or generate any revenues, if ever. Additionally, other gene editing technologies may be discovered that provide significant advantages over RNA editing, which could materially harm our business.

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates. We are very early in our development efforts and have focused our research and development efforts to date on developing OPERA, our RNA editing platform, and identifying our initial targeted disease indications. Although we believe we can demonstrate many of the key advantages of RNA editing, because we are very early in our development efforts, we are not yet certain of the results we may achieve, which may be important for registration and commercialization of our products. Such uncertainties include, but are not limited to, the level of editing efficiency needed in a target tissue type to achieve a clinical benefit, and associated safety of our edits in humans. We have also not yet shown that preclinical editing activity can result in clinically important effects, nor that the data generated by our preclinical studies can translate into positive results in clinical trials.

All of our product development programs are still in the research or preclinical stage of development. Our research methodology may be unsuccessful in identifying product candidates, our product candidates may be shown to have harmful side effects in preclinical *in vitro* experiments or animal model studies, they may not show promising signals of therapeutic effect in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable, or unlikely to receive marketing approval.

The pharmacological properties ascribed to the product candidates we are testing in preclinical studies may not be positively demonstrated in clinical trials in patients, and they may interact with human biological systems in unforeseen, ineffective or harmful ways. If our product candidates prove to be ineffective, unsafe or commercially unviable, OPERA and our pipeline would have little, if any, value, which would substantially harm our business, financial condition, results of operations and prospects. In addition, our approach, which focuses on using oligonucleotides for drug development, as opposed to multiple or other, more advanced proven technologies, and new products and technologies that may enter the market, may expose us to additional financial risks and make it more difficult to raise additional capital if we are not successful in developing one or more product candidates that receive regulatory approval. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of any product candidates we may discover, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

In addition, although we believe OPERA, our RNA editing platform, will position us to expand our portfolio of product candidates beyond the initial product candidates we may develop, we have not yet successfully developed any product candidate and our ability to expand our portfolio may never materialize.

Commencing clinical trials in the United States is also subject to acceptance by the FDA of any future investigational new drug applications, or INDs, and finalization of trial designs based on discussions with the FDA and other regulatory authorities. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial designs or any clinical endpoints selected, which may require us to complete additional studies or trials or impose stricter approval conditions than we expect. There are equivalent processes and risks applicable to clinical trial applications, or CTAs, in other countries, including in Europe, the UK and Australia.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize our product candidates in the United States or any other jurisdiction, and any such approval may be for a narrower indication than we seek. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. We may conduct one or more of our clinical trials with one or more trial sites that are located outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of these data is subject to conditions imposed by the FDA, and there can be no assurance that the FDA will accept data from trials conducted outside of the United States. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and could delay or permanently halt our development of the applicable product candidates. Similarly, marketing approval by the FDA in the United States, if obtained, does not ensure

approval by regulatory authorities in other countries or jurisdictions. Approval processes vary among countries and can involve additional product candidate testing and validation and additional administrative review periods.

Commercialization of any product candidates we may develop will also require obtaining manufacturing supply, capacity and expertise; building of a commercial organization; and significant marketing efforts. If we do not successfully commercialize any product candidates we may develop, we could experience a material harm to our business.

RNA editing is a novel technology that is not yet clinically validated for human therapeutic use. The approaches we take to discover and develop novel therapeutics are unproven and may never lead to marketable products.

We are focused on developing therapies based on RNA editing. Although there have been significant advances in the field of gene editing in recent years, RNA editing technologies are new and largely unproven. The technologies that we have developed have not yet been clinically tested, nor are we aware of any clinical trials for safety or efficacy having been completed by third parties using RNA editing or similar technologies. The scientific evidence to support the feasibility of developing product candidates based on these technologies is both preliminary and limited. Successful development of product candidates by us will require solving a number of issues, including optimizing the efficiency and specificity of such product candidates, and ensuring the therapeutic selectivity of such product candidates. There can be no assurance we will be successful in solving any or all of these issues.

We have concentrated our research efforts to date on preclinical work to bring therapeutics to the clinic for our initial indications, and our future success is highly dependent on the successful development of OPERA, our RNA editing platform, as well as cellular delivery methods and therapeutic applications of that technology. While some of the existing, non-RNA editing, gene editing technologies developed by third parties have progressed to clinical trials, they continue to suffer from various limitations, and such limitations may affect our future success. While a number of clinical trials for oligonucleotide products conducted by other companies have not been successful, some have received regulatory approval. The pharmacological properties ascribed to the product candidates we are testing or will test in the future may not be positively demonstrated in clinical trials in patients, and they may interact with human biological systems in unforeseen, ineffective or harmful ways. If our product candidates prove to be ineffective, unsafe or commercially unviable, our OPERA platform and our pipeline would have little, if any, value, which would substantially harm our business, financial condition, results of operations and prospects. We may decide to alter or abandon our initial programs as new data becomes available and we gain experience in developing base editing therapeutics. We cannot be sure that our technologies will yield satisfactory products that are safe and effective, scalable or profitable in our initial indications or any other indication we pursue.

Development activities in the field of RNA editing are currently subject to a number of risks related to the ownership and use of certain intellectual property rights that are subject to patent reexamination and inter partes proceedings in the United States and opposition proceedings in Europe. For additional information regarding the risks that may apply to our and our licensors' intellectual property rights, see "*—Risks Related to Intellectual Property.*"

We are very early in our development efforts, and our preclinical studies and clinical trials may not be successful. If we are unable to commercialize our product candidates or experiences significant delays in doing so, our business will be materially harmed.

We are very early in our development of product candidates and have focused our efforts to date on platform development, discovery, research, and preclinical development. Currently, all of our programs are still in the research or preclinical stage of development. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development, marketing approval and eventual commercialization of our product candidates, which may never occur. We have not yet generated revenue from product sales or otherwise, and we may never be able to develop or commercialize a marketable product.

Commencing clinical trials in the United States is subject to acceptance by the FDA of an IND and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In the event that the FDA requires us to complete additional preclinical studies or we are required to satisfy other FDA requests prior to commencing clinical trials, the start of our first clinical trials may be delayed or we may be unsuccessful obtaining clearance to proceed into clinical development. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial designs or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials, delay the enrollment of our clinical trials, abandon our clinical development plans or meet stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to CTAs in other countries, including countries in the European Union, or EU.

Commercialization of any product candidates we may develop will require preclinical and clinical development; regulatory and marketing approval in multiple jurisdictions, including by the FDA and the EMA; manufacturing supply, capacity and expertise; a commercial organization; and significant marketing efforts. The success of our product candidates will depend on many factors, including the following:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable;
- effective INDs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including under the FDA's current GCPs, current Good Laboratory Practices, or GLPs, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our future clinical trials that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations;
- receipt of marketing approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for any product candidates we may develop;
- commercial launch of any product candidates we may develop, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of the product candidates we may develop, including method of administration, if and when approved, by patients, the medical community and third-party payors;
- effective competition with other therapies;
- maintenance of a continued acceptable safety, tolerability and efficacy profile of any product candidates we may develop following approval; and
- establishment and maintenance of healthcare coverage and adequate reimbursement by payors.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may develop, which would materially harm our business.

Any product candidates we develop may fail in preclinical or clinical development or be delayed to a point where they do not become commercially viable.

Before obtaining regulatory approval for the commercial distribution of any of our product candidates, we must conduct, at our own expense, extensive preclinical studies and clinical trials to demonstrate the safety and efficacy in humans of our product candidates. Preclinical and clinical testing are expensive, difficult to design and implement, can take many years to complete, are uncertain as to outcome, and the historical failure rate for drugs in preclinical and clinical development is high. For example, we depend on the availability of non-human primates to conduct certain preclinical studies. Over the past several years there has been an increasing global shortage of non-human primates available for drug development that has matured into an acute global supply chain issue. The supply of these non-human primates is currently constrained due to factors such as their limited worldwide availability, domestic regulatory restrictions and trade relations. If we are unable to obtain access to a sufficient supply of these non-human primates in a timely manner or at all, our timelines and our ability to complete preclinical testing and submit IND or CTA applications may be adversely affected.

The development of one or more of our product candidates can fail at any stage of testing. We may experience numerous unforeseen events during, or as a result of, preclinical studies and clinical trials that could delay or prevent regulatory approval or our ability to commercialize our product candidates, including:

- our preclinical studies or clinical trials may produce negative or inconclusive results, including results that may not meet the level of significance or clinical benefit required by the FDA or other regulators, and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials, or we may abandon projects that we had expected to be promising;

- delays in filing INDs or comparable foreign applications or delays or failure in obtaining the necessary approvals from regulators or institutional review boards, or IRBs, in order to commence a clinical trial at a prospective trial site, or their suspension or termination of a clinical trial once commenced;
- conditions imposed on us by the FDA or comparable foreign authorities regarding the scope or design of our clinical trials;
- divergent views between FDA and other homologue regulatory authorities as to the objectives and/or design of the clinical trials required in support of marketing registration;
- problems in obtaining or maintaining IRB approval of trials;
- delays in enrolling patients or volunteers into clinical trials, and variability in the number and types of patients eligible for clinical trials;
- an inability to open study sites, or enroll, treat, and monitor patients due to local restrictions, including as a result of COVID-19 or any other pandemic or endemic or other events, such as the Russian invasion of Ukraine or Israeli-Hamas conflict;
- delays in developing and receiving regulatory approval for companion diagnostic tests, to the extent such tests are needed, to identify patients for our clinical trials;
- high drop-out rates for patients in clinical trials and substantial missing data;
- negative or inconclusive results from our clinical trials or the clinical trials of others for product candidates similar to ours;
- failure of future clinical trials to confirm positive results, if any, from earlier preclinical studies and clinical trials;
- inability to consistently manufacture, inadequate supply, or unacceptable quality of product candidate materials or other materials necessary for the conduct of our clinical trials;
- greater than anticipated clinical trial costs;
- serious and unexpected side effects that may or may not be related to the product candidate being tested that are experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- poor or disappointing effectiveness of our product candidates during clinical trials;
- unfavorable outcome of FDA or other regulatory agency inspection and review of a manufacturing or clinical trial site or other records relating to the clinical investigation;
- failure of our third-party contractors, investigators, or collaboration partners to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner, or at all;
- governmental or regulatory delays and changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around manufacturing, preclinical, or clinical testing generally or with respect to our product candidates class, in particular; or
- varying interpretations of data by the FDA and similar foreign regulatory agencies.

If we do not successfully conduct clinical development, we will not be able to market and sell products derived from our product candidates or generate product revenues. Even if we do successfully complete clinical trials, those results are not necessarily predictive of results of additional trials that may be needed before we can submit an application for regulatory approval to the FDA or foreign regulatory agencies. If the development of any of our product candidates fails or is delayed to a point where such product candidate is no longer commercially viable, our business may be materially harmed.

We may not be able to conduct clinical trials successfully due to various process-related factors that could negatively impact our business plans.

The successful initiation and completion of any of our clinical trials, within timeframes consistent with our business plans, is dependent on various factors, which include, but are not limited to, our ability to:

- retain and recruit employees, contractors or consultants with the required level of knowledge and experience;

- retain and recruit, in a timely manner, a sufficient number of patients necessary to conduct a clinical trial, which is a function of many factors, including the impact of the COVID-19 endemic, the proximity of participants to clinical sites, the size of the relevant population, the eligibility criteria for the trial, possible adverse effects from treatments, the existence of competing clinical trials, the involvement of patient advocacy groups, the availability of new or alternative treatments, lack of efficacy, personnel issues and ease of participation in our clinical trials;
- manage the impact of the COVID-19 endemic or other global health pandemics or endemics on our early-stage discovery efforts and clinical trials; open study sites, and enroll, treat, and monitor patients due to local restrictions implemented in response to remaining COVID-19 effects or other global health pandemics or endemics;
- develop companion diagnostic tests for use with certain of our product candidates or identify partners with such expertise;
- manufacture and maintain a sufficient amount of clinical material, internally or through third parties;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable regulatory and legal guidelines;
- apply the appropriate pharmacovigilance measures in case of adverse effects emerging during a clinical trial;
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the CROs involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and IRBs, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting us to various risks; and
- conduct clinical trials in a cost-effective manner, including management of foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain functions during the clinical trial.

If we are not able to manage the clinical trial process successfully, our business plans could be delayed or be rendered unfeasible for us to execute within our planned or required time frames, or at all.

If we cannot successfully manufacture our product candidates for our research and development and preclinical activities, or manufacture sufficient amounts of our product candidates to meet our clinical requirements and timelines, our business may be materially harmed.

In order to develop our product candidates, apply for regulatory approvals and commercialize our product candidates, we will need to develop, contract for, or otherwise arrange for the necessary manufacturing and supply capabilities. In addition to the oligonucleotides that we manufacture internally, we may utilize CMOs to manufacture the oligonucleotides required for our preclinical studies and clinical trials. There are a limited number of manufacturers that supply oligonucleotides. There are risks inherent in pharmaceutical manufacturing that could affect our ability or the ability of our CMOs to meet delivery time requirements or provide adequate amounts of material to meet our clinical trial demands on our projected timelines. Included in these risks are potential synthesis and purification failures and/or contamination during the manufacturing process, as well as other issues with our facility or the CMOs' facilities and ability to comply with the applicable manufacturing requirements and quality standards, which could result in unusable product and cause delays in our manufacturing timelines and ultimately delay our clinical trials, as well as result in additional expense. To manufacture our oligonucleotides, we rely on third parties to supply the required raw materials. We will likely need to secure alternative suppliers for these raw materials, and such alternative suppliers are limited and may not be readily available, or we may be unable to enter into agreements with them on reasonable terms and in a timely manner. For example, we source certain materials used in the manufacture of our products from China and other countries outside of the United States; the coronavirus outbreak or other similar global disruptions has made access to our existing supply chain difficult and further supply chain disruptions could impact our business. Additionally, our cost of goods development is at an early stage. The actual cost to manufacture and process our product candidates could be greater than expected and could materially and adversely affect the commercial viability of our product candidates.

Moreover, we license the LNP technology used to deliver KRRO-110 from a third party. Although our current partner, Genevant Sciences GmbH, or Genevant, is a well established leader in the LNP space, and our preclinical studies of this LNP delivery technology have shown improved dose-dependent efficacy with reduced clinical chemistry and adverse events, there is

no guarantee that this will be replicated in clinical trials. There is also no guarantee that we will continue to source the LNP delivery system for KRRO-110 from Genevant. The process of establishing and maintaining collaborative relationships and identifying and securing access to optimized delivery systems that are fit-for-purpose is difficult, time-consuming, and involves significant uncertainty. If the current arrangement with Genevant is terminated, our clinical development, manufacturing, or commercialization efforts for KRRO-110 could be delayed or terminated, while we secure an alternative delivery system, which could have a material adverse impact on our clinical development plans and business.

The process of manufacturing oligonucleotides is complex and we may encounter difficulties in production, particularly with respect to process development or scaling-up of our manufacturing capabilities.

The process of manufacturing oligonucleotides is complex, highly-regulated and subject to multiple risks. The complex processes associated with the manufacture of our product candidates expose us to various manufacturing challenges and risks, which may include delays in manufacturing adequate supply of our product candidates, limits on our ability to increase manufacturing capacity, and the potential for product failure and product variation in quality that may interfere with preclinical studies and clinical trials, along with additional costs. We may also make changes to our manufacturing process or the delivery system we use at various points during development, and even after commercialization, for various reasons, such as optimizing costs, achieving scale, decreasing processing time, increasing manufacturing success rate, or other reasons. Such changes carry the risk that they will not achieve their intended objectives, and any of these changes could cause our product candidates to perform differently and affect the results of current or future clinical trials, or the performance of the product, once commercialized. In some circumstances, changes in the manufacturing or delivery system may require us to perform ex vivo comparability studies, and/or conduct animal studies, and to collect additional data from patients prior to undertaking more advanced clinical trials. For instance, changes in our manufacturing process during the course of clinical development may require us to show the comparability of the product used in earlier clinical trials or at earlier portions of a trial to the product used in later clinical trials or later portions of the trial. We may also make further changes to our manufacturing or delivery system before or after commercialization, and such changes may require us to show the comparability of the resulting product to the product produced via earlier manufacturing processes and supplied or delivery system used in clinical studies. We may be required to collect additional preclinical and/or clinical data from any modified process prior to obtaining marketing approval for the product candidate produced with such modified process. If preclinical and/or clinical data are not ultimately comparable to those seen in the earlier trials, we may be required to make further changes to our process and/or undertake additional clinical testing, either of which could significantly delay the clinical development or commercialization of the associated product candidate.

Although we continue to build on our experience in manufacturing oligonucleotides, we have limited experience as a company manufacturing product candidates for commercial supply. We may never be successful in manufacturing product candidates in sufficient quantities or with sufficient quality for commercial use. Our manufacturing capabilities could be affected by cost-overruns, unexpected delays, equipment failures, labor shortages, operator error, natural disasters, unavailability of qualified personnel, difficulties with logistics and shipping, problems regarding yields or stability of product, contamination or other quality control issues, power failures, and numerous other factors that could prevent us from realizing the intended benefits of our manufacturing strategy and have a material adverse effect on our business.

Furthermore, compliance with cGMP requirements and other quality issues may arise during any internal efforts to scale-up manufacturing, and with our current or any future CMOs. If contaminants are discovered in our supply of our product candidates or in the manufacturing facilities of our CMOs, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability failures or other issues relating to the manufacture of our product candidates will not occur in the future. Additionally, our CMOs may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our CMOs were to encounter any of these difficulties, our ability to provide our product candidate to patients in clinical trials, or to provide product for treatment of patients once approved, would be jeopardized.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial resources, we intend to focus on developing product candidates for specific indications that we identify as most likely to succeed, in terms of both regulatory approval and commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that may prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through

collaboration, licensing or other royalty arrangements in cases in which we would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We have not tested any of our proposed delivery methods or gene editing approaches in clinical trials and any favorable results we may have may not be predictive of results that may be observed in later preclinical studies or clinical trials.

The scientific evidence to support the feasibility of developing product candidates using our RNA editing technology is both preliminary and limited. We have not tested any of our potential delivery modalities or gene editing approaches in clinical trials and any favorable results we may have may not be predictive of results that may be observed in later preclinical studies or clinical trials. For example, we may use LNPs or other delivery modalities to deliver our product candidates. While LNPs have been validated clinically to deliver oligonucleotides, such as siRNA, they have not been clinically proven to deliver oligonucleotides for RNA editing, such as our product candidates.

In addition, our RNA editing technology itself may lead to other issues, such as inability to deliver the desired efficacy or safety-related consequences as it is tested in clinical trials. We have not generated any clinical trial results to date. The design of a clinical trial can determine whether its results will support approval of a product candidate and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. Furthermore, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Many product candidates that initially showed promise in early stage testing for treating a variety of diseases have later been found to lack efficacy or to cause side effects that prevented further clinical development of the product candidates.

Our future product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

We have not evaluated any product candidates in human clinical trials. Moreover, there have been only a limited number of clinical trials involving the use of gene editing technologies and none involving RNA editing technology similar to our technology. It is impossible to predict when, or if, any product candidates we may develop will prove safe in humans. In the genetic medicine field, there have been several significant adverse events from gene therapy treatments in the past, including reported cases of leukemia and death. There can be no assurance that RNA editing technologies will not cause undesirable side effects, such as lymphoma, leukemia, or other cancers, or other aberrantly functioning cells.

If any such adverse events occur, our future clinical trials could be suspended or terminated. If we are unable to demonstrate that any adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any future product candidates for any or all targeted indications. Even if we can demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete any future trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may adversely affect our business, financial condition, results of operations and prospects significantly.

Additionally, if any of our future product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits of the product outweigh its risks, which may include, for example, a Medication Guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners, or other elements to assure safe use of the product. Furthermore, if we or others later identify undesirable side effects caused by any of our future product candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these occurrences may harm our business, financial condition, results of operations and prospects significantly.

If we are unable to successfully identify patients who are likely to benefit from therapy with any product candidates we develop, or experience significant delays in doing so, we may not realize the full commercial potential of any medicines we may develop.

Our success may depend, in part, on our ability to identify patients who are likely to benefit from therapy with any medicines we may develop, which may require those potential patients to have their DNA analyzed for the presence or absence of a particular sequence. If we, or any third parties that we engage to assist us, are unable to successfully identify such patients, or experience delays in doing so, then:

- our ability to develop any product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials; and
- we may not realize the full commercial potential of any product candidates we develop that receive marketing approval if, among other reasons, we are unable to appropriately select patients who are likely to benefit from therapy with our medicines.

As a result of these factors, we may be unable to successfully develop and realize the commercial potential of any product candidates we may identify and develop, and our business, financial condition, results of operations, and prospects would be materially adversely affected.

If we are unable to successfully develop or obtain regulatory approval for companion diagnostic tests for our product candidates, or experience significant delays in doing so, our clinical trials may be delayed and our business could be materially harmed.

The development programs for some of our product candidates contemplate the development of companion diagnostic tests, which are assays or tests to identify an appropriate patient population. If safe and effective use of any of our product candidates we may develop depends on a companion diagnostic, we may not receive marketing approval, or marketing approval may be delayed, if we are unable to or are delayed in developing, identifying, or obtaining regulatory approval or clearance for the companion diagnostic product for use with our product candidate. Identifying a manufacturer of the companion diagnostic and entering into an agreement with the manufacturer could also delay the development of our product candidates.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell any future product candidates, we may be unable to generate any revenues.

We currently do not have an organization for the sales, marketing and distribution of any future product candidates and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. To market any products that may be approved, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. With respect to certain of our current programs as well as future programs, we may rely completely on an alliance partner for sales and marketing. In addition, although we intend to establish a sales organization if we are able to obtain approval to market any product candidates, we may enter into strategic alliances with third parties to develop and commercialize any future product candidates, including in markets outside of the United States or for other large markets that are beyond our resources. This will reduce the revenue generated from the sales of these products.

Any future strategic alliance partners may not dedicate sufficient resources to the commercialization of our product candidates or may otherwise fail in their commercialization due to factors beyond our control. If we are unable to establish effective alliances to enable the sale of our product candidates to healthcare professionals and in geographical regions, including the United States, that will not be covered by our own marketing and sales force, or if our potential future strategic alliance partners do not successfully commercialize the product candidates, our ability to generate revenues from product sales will be adversely affected.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate sufficient product revenue and may not become profitable. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Even if we receive regulatory approval to market our product candidates, the market may not be receptive to our product candidates upon their commercial introduction, which will prevent us from becoming profitable.

Our product candidates are based upon new discoveries, technologies and therapeutic approaches. Key participants in pharmaceutical marketplaces, such as physicians, third-party payors and consumers, may not adopt a product intended to improve

therapeutic results that is based on the technology employed by oligonucleotides. As a result, it may be more difficult for us to convince the medical community and third-party payors to accept and use our product, or to provide favorable reimbursement.

Other factors that we believe will materially affect market acceptance of our product candidates include:

- the timing of our receipt of any regulatory approvals, the terms of any approvals and the countries in which approvals are obtained;
- the ability to consistently manufacture our products within acceptable quality standards;
- the safety and efficacy of our product candidates, as demonstrated in clinical trials and as compared with alternative treatments, if any;
- the incidence, seriousness and severity of any side effects;
- the relative convenience and ease of administration of our product candidates;
- the willingness of patients to accept potentially new routes of administration and their risk tolerance as it relates to potentially serious side effects;
- the success of our physician education programs;
- the availability of government and third-party payor coverage and adequate reimbursement;
- the pricing of our products, particularly as compared to alternative treatments; and
- the availability of alternative effective treatments for the diseases that product candidates we develop are intended to treat and the relative risks, benefits and costs of those treatments.

In addition, our estimates regarding the potential market size may be materially different from what we currently expect by the time we commence commercialization, which could result in significant changes in our business plan and may significantly harm our results of operations and financial condition.

The pharmaceutical industry is intensely competitive. If we are unable to compete effectively with existing drugs, new treatment methods and new technologies, we may be unable to commercialize successfully any drugs that we develop.

The pharmaceutical industry is intensely competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations are pursuing the development of novel drugs for the same diseases that we are targeting or expect to target. Many of our competitors have:

- much greater financial, technical and human resources than we have at every stage of the discovery, development, manufacture and commercialization of products;
- more extensive experience in designing and conducting preclinical studies and clinical trials, obtaining regulatory approvals, and manufacturing, marketing and selling pharmaceutical products;
- product candidates that are based on previously tested or accepted technologies;
- products that have been approved or are in late stages of development; and
- collaborative arrangements in our target markets with leading companies and research institutions.

We will face intense competition from drugs that have already been approved and accepted by the medical community for the treatment of the conditions for which we may develop therapies. We also expect to face competition from new drugs that enter the market. We believe a significant number of drugs are currently under development, and may become commercially available in the future, for the treatment of conditions that our current or future product candidates are or may be designed to treat. These drugs may be more effective, safer, less expensive, or marketed and sold more effectively, than any products we develop.

Our competitors may develop or commercialize products with significant advantages over any products we are able to develop and commercialize based on many different factors, including:

- the safety and effectiveness of our products relative to alternative therapies, if any;
- the ease with which our products can be administered and the extent to which patients accept relatively new routes of administration;

- the timing and scope of regulatory approvals for these products;
- the availability and cost of manufacturing, marketing and sales capabilities;
- price;
- more extensive coverage and higher levels of reimbursement; and
- patent position.

Our competitors may therefore be more successful in commercializing their products than we are, which could adversely affect our competitive position and business. Competitive products may make any products we develop obsolete or noncompetitive before we can recover the expenses of developing and commercializing our product candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute on our business plan.

The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect that coverage and reimbursement by third-party payors will be essential for most patients to be able to afford any gene therapies for which we are able to successfully complete clinical development. Accordingly, sales of any future products will depend substantially, both domestically and internationally, on the extent to which the costs of any such products will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on is investment. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payors, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products in both the United States and globally. Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in the United States, the EU, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as its product candidates. Moreover, increasing efforts by governmental and third-party payors, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of certain third-party payors, such as health maintenance organizations, and additional legislative changes. For an overview and discussion of the regulatory framework for pricing and reimbursement, see Item 1 "*Business—Government Regulation—Patients Rely on Insurance Coverage by Third-Party Payors (third-party payors include Medicare and Medicaid (government payors) and commercial insurance companies such as Blue Cross Blue Shield, Humana, Cigna, etc.) to Pay for Products."*

If the market opportunities for any product candidates we may develop are smaller than we believe they are, our potential revenues may be adversely affected, and our business may suffer.

Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with product candidates we may develop, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. Additionally, our estimates regarding the potential market size may be materially different from what we currently expect by the time we commence commercialization. The number of patients in the United States, Europe, and elsewhere may turn out to be lower than expected, and patients may not be amenable to treatment with our product candidates, or may become increasingly difficult to identify or gain access to, all of which would adversely affect our business, financial condition, results of operations, and prospects.

While we intend to seek designations for our product candidates with the FDA and comparable foreign regulatory authorities that are intended to confer benefits such as a faster development process or an accelerated regulatory pathway, there can be no assurance that we will successfully obtain such designations. In addition, even if one or more of our product candidates are granted such designations, we may not be able to realize the intended benefits of such designations.

The FDA and comparable foreign regulatory authorities offer certain designations for product candidates that are designed to encourage the research and development of product candidates that are intended to address conditions with significant unmet medical need. These designations include fast track, or breakthrough therapy, among others, and may confer benefits such as additional interaction with regulatory authorities, a potentially accelerated regulatory pathway and priority review. However, there can be no assurance that we will successfully obtain such designations for any product candidates. See Item 1 "*Business—Government Regulation—Expedited Development and Review Programs for Drugs*" for more information regarding these designations. While such designations could expedite the development or approval process, they generally do not change the standards for approval. Even if we obtain such designations for one or more of our product candidates, there can be no assurance that we will realize their intended benefits.

In the future, we may also seek approval of product candidates under the FDA's accelerated approval pathway or request priority review. There can be no assurance that FDA would allow any of the product candidates we may develop to proceed on an accelerated approval pathway or grant priority review, and even if FDA did allow such pathway, there can be no assurance that such submission or application will be accepted or that any expedited development, review or approval will be granted on a timely basis, or at all. Moreover, even if we received accelerated approval, any post-approval studies required to confirm and verify clinical benefit may not show such benefit, which could lead to withdrawal of any approvals we have obtained. Receiving accelerated approval does not assure that the product's accelerated approval will eventually be converted to a traditional approval.

In addition, in the EU, we may seek to participate in The PRIority Medicines, or PRIME scheme for our product candidates. The PRIME scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation, where the marketing authorization application will be made through the centralized procedure in the EU. There is no guarantee, however, that our product candidates would be deemed eligible for the PRIME scheme and even if we do participate in the PRIME scheme, where during the course of development a medicine no longer meets the eligibility criteria, support under the PRIME scheme may be withdrawn. PRIME eligibility does not change the standards for product approval, and there is no assurance that any such designation or eligibility will result in expedited review or approval. For more information regarding PRIME and the EU regulatory framework, see Item 1 "*Business—Government Regulation—Regulation Outside of the United States*."

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, or additional global financial crises, could result in a variety of risks to our business, including weakened demand for any future product candidates, if approved, or our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in clinical or commercial supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Our business may be impacted by macroeconomic conditions, including fears concerning the financial services industry, inflation, rising interest rates and volatile market conditions, and other uncertainties beyond our control.

Actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, in March 2023, Silicon Valley Bank Signature Bank and Silvergate Capital Corp. were each swept into receivership by the Federal Deposit Insurance Corporation and then a syndicate of U.S. banks infused $30 billion in First Republic Bank; and later that same week, the Swiss Central Bank provided $54 billion in covered loan and short-term liquidity facilities to Credit Suisse Group AG, all in an attempt to reassure depositors and calm fears of a banking contagion.

Our ability to effectively run our business could be adversely affected by general conditions in the global economy and in the financial services industry. Various macroeconomic factors could adversely affect our business, including fears concerning the banking sector, changes in inflation, interest rates and overall economic conditions and uncertainties. A severe or prolonged economic downturn could result in a variety of risks, including our ability to raise additional funding on a timely basis or on acceptable terms. A weak or declining economy could also impact third parties upon whom we depend to run our business. Increasing concerns over bank failures and bailouts and their potential broader effects and potential systemic risk on the banking sector generally and on the biotechnology industry and its participants may adversely affect our access to capital and our business and operations more generally. Although we assess our banking relationships as we believe necessary or appropriate, our access

to funding sources in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect us, the financial institutions with which we have arrangements directly, or the financial services industry or economy in general.

Risks Related to Regulatory, Legal, and Clinical Trials

Because we are developing oligonucleotides, which are considered a relatively new class of drugs, there is increased risk that the outcome of our clinical trials will not be sufficient to obtain regulatory approval.

The FDA and comparable ex-U.S. regulatory agencies have relatively limited experience with oligonucleotides, which may increase the complexity, uncertainty and length of the regulatory review process for any future product candidates. Even though the FDA issued two draft guidance documents in December 2021 relating to IND submissions for individualized antisense oligonucleotide drugs for severely debilitating or life-threatening genetic diseases, one with clinical focus, the other with chemistry manufacturing and controls focus, and in June 2022 a draft guidance on clinical pharmacology considerations for the development of oligonucleotide therapeutics, the FDA and its foreign counterparts have not yet established any definitive policies, practices or guidelines in relation to overall development considerations for RNA editing oligonucleotide therapies. The general lack of policies, practices or guidelines specific to oligonucleotides may hinder or slow review by the FDA or other foreign homologues of any regulatory filings that we may submit. Moreover, the FDA or other foreign homologues may respond to these submissions by defining requirements we may not have anticipated. Addressing such requirements could lead to significant delays in the development of our product candidates. In addition, because there may be approved treatments for some of the diseases for which we may seek approval, in order to receive regulatory approval, we may need to demonstrate through clinical trials that the product candidates we develop to treat these diseases, if any, are not only safe and effective, but safer or more effective than existing products. Furthermore, in recent years, there has been increased public and political pressure on the FDA with respect to the approval process for new drugs. As a result of the foregoing factors, we may never receive regulatory approval to market and commercialize any product candidate. Even if we obtain regulatory approval, the approval may be for disease indications or patient populations that are not as broad as we intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We may be required to perform additional or unanticipated clinical trials to obtain regulatory approval or be subject to additional post-marketing studies or other requirements to maintain such approval. As a result, we may never succeed in developing a marketable product, we may not become profitable and the value of our common stock could decline.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

Existing regulatory policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability. For more information, see Item 1 "*Business—Governmental Regulation*."

We cannot predict the ultimate content, timing or effect of any healthcare reform legislation or the impact of potential legislation on us. In addition, other legislative changes have been proposed and adopted since the Patient Protection and Affordable Care Act, or the ACA was enacted. We may choose to seek an expanded access program for our product candidates, or to utilize comparable rules in other countries that allow the use of a drug, on a named patient basis or under a compassionate use program.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs and those methods are not always specifically adapted for new technologies such as gene therapy and therapies addressing rare diseases such as those we are developing. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval

process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Because we are developing product candidates in the field of genetic medicines in which there is little clinical experience, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

In order to proceed into clinical development of any product candidates we identify, we will need to submit INDs or comparable foreign applications to regulatory authorities and obtain regulatory clearance to commence clinical development. Because the product candidates we identify are based on novel gene-editing technology, we may be unsuccessful in obtaining clearance from regulatory authorities to proceed into clinical development. In order to commence clinical development, we will need to identify success criteria and endpoints such that the FDA, the EMA or other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop. As we are initially seeking to identify and develop product candidates to treat diseases in which there is little clinical experience using new technologies, and while we may have opportunities to discuss our clinical development plans with regulatory authorities prior to commencing clinical development, there is heightened risk that the FDA, the EMA or other regulatory authorities may not consider the clinical trial endpoints that we propose to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze.

Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. Furthermore, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the EU and other countries may make similar comments with respect to these endpoints and data. Any product candidates we may develop will be based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No gene editing therapeutic product has been approved in the United States or in Europe. Within the broader genome product field, only a limited number of gene therapy products, such as uniQure N.V.'s Glybera and Abecma from Bristol Myers Squibb and bluebird bio, have received marketing authorization or marketing approval from the European Commission or the FDA. Some of these products have taken years to register and have had to deal with significant issues in their post-marketing experience.

If preclinical studies or clinical trials of any product candidates we may identify and develop fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of any product candidates we may identify and develop, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates.

We and our collaborators, if any, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize any product candidates we may identify and develop, including:

- delays in reaching a consensus with regulators on trial design;
- regulators, IRBs, or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching or failing to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective CROs and clinical trial sites;

- clinical trials of any product candidates we may develop may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development or research programs;
- difficulty in designing well-controlled clinical trials due to ethical considerations that may render it inappropriate to conduct a trial with a control arm that can be effectively compared to a treatment arm;
- difficulty in designing clinical trials and selecting endpoints for diseases that have not been well-studied and for which the natural history and course of the disease is poorly understood;
- the number of patients required for clinical trials of any product candidates we may develop may be larger than we anticipate; enrollment of suitable participants in these clinical trials, which may be particularly challenging for some of the rare genetically defined diseases we are targeting in our most advanced programs, may be delayed or slower than we anticipate; or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, IRBs, or independent ethics committees may require that we or our investigators suspend or terminate clinical research or clinical trials of any product candidates we may develop for various reasons, including noncompliance with regulatory requirements, a finding of undesirable side effects or other unexpected characteristics, or that the participants are being exposed to unacceptable health risks or after an inspection of our clinical trial operations or trial sites;
- the cost of clinical trials of any product candidates we may develop may be greater than we anticipate;
- the supply or quality of any product candidates we may develop or other materials necessary to conduct clinical trials of any product candidates we may develop may be insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing, and delivery of any product candidates we may develop to the clinical sites by us or by third parties with whom we have contracted to perform certain of those functions;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with any product candidates we may develop that are viewed to outweigh their potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; and
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

If we or our collaborators are required to conduct additional clinical trials or other testing of any product candidates we may develop beyond those that we currently contemplate, if we or our collaborators are unable to successfully complete clinical trials or other testing of any product candidates we may develop, or if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we or our collaborators may:

- be delayed in obtaining marketing approval for any such product candidates we may develop or not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on our distribution in the form of a REMS or through modification to an existing REMS;

- be sued; or
- experience damage to our reputation.

Product development costs will also increase if we or our collaborators experience delays in clinical trials or other testing or in obtaining marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop, could allow our competitors to bring products to market before we do, and could impair our ability to successfully commercialize any product candidates we may develop, any of which may harm our business, financial condition, results of operations, and prospects.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

Clinical trials of a new product candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease the product candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, including the COVID-19 global endemic or similar events, the stage and severity of disease, the nature and requirements of the protocol, the proximity of patients to clinical sites, the availability of effective treatments for the relevant disease, and the eligibility criteria for the clinical trial. Delays or difficulties in patient enrollment or difficulties retaining trial participants, including as a result of the availability of existing or other investigational treatments, can result in increased costs, longer development times or termination of a clinical trial.

Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.

Prior to commercialization, any of our product candidates must be approved by the FDA pursuant to a new drug application, or NDA, in the United States and pursuant to similar marketing applications by the EMA and similar regulatory authorities outside the United States. The process of obtaining marketing approvals, both in the United States and abroad, is expensive and takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have no experience in submitting and supporting the applications necessary to gain marketing approvals, and, in the event regulatory authorities indicate that we may submit such applications, we may be unable to do so as quickly and efficiently as desired.

Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude us from obtaining marketing approval or prevent or limit commercial use. Regulatory authorities have substantial discretion in the approval process and may refuse to accept or file any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate.

Approval of any of our product candidates may be delayed or refused for many reasons, including:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- We may be unable to demonstrate, to the satisfaction of the FDA or comparable foreign regulatory authorities, that our product candidates are safe and effective for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- We may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical programs or clinical trials;

- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the facilities of third-party manufacturers with which we contract or procure certain service or raw materials, may not be adequate to support approval of our product candidates; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and adversely affect our business, financial condition, results of operations and prospects.

We may be unable to obtain regulatory approval in the United States or foreign jurisdictions and, as a result, be unable to commercialize our product candidates and our ability to generate revenue will be materially impaired.

Our product candidates are subject to extensive governmental regulations relating to, among other things, research, testing, development, manufacturing, quality, safety, efficacy, approval, recordkeeping, reporting, labeling, storage, packaging, advertising and promotion, pricing, marketing and distribution of drugs. Rigorous preclinical studies and clinical trials, and an extensive regulatory approval process are required to be successfully completed in the United States and in many foreign jurisdictions before a new drug can be marketed. Satisfaction of these and other regulatory requirements is costly, time consuming, uncertain, and subject to a continuously evolving regulatory environment and unanticipated delays. It is possible that none of the product candidates we may develop will obtain the regulatory approvals necessary for us or our collaborators to begin selling them.

The time required to obtain FDA and other approvals is unpredictable but typically takes many years following the commencement of clinical trials, depending upon the type, complexity and novelty of the product candidate. The standards that the FDA and its foreign counterparts use when regulating companies such as ours are not always applied predictably or uniformly and can change. Any analysis we perform of data from chemistry, manufacturing and controls, preclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent regulatory approval. We may also encounter unexpected delays or increased costs due to new government regulations, for example, from future legislation or administrative action, or from changes in FDA policy during the period of product development, clinical trials and FDA regulatory review. It is impossible to predict whether legislative changes will be enacted, or whether FDA or foreign regulations, guidance or interpretations will be changed, or what the impact of such changes, if any, may be.

Any delay or failure in obtaining required approvals could adversely affect our ability to generate revenues from the particular product candidate for which we are seeking approval. Furthermore, any regulatory approval to market a product may be subject to limitations on the approved uses for which we may market the product or the labeling or other restrictions. In addition, the FDA has the authority to require a REMS as a condition of approval, which may impose further requirements or restrictions on the distribution or safe use of an approved drug, such as limiting prescribing rights to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients as specially defined by the indication statement or who meet certain safe-use criteria, and requiring treated patients to enroll in a registry, among other requirements. These limitations and restrictions may limit the size of the market for the product and affect reimbursement by third-party payors.

We are also subject to numerous foreign regulatory requirements governing, among other things, the conduct of clinical trials, manufacturing and marketing authorization, pricing and payment. The foreign regulatory approval process varies among countries and may include all of the risks associated with FDA approval described above, as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Approval by the FDA does not ensure approval by comparable regulatory authorities outside of the United States and vice versa.

Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.

Our product candidates and the activities associated with their development and potential commercialization, including their testing, manufacturing, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other U.S. and international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, including cGMPs, quality control, quality assurance and corresponding maintenance of records and documents, including periodic inspections by the FDA and other regulatory authorities and requirements regarding the distribution of samples to providers and recordkeeping.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of any approved product. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding use of their products. If we promote our product candidates in a manner inconsistent with FDA-approved labeling or otherwise not in compliance with FDA regulations, we may be subject to enforcement action. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws and similar laws in international jurisdictions.

In addition, later discovery of previously unknown adverse events or other problems with our product candidates, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such product candidates, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of any approved product from the market;
- refusal to approve pending applications or supplements to approved applications that we may submit;
- recall of product candidates;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our product candidates;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Even if we obtain regulatory approvals, our marketed drugs will be subject to ongoing regulatory oversight. If we fail to comply with continuing U.S. and foreign requirements, our approvals, if obtained, could be limited or withdrawn, we could be subject to other penalties, and our business would be seriously harmed.

Following any initial regulatory approval of any drugs we may develop, we will also be subject to continuing regulatory oversight, including the review of adverse drug experiences and safety data that are reported after our drug products are made commercially available. This would include results from any post-marketing studies or surveillance to monitor the safety and efficacy of the drug product required as a condition of approval or agreed to by us. Any regulatory approvals that we receive for

our product candidates may also be subject to limitations on the approved uses for which the product may be marketed. Other ongoing regulatory requirements include, among other things, submissions of safety and other post-marketing information and reports, registration and listing, as well as continued maintenance of our marketing application, compliance with cGMP requirements and quality oversight, compliance with post-marketing commitments, and compliance with GCP for any clinical trials that we conduct post-approval. Failure to comply with these requirements could result in warning or untitled letters, criminal or civil penalties, recalls, or product withdrawals. In addition, we intend to seek approval to market our product candidates in jurisdictions outside of the United States, and therefore will be subject to, and must comply with, regulatory requirements in those jurisdictions.

The FDA has significant post-market authority, including, for example, the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials for a variety of reasons. The FDA also has the authority to require a REMS plan after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug.

We, our CMOs, and the manufacturing facilities we use to make our product candidates will also be subject to ongoing assessment of product quality, compliance with cGMP, and periodic inspection by the FDA and potentially other regulatory agencies. We or our CMOs may not be able to comply with applicable cGMP regulations or similar regulatory requirements outside of the United States. Our failure, or the failure of our CMOs, to comply with applicable regulations could result in regulatory actions, such as the issuance of FDA Form 483 notices of observations, warning letters or sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products. We may not have the ability or capacity to manufacture material at a broader commercial scale in the future. We and our CMOs currently manufacture a limited supply of clinical trial materials. Reliance on CMOs entails risks to which we would not be subject if we manufactured all of the material ourselves, including reliance on the CMO for regulatory compliance. Our product promotion and advertising will also be subject to regulatory requirements and continuing regulatory review.

If we or our collaborators, manufacturers or service providers fail to comply with applicable continuing regulatory requirements in the United States or foreign jurisdictions in which we may seek to market our products, we or they may be subject to, among other things, fines, warning letters, holds on clinical trials, refusal by the FDA or comparable foreign regulatory authorities to approve pending applications or supplements to approved applications, suspension or withdrawal of regulatory approval, product recalls and seizures, refusal to permit the import or export of products, operating restrictions, injunction, consent decree, civil penalties and criminal prosecution.

Any drugs we develop may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.

Because our product candidates represent new approaches to the treatment of genetic-based diseases, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, our product candidates or assure that coverage and reimbursement will be available for any product that we may develop. The regulations that govern marketing approvals, pricing and reimbursement for new drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. We are monitoring these regulations as several of our programs move into later stages of development; however, many of our programs are currently in the earlier stages of development and we will not be able to assess the impact of price regulations for a number of years. As a result, we might obtain regulatory approval for a product in a particular country, but then be subject to price regulations that could delay our commercial launch of the product and negatively impact any potential revenues we may be able to generate from the sale of the product in that country and potentially in other countries due to reference pricing. For more information, see Item 1 "*Business – Government Regulation – No Uniform Policy Exists for Coverage and Reimbursement in the U.S.*" and "*– Patients Rely on Insurance Coverage by Third-Party Payors (third-party payors include Medicare and Medicaid (government payors) and commercial insurance companies such as Blue Cross Blue Shield, Humana, Cigna, etc.) to Pay for Products.*"

Our ability to commercialize any products successfully will also depend in part on the extent to which coverage and adequate reimbursement/payment for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Even if we succeed in bringing one or more products to the market, these products may not be considered medically necessary and/or cost-effective, and the amount reimbursed for any products may be insufficient to allow us to sell our products on a competitive basis. At this time, we are unable to determine their cost effectiveness or the likely level or method of reimbursement for our product candidates. Increasingly, third-party payors, such as government and private insurance plans, are requiring that drug companies provide them with predetermined discounts from list prices, and are seeking to reduce the prices charged or the amounts paid for pharmaceutical products. If the price we are

able to charge for any products we develop, or the payments provided for such products, is inadequate in light of our development and other costs, our return on investment could be adversely affected.

There may be significant delays in obtaining coverage for newly-approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or comparable foreign regulatory authorities. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to pay all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and payment is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate payment is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. Moreover, eligibility for coverage does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim payments for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement may be based on payments allowed for lower-cost drugs that are already reimbursed, may be incorporated into existing payments for other services and may reflect budgetary constraints or imperfections in Medicare data. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates. However, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payors in the United States. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for new drugs that we develop and for which we obtain regulatory approval could adversely affect our operating results, our ability to raise capital needed to commercialize products, and our overall financial condition.

We believe that the efforts of governments and third-party payors to contain or reduce the cost of healthcare and legislative and regulatory proposals to broaden the availability of healthcare will continue to affect the business and financial condition of pharmaceutical and biopharmaceutical companies. A number of legislative and regulatory changes in the healthcare system in the United States and other major healthcare markets have been proposed and/or adopted in recent years, and such efforts have expanded substantially in recent years. For more information on these changes, see Item 1 "*Business—Governmental Regulation—Affordable Care Act and Legislative Reform Measures*."

We may not be able to obtain orphan drug exclusivity for one or more of our product candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is a drug intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan product candidates by the EMA in the EU. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for another product candidate for the same orphan therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the EU. The exclusivity period in the EU can be reduced to six years if a product no longer meets the criteria for orphan designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

The FDA's standards for granting orphan drug exclusivity in the gene therapy context are unclear and evolving. In order for the FDA to grant orphan drug exclusivity to one of our product candidates, the agency must find that the product candidate is indicated for the treatment of a condition or disease that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product candidate available for the disease or condition will be recovered from sales of the product in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different product candidates can be approved for the same condition. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product candidate for the same condition if the FDA concludes that the later product candidate is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care compared with the product that has orphan exclusivity. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

In August 2017, the Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

If we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.

Our research, development and manufacturing processes involve the use of hazardous materials. We maintain quantities of various flammable and toxic chemicals in our facilities that are required for our research, development and manufacturing activities. We are subject to federal, state and local laws and regulations governing the use, manufacture, storage, handling and disposal of these hazardous materials. Our procedures for storing, handling and disposing of these materials are reviewed against the relevant guidelines and laws of the jurisdictions in which our facilities are located on a regular basis. Although we believe that our safety procedures for handling and disposing of these materials sufficiently mitigate the risk of accidental contamination or injury from these materials, the risk cannot be completely eliminated. If an accident occurs, we could be held liable for resulting damages, which could be substantial. We are also subject to numerous environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of biohazardous materials. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of these materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological or hazardous materials. Additional federal, state and local laws and regulations affecting our operations may become applicable in the future. We may incur substantial costs to comply with, and substantial fines or penalties if we violates any of, these laws or regulations.

If we or our collaborators, manufacturers, service providers or other third parties fail to comply with applicable healthcare laws and regulations, we could be subject to enforcement actions, which could affect our ability to develop, market and sell our products and may harm our reputation.

We are currently, or may in the future, be subject to federal, state, local, and comparable foreign healthcare laws and regulations relating to areas such as fraud and abuse and patients' rights. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our products for which we obtain marketing approval. For more information on these laws, see Item 1 "*Business—Governmental Regulation—Other Healthcare Laws*."

If our operations are found to be in violation of any such requirements, we may be subject to penalties, including civil or criminal penalties, criminal prosecution, monetary damages, the curtailment or restructuring of our operations, loss of eligibility to obtain approvals from the FDA, exclusion from participation in federal healthcare programs including Medicare and Medicaid, the imposition of a corporate integrity agreement with the Office of Inspector General of the Department of Health and Human Services, disgorgement, individual imprisonment, contractual damages, reputational harm, and diminished profits and future earnings, any of which could adversely affect our financial results and adversely affect our ability to operate our business. We intend to develop and implement a comprehensive corporate compliance program prior to the commercialization of our product candidates. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses, could divert our management's attention from the operation of our business, and could harm our reputation, even if our defense is successful. In addition, achieving and sustaining compliance with applicable laws and regulations may be costly to us in terms of money, time and resources.

If we or our collaborators, manufacturers or service providers fail to comply with applicable federal, state or foreign laws or regulations, we could be subject to enforcement actions, which could affect our ability to develop, market and sell our products successfully and could harm our reputation and lead to reduced acceptance of our products by the market. These enforcement actions include, among others:

- adverse regulatory inspection findings;
- warning and/or untitled letters;

- voluntary or mandatory product recalls or public notification or medical product safety alerts to healthcare professionals;
- restrictions on, or prohibitions against, marketing our products;
- restrictions on, or prohibitions against, importation or exportation of our products;
- suspension of review or refusal to approve pending applications or supplements to approved applications;
- exclusion from participation in government-funded healthcare programs;
- exclusion from eligibility for the award of government contracts for our products;
- suspension or withdrawal of product approvals;
- product seizures;
- injunctions;
- consent decrees; and
- civil and criminal penalties, up to and including criminal prosecution resulting in fines, exclusion from healthcare reimbursement programs and imprisonment.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Moreover, federal, state or foreign laws or regulations are subject to change, and while we, our collaborators, manufacturers and/or service providers currently may be compliant, that could change due to changes in interpretation, prevailing industry standards or other reasons.

Our employees, consultants and collaborators may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of fraud and other misconduct by our employees, consultants and collaborators. Such misconduct could include intentional failures to comply with FDA and other foreign agency regulations, provide accurate information to the FDA, comply with manufacturing standards required by the FDA or us, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any medicines that we may develop.

We face an inherent risk of product liability exposure related to the testing in human clinical trials of any product candidates we may develop and will face an even greater risk if we commercially sell any medicines that we may develop. If we cannot successfully defend ourself against claims that our product candidates or medicines caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or medicines that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;

- significant time and costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any medicines that we may develop.

We anticipate that we will need to increase our insurance coverage when we begin clinical trials and if we successfully commercialize any medicine. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Our internal computer and information systems, or those used by our CROs, CMOs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our development programs.

Despite the implementation of appropriate security measures, our internal computer and information systems and those of our current and any future CROs, CMOs and other contractors or consultants may become vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, or accident, and are unaware of any security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of data from completed or future preclinical studies or clinical trials could result in significant delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be significantly delayed.

We may be unable to adequately protect our information systems from cybersecurity incidents, which could result in the disclosure of confidential information, damage our reputation, and subject us to significant financial and legal exposure.

Cybersecurity incidents are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cybersecurity incidents could include wrongful conduct by hostile foreign governments, industrial espionage, wire fraud and other forms of cyber fraud, the deployment of harmful malware, denial-of-service, social engineering fraud or other means to threaten data confidentiality, integrity and availability. A cybersecurity incident could cause serious negative consequences for us, including, without limitation, the disruption of operations, the misappropriation of confidential business information, including financial information, trade secrets, financial loss and the disclosure of corporate strategic plans. To date, we have not experienced a material compromise of our data or information systems. However, although we devote resources to protect our information systems, we realize that cybersecurity incidents are a threat, and there can be no assurance that our efforts will prevent information security breaches that would result in business, legal, financial or reputational harm to us, or would have a material adverse effect on our results of operations and financial condition.

Our failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our product candidates outside the United States.

To market and sell our future product candidates in other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, we must secure product reimbursement approvals before regulatory authorities will approve the product for sale in that country. Failure to obtain foreign regulatory approvals or non-compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

If we fail to comply with the regulatory requirements in international markets and receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business will be adversely affected. We may not obtain foreign regulatory approvals on a timely basis, if at all. Our failure to obtain approval of any of our product candidates by regulatory authorities in another country may significantly diminish the commercial prospects of that product candidate and our business prospects could decline.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved, or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs or modifications to approved drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

We, our collaborators and our service providers may be subject to a variety of privacy and data security laws, regulations and contractual obligations, and our failure to comply with them could harm our business.

We maintain a large quantity of sensitive information, including confidential business and patient health information in connection with our preclinical studies, and are subject to laws and regulations governing the privacy and security of such information. In the United States, there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, and federal and state consumer protection laws. Each of these laws is subject to varying interpretations and new laws continue to be proposed. Outside of the United States, many jurisdictions have enacted stringent privacy and data protection laws. The collection, use, disclosure, transfer or other processing of personal data originating from the European Economic Area, or EEA, and United Kingdom, or UK, is governed by the General Data Protection Regulation, or EU GDPR, and the UK General Data Protection Regulation, or UK GDPR, which, together with the EU GDPR, is referred to as the GDPR. For additional information on these regimes, see Item 1 "*Business—Government Regulation—Privacy and Cybersecurity*". Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms to ensure compliance, and despite those efforts, if we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our reputation, business, financial condition and results of operations.

The use of new and evolving technologies, such as artificial intelligence, or AI, in our offerings may result in spending material resources and presents risks and challenges that can impact our business including by posing security and other risks to our confidential information, proprietary information and personal information, and as a result we may be exposed to reputational harm and liability.

The use and integration of AI presents risks and challenges that could affect its adoption, and therefore our business. The use of certain artificial intelligence technology can give rise to intellectual property risks, including compromises to proprietary intellectual property and intellectual property infringement. Additionally, we expect to see increasing government and supranational regulation related to artificial intelligence use and ethics, which may also significantly increase the burden and cost of research, development and compliance in this area. For example, the EU's Artificial Intelligence Act, or AI Act, is anticipated to enter into force in 2024 and, with some exceptions, become effective 24 months thereafter. This legislation imposes significant obligations on providers and deployers of high risk artificial intelligence systems, and encourages providers and deployers of artificial intelligence systems to account for EU ethical principles in their development and use of these systems. If we develop or deploy AI systems that are governed by the AI Act, we may be required to adopt higher standards of data quality, transparency, and human oversight, and adhere to specific and potentially burdensome and costly ethical, accountability, and administrative requirements. The rapid evolution of AI will require the application of significant resources to help ensure that AI is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. Our vendors may in turn incorporate AI tools into their own offerings, and the providers of these AI tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of AI, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

Risks Related to Our Third Party Relationships

We may be subject to claims that we or our employees or consultants have wrongfully used or disclosed alleged trade secrets of employees' or consultants' former employers or their clients. These claims may be costly to defend and if we do not successfully do so, we may be required to pay monetary damages and may lose valuable intellectual property rights or personnel.

Many of our employees were previously employed at universities or biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although no claims against us are currently pending, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. A loss of key research personnel or their work product could hamper our ability to commercialize, or prevent us from commercializing, our product candidates, which could severely harm our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

We expect to rely on third parties to conduct our clinical trials and some aspects of our research, as well as some aspects of our delivery methods, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.

We currently, and expect to continue to, rely on third parties, such as CROs, clinical data management organizations, medical institutions, preclinical laboratories and clinical investigators, to conduct some aspects of our research. For example, we may rely on a third party to supply LNPs, or to conduct some of our preclinical animal experiments. Any of these third parties may terminate their engagements with us at any time under certain criteria. If we need to enter into alternative arrangements, we may delay our product development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA, the EMA and other regulatory authorities require us and the study sites and investigators we work with to comply with standards, commonly referred to as GLPs and GCPs for conducting, recording and reporting the results of preclinical studies and clinical trials to assure, amongst other things, that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected.

We may not be successful in finding strategic collaborators for continuing development of certain of our future product candidates or successfully commercializing or competing in the market for certain indications.

In the future, we may decide to collaborate with non-profit organizations, universities, pharmaceutical and biotechnology companies for the development and potential commercialization of existing and new product candidates. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for its product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay our development program or one or more of our other development programs, delay our potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have

sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

The success of any potential collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of such collaboration arrangements. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into ours;
- the need to implement or improve controls, procedures, and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities, and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm our business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

We rely, and anticipate that we will rely, on third parties to design, conduct, supervise and monitor our preclinical studies and clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We rely, and anticipate that we will rely, on third party clinical investigators, CROs, clinical data management organizations and consultants to design, conduct, supervise and monitor preclinical studies and clinical trials of our product candidates. Because we rely on third parties and do not have the ability to conduct preclinical studies or clinical trials independently, we have less control over the timing, quality and other aspects of preclinical studies and clinical trials than we would if we conducted them on own, including our inability to control whether sufficient resources are applied to our programs. If any of our CROs are acquired or consolidated, these concerns are likely to be exacerbated and our preclinical studies or clinical trials may be further impacted due to potential integration, streamlining, staffing and logistical changes. These investigators,

CROs and consultants are not our employees and we have limited control over the amount of time and resources that they dedicate to our programs. These third parties may have contractual relationships with other entities, some of which may be our competitors, which may draw time and resources from our programs. Further, these third parties may not be diligent, careful or timely in conducting our preclinical studies or clinical trials, resulting in the preclinical studies or clinical trials being delayed or unsuccessful.

If we cannot contract with acceptable third parties on commercially reasonable terms, or at all, or if these third parties do not carry out their contractual duties, satisfy legal and regulatory requirements for the conduct of preclinical studies or clinical trials or meet expected deadlines, our preclinical and clinical development programs could be delayed and otherwise adversely affected. In all events, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. The FDA and other health authorities require certain preclinical studies to be conducted in accordance with GLP, and clinical trials to be conducted in accordance with GCP, including conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. If we or our CROs fail to comply with these requirements, the data generated in our clinical trials may be deemed unreliable or uninterpretable and the FDA and other health authorities may require us to perform additional clinical trials. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. In the United States, we are also required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. Any such event could adversely affect our business, financial condition, results of operations and prospects.

We rely on third parties in the supply and manufacture of our product candidates for our research, preclinical and clinical activities, and may do the same for commercial supplies of our product candidates.

We have not yet manufactured our product candidates on a commercial scale, and may not be able to do so for any of our product candidates. We currently rely on third parties in the supply and manufacture of materials for our research, preclinical and clinical activities and may continue to do so for the foreseeable future, including if we received regulatory approval for any product candidate. We may do the same for the commercial supply of our drug product. We use third parties to perform additional steps in the manufacturing process, such as the filling, finishing and labeling of vials and storage of our product candidates and we expect to do so for the foreseeable future. There can be no assurance that our supply of research, preclinical and clinical development drug candidates and other materials will not be limited, interrupted or restricted or will be of satisfactory quality or continue to be available at acceptable prices. Replacement of any of the third parties we may engage could require significant effort and expertise because there may be a limited number of qualified replacements. In addition, raw materials, reagents, and components used in the manufacturing process, particularly those for which we have no other source or supplier, may not be available, may not be suitable or acceptable for use due to material or component defects, or may introduce variability into the supply of our product candidates. Furthermore, with the increase of companies developing nucleic acid therapeutics, there may be increased competition for the supply of the raw materials that are necessary to make our oligonucleotides, which could severely impact the manufacturing of our product candidates.

We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and they must be acceptable to the FDA or approved by foreign regulatory authorities. Suppliers and manufacturers, including us, must meet applicable manufacturing requirements, including compliance with cGMP regulations, and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory standards. In the event that any of our suppliers or manufacturers fail to comply with such requirements or to perform their obligations to us in relation to quality, timing or otherwise, some of which may be out of their or our control, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to increase the manufacturing of the materials ourself, for which we currently have limited capabilities and resources, or enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. Any interruption of the development or operation of the manufacturing of our product candidates, such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility resulting from natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available product candidates or materials. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills or technology to another third party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third party manufacture our product candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget.

We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties which could have a material adverse effect on our business prior to or after commercialization of any of our product candidates. If we are unable to obtain or maintain third-party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Failure to execute on our manufacturing requirements, either by us or by one of our third-party vendors, could adversely affect our business.

Our relationships with healthcare providers, physicians, and third-party payors will be subject to applicable anti-kickback, fraud and abuse, anti-bribery and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, and diminished profits and future earnings.

Healthcare providers, physicians, and third-party payors play a primary role in the recommendation and prescription of any product candidates that we may develop for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell, and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations listed in the section above titled "*Risks Related to Regulatory, Legal, and Clinical Trials*", including certain laws and regulations applicable only if we have marketed products.

Some state laws also require pharmaceutical companies to comply with specific compliance standards, restrict financial interactions between pharmaceutical companies and healthcare providers or require pharmaceutical companies to report information related to payments to health care providers or marketing expenditures.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Given the breadth of the laws and regulations, limited guidance for certain laws and regulations and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices may not comply with healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations, and prospects.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order, or use of medicinal products is prohibited in the EU. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the U.K. Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization, and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes, or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Risks Related to Our Personnel, Operations and Growth

If we are unable to attract and retain qualified key management and scientists, staff, consultants and advisors, our ability to implement our business plan may be adversely affected.

We are highly dependent upon our senior management and our scientific, clinical and medical staff and advisors. The loss of the service of any of the members of our senior management or other key employees could delay our research and development programs and materially harm our business, financial condition, results of operations and prospects. In addition, we expect that we will continue to have an increased need to recruit and hire qualified personnel as we advance our programs and expand operations. Failure to successfully recruit and retain personnel could impact our anticipated development plans and timelines. For example, as a result of the COVID-19 endemic, we have faced challenges in retaining and attracting employees to support our research and development efforts, and our failure to do so could have an adverse effect on our ability to execute on our business plan. We are dependent on the continued service of our technical personnel because of the highly technical and novel nature of our product candidates, platform and technologies and the specialized nature of the regulatory approval process. Replacing such personnel may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully execute our business strategy, and we cannot assure you that we will be able to identify or employ qualified personnel for any such position on acceptable terms, if at all. Many of the

biotechnology and pharmaceutical companies with whom we compete for qualified personnel have greater financial and other resources, different risk profiles and longer histories in the industry than we do. Because our management team and key employees are not obligated to provide us with continued service, they could terminate their employment with us at any time without penalty. We do not maintain key person life insurance policies on any of our management team members or key employees. Our future success will depend in large part on our continued ability to attract and retain highly qualified scientific, technical and management personnel, as well as personnel with expertise in preclinical and clinical testing, manufacturing, governmental regulation and commercialization. In order to do so, we may need to pay higher compensation or fees to our employees or consultants than we currently expect, and such higher compensation payments may have a negative effect on our operating results. We face increased competition for personnel from other companies, universities, public and private research institutions, government entities and other organizations. If we are unable to attract and retain qualified personnel, the rate and success at which we may be able to discover and develop our product candidates and implement our business plan will be limited.

We expect to expand our research, development, delivery, manufacturing, commercialization, regulatory and future sales and marketing capabilities over time, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of December 31, 2023, we had 101 full-time employees, including 34 who hold Ph.D. degrees, and one part-time employee; 74 employees are engaged in research and development and 27 employees in management or general and administrative activities. In connection with the growth and advancement of our pipeline and becoming a public company through the Merger, we expect to increase the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities, and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expected expansion of our operations or recruit and train additional qualified personnel. Moreover, our current physical laboratory space may be insufficient for our near-term research and development hiring plans, and the expected physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a growing biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for and fully understanding the regulatory and manufacturing pathways to all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our potential product candidates. If our management is unable to effectively manage the expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize any product candidates we may develop will depend in part on our ability to effectively manage our future development and expansion.

Risks Related to Intellectual Property

If we are not able to obtain or protect intellectual property rights related to any of our product candidates, development and commercialization of our product candidates may be adversely affected.

In our industry, the majority of an innovative product's commercial value is usually realized during the period in which it has market exclusivity. Market exclusivity is comprised of both patent and other intellectual property protection, as well as regulatory exclusivity. In the United States and some other countries, when market exclusivity expires and generic versions of a product are approved and marketed, there usually are very substantial and rapid declines in the product's sales. Accordingly, our success depends in part on our ability to obtain and maintain patents and other forms of intellectual property rights, including trademarks, trade secrets and, where necessary in-licenses of intellectual property rights of others, in the United States and in other countries for our product candidates and platform technologies, as well as for methods used to manufacture our product candidates, and methods for treating patients for approved indications using our product candidates, as well as our ability to preserve our trade secrets, to prevent third parties from infringing upon our proprietary rights and to operate without infringing upon the proprietary rights of others. Certain research and development activities involved in pharmaceutical development are exempt from patent infringement in the United States and other jurisdictions, for example, in the United States by the provisions

of 35 U.S.C. § 271I(1), or the Safe Harbor. However, in the United States and certain other jurisdictions, the Safe Harbor exemption can terminate when the sponsor submits an application for marketing approval (e.g., a New Drug Application, or NDA, in the United States). Therefore, the risk that a third party might allege patent infringement may increase as our product candidates approach commercialization.

We cannot offer any assurances about which of our patent applications will issue, the breadth of any resulting patent or whether any of the issued patents will be found invalid and unenforceable or will be threatened by third parties. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us in the future after patent issuance could deprive us of rights necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

We may not be able to apply for patents or obtain patent protection on certain aspects of our product candidates or our RNA editing platform OPERA in a timely fashion or at all. The patent prosecution process is expensive and time-consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. We may not be able to obtain or maintain patent applications and patents due to the subject matter claimed in such patent applications and patents being in the public domain.

Our existing issued and granted patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technology. There is no guarantee that any of our pending patent applications will result in issued or granted patents, that any of our issued or granted patents will not later be found to be invalid or unenforceable, or that any issued or granted patents will include claims that are sufficiently broad to cover our product candidates, platform technologies, or any methods relating to them, or to provide meaningful protection from competitors. Consequently, it is unknown whether our platform technology or product candidates will be protectable or remain protected by valid and enforceable patents. Any failure to obtain, maintain or defend our patents and other intellectual property could have a material adverse effect on our business, financial conditions, results of operations and prospects.

We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if they are not, we may be subject to entitlement disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. Because patent applications in the United States and other countries are confidential for a period of time after filing, at any moment in time, we cannot be certain that we were in the past or will be in the future the first to file any patent application related to our product candidates. For example, some patent applications in the United States may be maintained in secrecy until the patents are issued. Further, publications in the scientific literature often lag behind actual discoveries. Consequently, we cannot be certain that others have not filed patent applications for technology covered by our owned and issued patents or pending applications, or that we or, if applicable, a licensor were the first to invent or first to file an application for the technology.

The patent position of biotechnology and pharmaceutical companies can be highly uncertain and involves complex legal and factual questions. As a result, the issuance, scope, validity, enforceability and commercial value of any patent rights are highly uncertain. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our current and future proprietary technology and product candidates are covered by valid and enforceable patents or are effectively maintained as trade secrets. If third parties disclose or misappropriate our proprietary rights, it may materially and adversely impact our position in the market.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If there are material defects in the form, preparation, prosecution, maintenance or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

Legal issues related to the patentability of biopharmaceuticals, and methods of their manufacture and use, are complex and uncertain in some countries. In some countries, applicants are not able to protect methods of treating human beings or medical treatment processes. Intellectual property protection varies throughout the world and is subject to change over time. Certain jurisdictions have enacted various rules and laws precluding issuance of patents encompassing any methods a doctor may practice on a human being or any other animal to treat a disease or condition. Further, many countries have enacted laws and regulatory regimes that do not allow patent protection for methods of use of known compounds. Thus, in some countries and jurisdictions, it

may not be possible to patent some of our product candidates at all. In some countries and jurisdictions, only composition claims may be obtained, and only when those compositions are or contain compounds that are new and/or novel. Also, patents issued with composition claims (*i.e.,* covering product candidates) cannot always be enforced to protect methods of using those compositions to treat or diagnose diseases or medical conditions. Legal systems in certain countries may not favor enforcement or protection of patents, trade secrets and other intellectual property. Lack of intellectual property protection in such cases may have a materially adverse effect on our business and financial condition.

Furthermore, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates, their manufacture or their use might expire before or shortly after those candidates receive regulatory approval and are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. We expect to seek extensions of patent terms where these are available upon regulatory approval in those countries where we are prosecuting patents. This includes in the United States under the Drug Price Competition and Patent Term Restoration Act of 1984, which permits a patent term extension of up to five years beyond the expiration of the patent. However, the applicable authorities, including the FDA in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be possible.

The U.S. Patent and Trademark Office, or USPTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent prosecution process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, or loss of right to enforce patent claims, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case. The standards applied by the USPTO and foreign patent offices in granting patents are not uniform, can vary substantially from country to country, and are not always applied predictably, requiring country-specific patent expertise in each jurisdiction in which patent protection is sought. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. As such, we do not know the degree of future protection that we will have on our product candidates and RNA editing technology. While we will endeavor to try to protect our product candidates and RNA editing technology with intellectual property rights such as patents, as appropriate, the process of filing and prosecuting patent applications, and obtaining, maintaining and defending patents is time-consuming, expensive, uncertain, and sometimes unpredictable.

Our pending patent applications may not issue as patents, and even issued patents may not provide sufficient protection of our RNA editing platform OPERA and our product candidates and issued patents may not provide.

In addition to claims directed toward the technology underlying our OPERA platform, our patents and patent applications contain claims directed to compositions of matter on the active pharmaceutical ingredients, or APIs, in our product candidates, as well as methods-of-use directed to the use of an API for a specified treatment. Composition-of-matter patents on the active pharmaceutical ingredient in prescription drug products provide protection without regard to any particular method of use of the API used. Method-of-use patents do not prevent a competitor or other third party from developing or marketing an identical product for an indication that is outside the scope of the patented method. Moreover, with respect to method-of-use patents, even if competitors or other third parties do not actively promote their product for our targeted indications or uses for which we may obtain patents, providers may recommend that patients use these products off-label, or patients may do so themselves. Although off-label use may infringe or contribute to the infringement of method-of-use patents, the practice is common and this type of infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or may in-license in the future may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. For example, while our patent applications are pending, we may be subject to a third party preissuance submission of prior art to the USPTO or become involved derivation proceedings, or equivalent proceedings in foreign jurisdictions.

Even if patents do successfully issue, third parties may challenge their inventorship, validity, enforceability or scope, including through opposition, revocation, reexamination, post-grant and *inter partes* review proceedings. An adverse determination in any such submission, proceeding or litigation may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. Moreover, some of our patents and patent applications may be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties,

including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. If the breadth or strength of protection provided by the patent applications we hold with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize our product candidates. Further, if we encounter delays in development, testing, and regulatory review of new product candidates, the period of time during which we could market our product candidates under patent protection would be reduced.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

Other parties have developed technologies that may be related or competitive to our, and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our own patent applications or issued patents, with respect to either the same methods or formulations or the same subject matter, in either case, that we may rely upon to dominate our patent position in the market. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first-to-file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to our own. Any such patent application may have priority over our patent applications or patents, which could require us to obtain rights to issued patents covering such technologies.

We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our programs in any jurisdiction. The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect. For example, we may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third-party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products. Moreover, we are also possible that prior art may exist that we are aware of but does not believe is relevant to our current or future patents, but that could nevertheless be determined to render our patents invalid.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time consuming and unsuccessful.

Competitors may infringe the patents for which we have applied. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the United States, counterclaims alleging invalidity and/or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, lack of written disclosure, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal allegations of invalidity and unenforceability during patent litigation is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our products or certain aspects of our platform technology. Further, a court or administrative body could construe certain patent claims narrowly or refuse to prevent the other party from using the technology at issue on the ground that our patents do not cover the technology.

In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and/or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patent claims do not cover the technology in question. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, inter partes review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in

revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business. Such a loss of patent protection could negatively impact our business. Patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without legally infringing our patents or other intellectual property rights.

An unfavorable outcome could require us to cease using the related technology or force us to take a license under the patent rights of the prevailing party, if available. Furthermore, our business could be harmed if the prevailing party does not offer a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Even if we establish infringement of any of our patents by a competitive product, a court may decide not to grant an injunction against further infringing activity, thus allowing the competitive product to continue to be marketed by the competitor. It is difficult to obtain an injunction in U.S. litigation and a court could decide that the competitor should instead pay us a "reasonable royalty" as determined by the court, and/or other monetary damages. A reasonable royalty or other monetary damages may or may not be an adequate remedy. Loss of exclusivity and/or competition from a related product would have a material adverse impact on our business.

If we in-licenses patent rights in the future, we may not have the right to file a lawsuit for infringement and may have to rely on a licensor to enforce these rights for us. If we are not able to directly assert our licensed patent rights against infringers or if a licensor does not vigorously prosecute any infringement claims on our behalf, we may have difficulty competing in certain markets where such potential infringers conduct their business, and our commercialization efforts may suffer as a result.

In addition, we or our future licensors, as the case may be, may not be able to detect infringement against our owned or in-licensed patents, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our future licensors detect infringement by a third party of owned or future in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we or our licensors later sue such third party for patent infringement, the third party may have certain legal defenses available to it that otherwise would not be available but for the delay between when the infringement was first detected and when the suit was brought. These legal defenses may make it impossible for us or our licensors to enforce owned or future in-licensed patents, as the case may be, against that third party.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Third-party claims of intellectual property infringement may prevent, delay or otherwise interfere with our product discovery and development efforts.

Our commercial success depends in part on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property or proprietary rights of third parties.

There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, *inter partes* review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights.

Numerous U.S. and foreign issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our

field, third parties may allege they have patent rights encompassing our product candidates, technologies or methods. We are aware of competitors in the oligonucleotide space whose patent application filings and/or issued patents may include claims directed to technologies and/or products related to some of our programs and product candidates. For example, we are aware of patents and patent applications owned by third parties that have generic claims that may relate to our technologies and products.

If a third party claims that we infringe, misappropriate or otherwise violate our intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims that, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages plus the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third party licenses its product rights to us, which it is not required to do, on commercially reasonable terms or at all;
- if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our product candidates;
- the requirement that we redesign our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time; and
- there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, financial condition, results of operations and prospects.

Third parties may assert that we are employing their proprietary technology without authorization, including by enforcing its patents against us by filing a patent infringement lawsuit against us. In this regard, patents issued in the United States by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof.

There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of its product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, or materials used in or formed during the manufacturing process, or any final product itself, the holders of those patents may be able to block our ability to commercialize our product candidate unless we obtain a license under the applicable patents, or until those patents were to expire or those patents are finally determined to be invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy or patient selection methods, the holders of that patent may be able to block our ability to develop and commercialize the product candidate unless we obtain a license or until such patent expires or is finally determined to be invalid or unenforceable. In either case, a license may not be available on commercially reasonable terms, or at all, particularly if such patent is owned or controlled by one of our primary competitors. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to it. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee time and resources from our business. In

the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any license of this nature would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates and we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could significantly harm our business.

Likewise, our patents and patent applications, if issued as patents, directed to our proprietary technologies and our product candidates are expected to expire from 2040 through 2044, without taking into account any possible patent term adjustments or extensions. Our earliest in-licensed patents may expire before, or soon after, our first product achieves marketing approval in the United States or foreign jurisdictions. Additionally, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of the pending patent applications we own or in-license currently or in the future. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, financial condition, results of operations and prospects.

We or our future licensors, collaborators or strategic partners may be subject to third-party claims for infringement or misappropriation of patent or other proprietary rights. We may be generally obligated under our future potential license or collaboration agreements to indemnify and hold harmless our licensors or collaborators for damages arising from intellectual property infringement by us. If we or our future licensors, collaborators or strategic partners are found to infringe a third-party patent or other intellectual property rights, we could be required to pay damages, potentially including treble damages, if we are found to have willfully infringed. In addition, we or our future licensors, collaborators or strategic partners may choose to seek, or be required to seek, a license from a third party, which may not be available on acceptable terms, if at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to it. If we fail to obtain a required license, we or our future collaborators may be unable to effectively market product candidates based on our technology, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. In addition, we may find it necessary to pursue claims or initiate lawsuits to protect or enforce our patent or other intellectual property rights. The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

Additionally, we may be required to protect our patents through procedures created to attack the validity of a patent at the USPTO. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

We may not be successful in acquiring or in-licensing necessary rights to key technologies or any product candidates we may develop.

The future growth of our business may depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates and technologies. There has been extensive patenting activity in the field of gene editing. Pharmaceutical companies, biotechnology companies and academic institutions are competing with us or are expected to compete with us in the in the field of gene editing technology and filing patent applications potentially relevant to our business. In order to market our product candidates, we may find it necessary or prudent to obtain licenses from third-party intellectual property holders. However, we may be unable to secure such licenses or otherwise acquire or in-license any compositions, methods of use, processes, or other intellectual property rights from third parties that we identify as necessary to develop or commercialize our product candidates or other key technologies. We may also require licenses from third parties for certain additional technologies, including technologies relating to RNA editing, such as guide RNA modification, or target sequences as well as delivery technologies for product candidates we may develop. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our product candidates or allow our competitors or other third parties the chance to access technology that is important to our business.

Additionally, we may collaborate with academic institutions to accelerate our research or development under written agreements with these institutions. In certain cases, these institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Even if we hold such an option, we may be unable to

negotiate a license from the institution within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, such institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program.

The licensing or acquisition of third-party intellectual property rights is a highly competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that it may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all.

It is possible that we may be unable to obtain required licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, we may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, programs, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected programs, which could harm our business, financial condition, results of operations, and prospects significantly.

Disputes may arise between us and our future licensors regarding intellectual property subject to a license agreement, including: the scope of rights granted under the license agreement and other interpretation- related issues; whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; our right to sublicense patents and other rights to third parties; our right to transfer or assign the license; the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our future licensors and us and our partners; and the priority of invention of patented technology.

If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our programs or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our future licensors may have relied on third-party consultants or collaborators or on funds from third parties, such as the U.S. government, such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Third parties may assert that our employees, consultants, or advisors have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals that are currently or were previously employed at universities, research institutions or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants, and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these individuals have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Also, we have in the past and may in the future be subject to claims that these individuals are violating non-compete agreements with their former employers. We may then have to pursue litigation to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our technical and management personnel from their normal responsibilities. In addition,

there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities, and we may not have sufficient financial or other resources to adequately conduct this type of litigation or proceedings. For example, some of our competitors may be able to sustain the costs of this type of litigation or proceedings more effectively than we can because of their substantially greater financial resources. In any case, uncertainties resulting from the initiation and continuation of intellectual property litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees, and various other government fees on patents and applications are due to be paid to the USPTO and foreign patent agencies outside of the United States over the lifetime of our owned or licensed patents and applications. The USPTO and foreign patent agencies require compliance with several procedural, documentary, fee payment, and other similar provisions during the patent application process. We are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. While an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations, however, in which non-compliance can result a partial or complete loss of patent rights in the relevant jurisdiction. Were a noncompliance event to occur, our competitors might be able to enter the market with similar or identical products or technology, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property and proprietary rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of foreign countries do not protect intellectual property rights to the same extent as federal and state laws of the United States. In addition, our intellectual property license agreements may not always include worldwide rights. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology and pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our patents and intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Moreover, the initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

Further, filing, prosecuting and defending patents on programs worldwide would be prohibitively expensive and our intellectual property rights in some foreign jurisdictions can be less extensive than those in the United States. As such, we may not have patents in all countries or all major markets and may not be able to obtain patents in all jurisdictions even if we apply for them. Our competitors may operate in countries where we do not have patent protection and can freely use our technologies and discoveries in such countries to the extent such technologies and discoveries are publicly known or disclosed in countries where we do have patent protection or pending patent applications.

Changes in patent law in the United States and in non-U.S. jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our RNA editing platform technology and product candidates.

As is the case with other biotech and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain.

Changes in either the patent laws or interpretation of the patent laws could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of our issued patents and pending patent applications. For example, in March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, the United States transitioned from a "first to invent" to a "first-to-file" patent system. Under a "first-to-file" system, assuming that other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on an invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after March 2013, but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application.

Because patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either file any patent application related to our technology or product candidates or invent any of the inventions claimed in our or our licensor's patents or patent applications. The America Invents Act also includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, allowing third party submission of prior art and establishing a post-grant review system including post-grant review, inter partes review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action.

In addition, recent U.S. Supreme Court and U.S. Court of Appeals for the Federal Circuit rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, these rulings have created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. We cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. Any adverse changes in the patent laws of other jurisdictions could also have a material adverse effect on our business, financial condition, results of operations and prospects.

Geopolitical actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of patent applications and the maintenance, enforcement or defense of issued patents. For example, the United States and foreign government actions related to Russia's invasion of Ukraine may limit or prevent filing, prosecution and maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees that have citizenship or nationality in, are registered in, or have predominately primary place of business or profit-making activities in the United States and other countries that Russia has deemed unfriendly without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

In addition, recently the European Unified Patent Court, or UPC, was created as a common patent court to hear patent infringement and revocation proceedings effective for member states of the EU. This could enable third parties to seek revocation

of a European patent in a single proceeding at the UPC rather than through multiple proceedings in each of the jurisdictions in which the European patent is validated. Although we do not currently own any European patents or applications, if we obtain such patents and applications in the future, any such revocation and loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and products. Moreover, the controlling laws and regulations of the UPC will develop over time, and may adversely affect our ability to enforce or defend the validity of any European patents we may obtain. We may decide to opt out from the UPC any future European patent applications that we may file and any patents we may obtain. If certain formalities and requirements are not met, however, such European patents and patent applications could be challenged for non-compliance and brought under the jurisdiction of the UPC. We cannot be certain that future European patents and patent applications will avoid falling under the jurisdiction of the UPC, if we decide to opt out of the UPC.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our technology and product candidates, we also rely on know-how and trade secret protection, as well as confidentiality agreements, non-disclosure agreements and invention assignment agreements with our employees, consultants and third-parties, to protect our confidential and proprietary information, especially where we does not believe patent protection is appropriate or obtainable.

It is our policy to require our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed by or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties, except in certain specified circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and that are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In the case of consultants and other third parties, the agreements provide that all inventions conceived in connection with the services provided are our exclusive property. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. Additionally, the assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regards as our intellectual property. Any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable.

In addition to contractual measures, we try to protect the confidential nature of our proprietary information through other appropriate precautions, such as physical and technological security measures. However, trade secrets and know-how can be difficult to protect. These measures may not, for example, in the case of misappropriation of a trade secret by an employee or third party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and any recourse we might take against this type of misconduct may not provide an adequate remedy to protect our interests fully. In addition, trade secrets may be independently developed by others in a manner that could prevent us from receiving legal recourse. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any of that information was independently developed by a competitor, our competitive position could be harmed.

In addition, some courts inside and outside the United States are sometimes less willing or unwilling to protect trade secrets. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. Even if we are successful, these types of lawsuits may consume our time and other resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. The terms of individual patents depends upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest non-provisional filing date in the applicable country. However, the actual protection afforded by a patent varies from country to country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent. Various extensions including PTE and PTA, may be available, but the life of a patent, and the protection it affords, is limited. For more information regarding PTA and PTE, see Item 1 "*Business—Intellectual Property*". Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics. Given the amount of time required for the development,

testing and regulatory review of new product candidates, patents protecting our product candidates might expire before or shortly after we or our partners commercialize those candidates. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain PTE and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited PTE under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments PTE term of up to five years as compensation for patent term lost during the FDA regulatory review process. A PTE cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent per product may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, even if we were to seek a PTE, it may not be granted because of, for example, the failure to exercise due diligence during the testing phase or regulatory review process, the failure to apply within applicable deadlines, the failure to apply prior to expiration of relevant patents, or any other failure to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain PTE or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- any product candidates we may develop will eventually become commercially available in generic or biosimilar product forms;
- others may be able to make gene therapy products that are similar to any product candidates we may develop or utilize similar base editing technology but that are not covered by the claims of the patents that we may own in the future;
- We, or our future license partners or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- We, or our future license partners or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- We, or our future license partners or collaborators, may fail to meet our obligations to the U.S. government regarding any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss or unenforceability of patent rights;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending patent applications or those that we may own in the future will not lead to issued patents;
- it is possible that there are prior public disclosures that could invalidate our patents, or parts of our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates or technology similar to ours;
- it is possible that our patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- issued patents that we hold rights to may be held invalid, unenforceable, or narrowed in scope, including as a result of legal challenges by our competitors;
- the claims of our issued patents or patent applications, if and when issued, may not cover our product candidates;

- the laws of foreign countries may not protect our proprietary rights or the proprietary rights of our future license partners or collaborators to the same extent as the laws of the United States;
- the inventors of our patents or patent applications may become involved with competitors, develop products or processes that design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- our competitors may conduct research and development activities in countries where we do not have patent rights or enforceable patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not develop additional proprietary technologies that are patentable;
- any product candidates we develops may be covered by third parties' patents or other exclusive rights;
- a third party may challenge, invalidate, circumvent or weaken our patents, and as a result, a court could hold that our patents are not valid, enforceable and infringed;
- the patents of others may harm our business; or
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our use of open source software could impose limitations on our ability to commercialize our product candidates.

Our use of open source software could impose limitations on our ability to commercialize our product candidates. Our technology utilizes open source software that contains modules licensed for use from third-party authors under open source licenses. In particular, some of the software that powers OPERA may be provided under license arrangements that allow use of the software for research or other non-commercial purposes. As a result, in the future, as we seek to use our platform in connection with commercially available products, we may be required to license that software under different license terms, which may not be possible on commercially reasonable terms, if at all. If we are unable to license software components on terms that permit our use for commercial purposes, we may be required to replace those software components, which could result in delays, additional cost and additional regulatory approvals.

Use and distribution of open source software may entail greater risks than use of third-party commercial software, as open source licensors generally do not provide warranties or other contractual protections regarding infringement claims or the quality of the software code. Some open source licenses contain requirements that we make available source code for modifications or derivative works we create based upon the type of open source software we use. If we combine our proprietary software with open source software in a certain manner, we could, under certain of the open source licenses, be required to release the source code of our proprietary software to the public. This could allow our competitors to create similar products with lower development effort and time, and ultimately could result in a loss of product sales for us. Although we monitor our use of open source software, the terms of many open source licenses have not been interpreted by U.S. courts, and there is a risk that those licenses could be construed in a manner that could impose unanticipated conditions or restrictions on our ability to commercialize our product candidates. We could be required to seek licenses from third parties in order to continue offering our product candidates, to re-engineer our product candidates or to discontinue the sale of our product candidates in the event re-engineering cannot be accomplished on a timely basis, any of which could materially and adversely affect our business, financial condition, results of operations and prospects.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims

brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and growth prospects.

Our business could be materially and adversely affected in the future by the effects of disease outbreaks, epidemics and pandemics.

Disease outbreaks, epidemics and pandemics in regions where we may have clinical trial sites or other business operations could adversely affect our business, including by causing significant disruptions in our operations and/or in the operations of CROs upon whom we may rely. Disease outbreaks, epidemics and pandemics have negative impacts on our ability to initiate new clinical trial sites, to enroll new patients and to maintain existing patients who are participating in our clinical trials, which may include increased clinical trial costs, longer timelines and delay in our ability to obtain regulatory approvals of product candidates, if at all.

General supply chain issues may be exacerbated during disease outbreaks, epidemics and pandemics and may also impact the ability of our clinical trial sites to obtain basic medical supplies used in our trials in a timely fashion, if at all. If any of our raw materials or components suppliers become subject to acts or orders of U.S. or foreign government entities to allocate or prioritize raw materials or components to the manufacture or distribution of vaccines or medical supplies needed to test or treat patients in a disease outbreak, epidemic or pandemic, this could delay our clinical trials, perhaps substantially, which could materially and adversely affect our business.

General Risk Factors

Our operations are vulnerable to interruption by disasters, terrorist activity, pandemics and other events beyond our control, which could harm our business.

Our facilities are located in Massachusetts. We have not undertaken a systematic analysis of the potential consequences to our business and financial results from a major flood, power loss, terrorist activity, pandemics or other disasters and do not have a recovery plan for such events. In addition, we do not carry sufficient insurance to compensate us for actual losses from interruption of our business that may occur, and any losses or damages incurred by us could harm our business. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

Litigation costs and the outcome of litigation could have a material adverse effect on our business.

From time to time we may be subject to litigation claims through the ordinary course of our business operations regarding, but not limited to, employment matters, security of employee personal information, contractual relations with third parties and intellectual property rights. Litigation to defend ourself against claims by third parties, or to enforce any rights that we may have against third parties, may continue to be necessary, which could result in substantial costs and diversion of our resources, causing a material adverse effect on our business, financial condition, results of operations or cash flows.

Risks Related to Our Operations Following the Merger

If any of the events described in "*Risks Related to Our Business*" occur, those events could cause potential benefits of the Merger not to be realized. To the extent any of the events in the risks described in that section occurs, the potential benefits of the Merger may not be realized and our results of operations and financial condition could be adversely affected in a material way. This could cause the market price of our common stock to decline.

The price of our common stock is volatile and fluctuates substantially, which could result in substantial losses for our stockholders.

Our stock price has been, and is likely to continue to be, volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- results of clinical trials and preclinical studies of our product candidates, or those of our competitors or our existing or future collaborators;
- failure to meet or exceed financial and development projections we may provide to the public;

- failure to meet or exceed the financial and development projections of the investment community;
- if we do not achieve the perceived benefits of the Merger as rapidly or to the extent anticipated by financial or industry analysts;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions or market conditions in the pharmaceutical and biotechnology sectors;
- sales of securities by us or our securityholders in the future;
- if we fail to raise an adequate amount of capital to fund our operations or continued development of our product candidates;
- trading volume of our common stock;
- announcements by competitors of new commercial products, clinical progress or lack thereof, significant contracts, commercial relationships or capital commitments;
- adverse publicity relating to precision medicine product candidates, including with respect to other products in such markets;
- the introduction of technological innovations or new therapies that compete with our products and services; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock. In addition, a recession, depression or other sustained adverse market event could materially and adversely affect our business and the value of our common stock. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against such companies. Furthermore, market volatility may lead to increased shareholder activism if we experience a market valuation that activists believe is not reflective of our intrinsic value. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results, financial condition and cash flows.

We may be unable to successfully integrate Frequency's and our businesses and realize the anticipated benefits of the Merger.

The Merger involved the combination of two companies that operated as independent companies. We are required to devote significant management attention and resources to integrating our business practices and operations. We may fail to realize some or all of the anticipated benefits of the Merger if the integration process takes longer than expected or is more costly than expected. Potential difficulties we may encounter in the integration process include the following:

- the inability to successfully combine our businesses in a manner that permits us to achieve the anticipated benefits from the Merger, which would result in the anticipated benefits of the Merger not being realized partly or wholly in the time frame currently anticipated or at all;
- creation of uniform standards, controls, procedures, policies and information systems; and

- potential unknown liabilities and unforeseen increased expenses, delays or regulatory conditions associated with the Merger.

In addition, prior to the Merger, we operated independently. It is possible that the integration process also could result in the diversion of our management's attention, the disruption or interruption of, or the loss of momentum in our ongoing businesses or inconsistencies in standards, controls, procedures and policies, any of which could adversely affect our ability to maintain our business relationships or the ability to achieve the anticipated benefits of the Merger, or could otherwise adversely affect our business and financial results.

We will incur additional costs and increased demands upon management as a result of complying with the laws and regulations affecting public companies.

We will incur significant legal, accounting and other expenses operating Legacy Korro's business as a public company that it did not incur as a private company, including costs associated with public company reporting obligations under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Our new post-Merger management team includes some individuals who have not previously managed and operated a public company. These executive officers and other personnel will need to devote substantial time to gaining expertise related to public company reporting requirements and compliance with applicable laws and regulations to ensure that we continue to comply with all of these requirements. Any changes we make to comply with these obligations may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with operating Legacy Korro's business as a public company, could also make it more difficult for us to attract and retain qualified persons to serve on the board of directors or on board committees or to serve as executive officers, or to obtain certain types of insurance, including directors' and officers' insurance, on acceptable terms.

Once we are no longer an emerging growth company, a smaller reporting company or otherwise no longer qualify for applicable exemptions, we will be subject to additional laws and regulations affecting public companies that will increase our costs and the demands on management and could harm our operating results and cash flows.

We are subject to the reporting requirements of the Exchange Act, which requires, among other things, that we file with the SEC, annual, quarterly and current reports with respect to our business and financial condition as well as other disclosure and corporate governance requirements. However, as an emerging growth company, we may take advantage of exemptions from various requirements such as an exemption from the requirement to have our independent auditors attest to our internal control over financial reporting under Section 404 of the Sarbanes-Oxley Act of 2002 as well as an exemption from the "say on pay" voting requirements pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010. We will no longer qualify as an emerging growth company after December 31, 2023. After we no longer qualify as an emerging growth company, we expect to still qualify as a "smaller reporting company," as such term is defined in Rule 12b-2 under the Exchange Act, in at least the near term, which will allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Once we are no longer an emerging growth company or a smaller reporting company or otherwise no longer qualify for these exemptions, we will be required to comply with these additional legal and regulatory requirements applicable to public companies and will incur significant legal, accounting and other expenses to do so. If we are not able to comply with the requirements in a timely manner or at all, our financial condition or the market price of our common stock may be harmed. For example, if we or our auditor identifies deficiencies in our internal control over financial reporting that are deemed to be material weaknesses we could face additional costs to remedy those deficiencies, the market price of our stock could decline or we could be subject to sanctions or investigations by the SEC or other regulatory authorities, which would require additional financial and management resources.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our annual report on Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This will require that we incur substantial professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. We may experience difficulty in meeting these reporting requirements in a timely manner.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our common stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

Our certificate of incorporation and bylaws and provisions under Delaware law could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our restated certificate of incorporation, as amended, and bylaws may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which our common stockholders might otherwise receive a premium price for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors will be responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66.67% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of Delaware, or the DGCL, which prohibits stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirors to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Our bylaws provide that, unless we consent in writing to the selection of an alternative forum, certain designated courts will be the sole and exclusive forum for certain legal actions between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of or based on a breach of a fiduciary duty owed by any of our current or former directors, officers, or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, our charter or our bylaws, or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein, which for purposes of this risk factor refers to herein as the "Delaware Forum Provision." The Delaware Forum Provision will not apply to any causes of action arising under the Securities Act and the Exchange Act. Our bylaws further provide that, unless we consent

in writing to an alternative forum, federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, which for purposes of this risk factor refers to herein as the "Federal Forum Provision." In addition, our bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock is deemed to have notice of and consented to the foregoing Delaware Forum Provision and Federal Forum Provision; provided, however, that stockholders cannot and will not be deemed to have waived its compliance with the U.S. federal securities laws and the rules and regulations thereunder.

The Delaware Forum Provision and the Federal Forum Provision may impose additional litigation costs on our stockholders in pursuing any such claims, particularly if our stockholders do not reside in or near the State of Delaware. Additionally, the forum selection clauses in our bylaws may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

The current expectation is that we will retain our future earnings, if any, to fund the growth of our business as opposed to paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future.

An active trading market for our common stock may not continue to develop or be sustained and our stockholders may not be able to resell their shares of common stock for a profit, if at all.

We cannot assure you that an active trading market for our shares of common stock may continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for our stockholders to sell their shares at an attractive price or at all.

Future sales of shares by existing stockholders could cause our stock price to decline.

If our existing securityholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after applicable legal and/or contractual restrictions on resale lapse, the trading price of our common stock could decline. Certain shares of our common stock are subject to a 180-day resale lock-up pursuant to certain lock-up agreement entered into prior to the closing of the Merger, and will be available for resale in the public market beginning 180 days after the closing of the Merger as a result of the expiration of such lock-up agreements. All other outstanding shares of common stock, other than shares held by our affiliates and shares issued in exchange for shares of Legacy Korro's common stock issued in the Pre-Closing Financing are freely tradable, without restriction, in the public market. In addition, shares of common stock that are subject to our outstanding options or warrants are eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements and Rules 144 and 701 under the Securities Act. The market price of the shares of our common stock could decline as a result of the sale of a substantial number of our shares of common stock in the public market or the perception in the market that the holders of a large number of shares intend to sell their shares.

Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.

Our executive officers, directors and principal stockholders, in the aggregate, beneficially own approximately 77% of our outstanding shares of common stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect to not provide research coverage of our common stock following the Merger, and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to

publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

We will have broad discretion in the use of our cash and cash equivalents and the proceeds from the Pre-Closing Financing and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.

We will have broad discretion over the use of our cash and cash equivalents, including the proceeds from the Pre-Closing Financing. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment. Our failure to apply these resources effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. You will not have the opportunity to influence our decisions on how to use our cash resources.

Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect our business and financial condition. In recent years, many such changes have been made and changes are likely to continue to occur in the future. For example, under Section 174 of the Code, in taxable years beginning after December 31, 2021, expenses that are incurred for research and development in the U.S. are capitalized and amortized, which may have an adverse effect on our cash flow. In addition, it is unclear how these U.S. federal income tax changes will affect state and local taxation. We cannot predict whether, when, in what form or with what effective dates, tax laws, regulations and rulings may be enacted, promulgated or decided or whether they could increase our tax liability or require changes in the manner in which we operate in order to minimize increases in our tax liability.

Our ability to use NOL carryforwards and other tax attributes may be limited, including as a result of the Merger.

Our ability to utilize NOL carryforwards and other tax attributes to offset future taxable income or tax liabilities may be limited as a result of ownership changes, including, as discussed below, in connection with the Merger or other transactions. Similar rules may apply under state tax laws. If we earn taxable income, such limitations could result in increased future income tax liability to us, and our future cash flows could be adversely affected.

For a more complete discussion of the risks related to the net operating loss carryforwards and certain of our other tax attributes, please see the discussion under "*Risks Related to Our Financial Position and Need for Capital—Our ability to utilize our net operating loss, or NOL, carryforwards and certain other tax attributes may be limited.*"

Unfavorable global economic conditions could adversely affect our business, financial condition, results of operations or cash flows.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity

Cybersecurity Risk Management and Strategy

We recognize the importance of assessing, identifying, and managing risks from cybersecurity threats. We have implemented a cybersecurity risk management process in accordance with our risk profile and business that is informed by industry standards and is integrated into our enterprise risk management process.

We leverage the support of third-party information technology and security providers, including for periodic security testing and risk assessments, as part of our risk management process, designed to identify, assess, and manage cybersecurity risks. We conduct employee cybersecurity training and maintain an incident response and notification plan designed to assist us in identifying, responding to, and recovering from cybersecurity incidents. Further, we intend to evaluate and update our existing cybersecurity policies and procedures as appropriate to continue to align them to our risk profile.

We have a process to assess the security practices of certain third-party vendors, including through the use of vendor security questionnaires, as appropriate.

Although risks from cybersecurity threats have to date not materially affected us, our business strategy, results of operations or financial condition, we have, from time to time, experienced threats to and breaches of our and our third party vendors' data and systems. For more information about these risks, see Item 1A "*Risk Factors—Risks Related to Our Business*."

Governance Related to Cybersecurity Risks

Our Vice President, Information Technology, or Vice President, who reports to the Chief Operating Officer, is responsible for the strategic leadership and direction of our cybersecurity program. With over 20 years of experience in information technology, the Vice President works alongside individuals across other functions, such as legal and engineering, to establish and implement our cybersecurity strategy.

The Vice President and our Chief Operating Officer and General Counsel participate in periodic discussions with other members of our management, including executive leadership, regarding implementation of our cybersecurity program, program enhancements, and relevant cybersecurity risks or threats.

Our audit committee has oversight over cybersecurity risks. With the input of the executive team, the Vice President provides annual presentations to the audit committee on our cybersecurity program, including updates on cybersecurity testing and assessments, cybersecurity risks, and related cybersecurity strategy as applicable. The management team will also update the full board of directors on matters related to cybersecurity as needed.

Additionally, we have implemented an enterprise risk management process, which addresses cybersecurity risks. This process is led by our General Counsel and includes participation by the board of directors, as appropriate. Our General Counsel reports regularly on the enterprise risk management process to executive leadership and the audit committee.

Item 2. Properties.

Our principal office is located at One Kendall Square, Building 600-700, Suite 6-401, Cambridge, MA 02139, where we lease approximately 22,500 square feet of office space. The lease term began in August 2020 and will end in September 2024. We also lease 18,148 square feet of laboratory and office space at 42 & 45 Cummings Park in Woburn, Massachusetts. We have plans to relocate our headquarters and occupy 50,453 square feet of laboratory and office space at 60 First Street in Cambridge, Massachusetts upon completion of the buildout of the space in 2024. We believe that these facilities will be adequate for our near-term needs. If required, we believe that suitable additional or substitute space will be available in the future on commercially reasonable terms to accommodate any such expansion of our operations.

Item 3. Legal Proceedings.

From time to time, we may be involved in various other claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings other than as disclosed below.

On June 3, 2021 and June 22, 2021, purported stockholders of Frequency filed putative class action lawsuits in the U.S. District Court for the District of Massachusetts against the Frequency and the Frequency's Chief Executive Officer, President, and Director, David Lucchino. On March 21, 2022, the two lawsuits were consolidated into a single lawsuit, Quinones et al. v. Frequency Therapeutics, Inc. et al. and on May 16, 2022, Frequency's Chief Development Officer, Dr. Carl Le Bel, was added as a defendant. The plaintiffs alleged violations of Sections 10(b), 20(a) and Rule 10b5 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, due to allegedly false and misleading statements and omissions about Frequency's Phase 2a clinical trial (FX-322-202) for its product candidate FX-322 in Frequency's public disclosures between October 29, 2020 and March 22, 2021. The lawsuit sought, among other things, damages in connection with Frequency's allegedly artificially inflated stock price between October 29, 2020 and March 22, 2021 as a result of those allegedly false and misleading statements and omissions, as well as interest, attorneys' fees and costs. On March 29, 2023, Frequency's motion to dismiss was granted and the lawsuit was dismissed in its entirety. On April 27, 2023, Plaintiff filed a notice of appeal to the United States Court of Appeals for the First Circuit from the order dismissing the lawsuit. On August 2, 2023, Plaintiff-Appellant submitted its opening brief to the

First Circuit. Frequency filed its response brief on October 27, 2023. The First Circuit heard oral argument on January 8, 2024, and has not yet issued a decision on plaintiff's appeal. The Company intends to vigorously defend against the lawsuit.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock is listed on the Nasdaq Capital Market under the symbol "KRRO".

Holders of Our Common Stock

As of March 1, 2024, we had 8,020,788 shares of common stock issued and outstanding held of record by 96 holders. The actual number of holders of these securities is greater than this number of record holders, as the actual number includes holders who are beneficial owners whose securities are held in street name by brokers and other nominees. This number of holders of record also does not include holders whose securities may be held in trust by other entities.

Recent Sales of Unregistered Securities

None.

Equity Compensation Plan Information

Information about our equity compensation plans is incorporated herein by reference to Item 12 "*Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters"* of this Annual Report on Form 10-K.

Purchases of Equity Securities by the Issuer or Affiliated Purchasers

None.

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our audited consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth under the heading "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. See also "Cautionary Statement Regarding Forward-Looking Statements."

Overview

We are a biopharmaceutical company with a mission to discover, develop and commercialize a new class of genetic medicines based on editing RNA, enabling the treatment of both rare and highly prevalent diseases.

We are generating a portfolio of differentiated programs that are designed to harness the body's natural RNA editing process to effect a precise yet transient single base edit. By editing RNA instead of DNA, we are expanding the reach of genetic medicines by delivering additional precision and tunability, which has the potential for increased specificity and improved long-term tolerability. Using an oligonucleotide-based approach, we expect to bring our medicines to patients by leveraging our proprietary platform with precedented delivery modalities, manufacturing know-how, and established regulatory pathways of approved oligonucleotide drugs. However, the scientific evidence to support the feasibility of developing product candidates using our RNA editing technology is both preliminary and limited. Moreover, regulators have not yet established any definitive guidelines related to overall development considerations for RNA editing therapies and no clinical data has been generated to date.

The versatility of RNA editing combined with our OPERA platform broadens the therapeutic target space significantly. While our approach can be used to repair pathogenic single nucleotide variants, or SNVs, as demonstrated by our most advanced program, our Alpha-1 Antitrypsin Deficiency, or AATD, product candidate, we can also engineer de novo SNVs and change amino acids on proteins to endow them with desired properties while preserving their broader functional capabilities, as exemplified by three of our other programs (severe Alcoholic Hepatitis, or SAH, amyotrophic lateral sclerosis, or ALS, Pain). In preclinical studies, we have demonstrated that single RNA changes can disrupt protein-protein interactions, prevent protein aggregation, selectively modulate ion channels and activate kinases. These modification approaches can unlock validated target classes that have historically been difficult to drug, enabling us to pursue a broad range of diseases traditionally out-of-scope for other genetic medicine approaches and current traditional drug modalities.

Our most advanced program is a development candidate, KRRO-110, for the treatment of AATD where, using our proprietary RNA editing approach, we are repairing a pathogenic variant on RNA. KRRO-110 has the potential to be disease-modifying and provide a differentiated therapeutic option. Preclinical development of KRRO-110 is ongoing in preparation for a potential regulatory filing in the second half of 2024 and an anticipated interim clinical readout in the second half of 2025.

Since inception, we have focused primarily on organizing and staffing our company, business planning, raising capital, securing related intellectual property, and conducting research and development activities for our potential programs and product candidates. Since inception, we have funded our operations primarily through the private placement of our equity securities. To date, we have raised approximately $223.6 million of aggregate gross proceeds from the sale of our convertible preferred stock, and $117.3 million from the sale of shares of common stock issued in a private placement that closed immediately prior to the Merger (as defined below), or the Pre-Closing Financing.

We have incurred significant operating losses since inception. Our net losses were $81.2 million and $58.0 million for the years ended December 31, 2023 and 2022, respectively. We had an accumulated deficit of $183.0 million as of December 31, 2023. We expect to continue to incur significant and increasing expenses and operating losses and negative operating cash flows for the foreseeable future as we continue our research and development efforts, advances product candidates through clinical stages, and seeks regulatory approvals for its pipeline candidates. As a result of the Merger (as defined below), we also expect to incur additional costs associated with maintaining compliance with Nasdaq listing rules and the requirements of the U.S. Securities and Exchange Commission, or SEC, director and officer liability insurance, investor and public relations activities and other expenses associated with operating as a public company. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of its preclinical studies, initiation and conduct of any clinical trials, and its expenditures on other research and development activities, including the expansion of its pipeline.

We do not have any product candidates approved for sale and have not generated any revenue from product sales. We will not generate revenue from product sales unless and until we successfully obtain regulatory approval for our product candidates, if ever, and as appropriate, move pipeline candidates into the clinic and complete clinical development. We have yet to commence clinical trials on any of our program candidates. If we obtain regulatory approval for our product candidates and do not enter into third-party commercialization partnerships, we expect to incur significant expenses related to developing commercialization capabilities to support product sales, marketing, manufacturing and distribution activities. As a result, we will need substantial additional funding to support our continuing operations and pursue our development and growth strategy. Until we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private offerings of securities, debt financings or other sources, such as potential collaboration agreements, strategic alliances and licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on acceptable terms, or at all. Our failure to raise capital or enter into such agreements as, and when needed, could have a negative effect on its business, results of operations and financial condition.

Recent Developments

Merger Agreement & Pre-Closing Financing

On July 14, 2023, we entered into the Merger Agreement with the private company formerly known as Korro Bio, Inc., or Legacy Korro, and Frequency Merger Sub, Inc., our wholly-owned subsidiary, or Merger Sub. Pursuant to the Merger Agreement, on November 3, 2023, or the Closing Date, following approval by our stockholders and Legacy Korro's stockholders, Merger Sub merged with and into Legacy Korro, or the Merger, with Legacy Korro continuing as our wholly owned subsidiary and the surviving corporation of the Merger. The Merger is intended to qualify as a "reorganization" within the meaning of Section 368(a) of the Internal Revenue Code of 1986, as amended. The Merger Agreement was approved by the members of the board of directors of both our company and Legacy Korro. In connection with the completion of the Merger, we changed our name from Frequency Therapeutics, Inc. to Korro Bio, Inc. The merger was accounted for as a reverse recapitalization and accordingly, the financial statements of Legacy Korro became our financial statements. Except as otherwise indicated, references herein to "we", "us" or the "Company", refer to Korro Bio, Inc. on a post-merger basis; references to "Legacy Korro" refer to the business of privately held Korro Bio, Inc., prior to completion of the merger; and references to Frequency refer to Frequency Therapeutics, Inc. prior to completion of the merger.

Immediately prior to the execution of the Merger Agreement, certain new and existing investors of Legacy Korro agreed to purchase shares of Legacy Korro's common stock at $2.78 per share for the aggregate amount of $117.3 million in the Pre-Closing Financing. The Pre-Closing Financing was contingent on and closed immediately prior to consummation of the Merger.

Subject to the terms and conditions of the Merger Agreement, immediately prior to the Closing Date, each then outstanding share of Legacy Korro's common stock (including common stock issued upon the conversion of Legacy Korro's preferred stock but excluding the common stock issued in the Pre-Closing Financing) converted into the right to receive 5,161,114 shares of our common stock calculated in accordance with the Merger Agreement (which takes into account the 1-for-50 reverse stock split of our common stock effected immediately prior to the Merger).

Shares of Legacy Korro's common stock issued pursuant to the Pre-Closing Financing were converted into the right to receive 2,077,864 shares of our common stock calculated in accordance with Merger Agreement at the effective time of the Merger (which takes into account the 1-for-50 reverse stock split of our common stock effected immediately prior to the Merger).

Financial Operations Overview

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery of novel genetic medicines and the development of our product candidates, salaries and benefits, and third-party license fees. We expense research and development costs as incurred, which include:

- employee-related expenses, including salaries, bonuses, benefits, stock-based compensation and other related costs for those employees involved in research and development efforts;
- external research and development expenses incurred under agreements with contract research organizations, or CROs as well as with consultants;
- laboratory supplies and research materials;

- payments made under third-party licensing agreements; and
- direct and allocated expenses for facilities.

Costs for certain activities are recognized based on an evaluation of the progress to completion of specific tasks using data such as information provided to us by our vendors and analyzing the progress of our preclinical studies or other services performed. Significant judgment and estimates are made in determining the accrued expense balances at the end of any reporting period.

Our external research and development expenses consist primarily of fees paid to CROs and outside consultants in connection with our preclinical development activities. Our external research and development expenses also include fees incurred under license agreements. As a pre-clinical company, we do not yet track these external research and development costs on a program-by-program basis. We plan to track program costs on our lead development candidate KRRO-110 upon filing of an Investigational New Drug, or IND, application.

We characterize research and development costs incurred prior to the identification of a product candidate as discovery costs. We use internal resources primarily to conduct our research and discovery activities as well as for managing our preclinical development activities.

The successful development of our product candidates is highly uncertain. We plan to substantially increase our research and development expenses for the foreseeable future as we continue the development of our product candidates, conducts discovery and research activities for our preclinical programs, and expands our pipeline. We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future preclinical studies and clinical trials, regulatory developments and our ongoing assessments as to each product candidate's commercial potential. Our clinical development costs are expected to increase significantly as we commence clinical trials. We anticipate that our expenses will increase substantially, particularly due to the numerous risks and uncertainties associated with developing product candidates, including the uncertainty of:

- the scope, rate of progress, and expenses of our ongoing research activities as well as any preclinical studies, clinical trials and other research and development activities;
- establishing an appropriate safety profile with IND enabling studies;
- successful enrollment in and completion of clinical trials;
- whether our product candidates show safety and efficacy in our clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- commercializing product candidates, if and when approved, whether alone or in collaboration with others; and
- continued acceptable safety profile of products following any regulatory approval.

Any changes in the outcome of any of these variables with respect to the development of our product candidates in preclinical and clinical development could mean a significant change in the costs and timing associated with the development of these product candidates. We may never succeed in achieving regulatory approval for any of our product candidates. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some product candidates or focus on other product candidates. For example, if the U.S. Food and Drug Administration, or FDA, European Medicines Agency, or EMA, or another regulatory authority were to delay the planned start of clinical trials or require us to conduct clinical trials or other testing beyond those that we currently expect or if we experience significant delays in enrollment in any planned clinical trial, we could be required to expend significant additional financial resources and time on the completion of clinical development of that product candidate.

General and Administrative Expenses

General and administrative expenses consist primarily of employee related costs, including salaries, bonuses, benefits, stock-based compensation and other related costs for our executive and administrative functions. General and administrative expenses also include professional services, including legal, accounting, auditing, tax services and other consulting fees. General and administrative expenses also include facility costs not otherwise included in research and development expenses.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our product candidates. We also anticipate that we will incur significantly increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses associated with operating as a public company.

Other Income, Net

Other income, net primarily consists of interest income earned on money market fund accounts.

Results of Operations

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022:

	Year Ended December 31,		Change
(in thousands)	2023	2022	
Operating expenses:			
Research and development	$ 57,250	$ 42,201	$ 15,049
General and administrative	27,284	16,797	10,487
Total operating expenses	84,534	58,998	25,536
Loss from operations	(84,534)	(58,998)	(25,536)
Other income, net			
Other income, net	3,389	976	2,413
Total other income, net	3,389	976	2,413
Loss before provision for income taxes	(81,145)	(58,022)	(23,123)
Provision for income taxes	27	10	17
Net loss	$ (81,172)	$ (58,032)	$ (23,140)

Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2023 and 2022:

	Year Ended December 31,		Change
(in thousands)	2023	2022	
External research and development	$ 18,683	$ 13,868	$ 4,815
Personnel-related expenses	15,298	9,960	$ 5,338
Lab supplies & consumables	7,447	9,123	$ (1,676)
Facilities costs	9,252	4,588	$ 4,664
Consulting	2,646	2,931	$ (285)
Sponsored research and license fees	2,104	376	$ 1,728
Other	1,820	1,355	465
Total research and development expenses	$ 57,250	$ 42,201	$ 15,049

Research and development expenses increased by $15.0 million from $42.2 million for the year ended December 31, 2022 to $57.2 million for the year ended December 31, 2023. The increase in research and development expenses was primarily attributable to the following:

- $4.8 million of increased external research and development expenses primarily attributable to increased oligonucleotide synthesis costs, screening and sequencing expenses and *in vivo* studies;

- $5.3 million of increased personnel-related expenses driven by an increase in headcount to support the expansion of our research and development function;
- $1.7 million of decreased lab supplies and consumables, primarily attributable to reduction consumables purchased for screening and sequencing;
- $4.7 million of increased facilities-related costs primarily due to the expansion of our mixed-use office spaces and depreciation of lab equipment;
- $0.3 million decrease in consulting costs attributed to the increase in internal resources which led to a reduction in our reliance on external consulting services.;
- $1.7 million of increased sponsored research and license fees primarily attributable to an upfront cash payment made to Genevant in March 2023 upon execution of a collaboration and license agreement; and
- $0.5 million of increased other expense primarily attributable to increased research and development software licensing fees and lab maintenance services.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the years ended December 31, 2023 and 2022:

	Year Ended December 31,		Change
(in thousands)	2023	2022	
Personnel-related expenses	$ 13,443	$ 8,970	$ 4,473
Professional services	8,507	4,529	3,978
Facilities expenses	3,253	1,600	1,653
Other	2,081	1,698	383
Total general and administrative expenses	$ 27,284	$ 16,797	$ 10,487

General and administrative expenses increased by $10.5 million from to $16.8 million for the year ended December 31, 2022 to $27.3 million for the year ended December 31, 2023. The increase in general and administrative expenses was primarily attributable to the following:

- $4.5 million of increased personnel-related expenses is primary driven by $2.5 million of severance cost to Frequency employees related to the Merger. The remaining increase of $2.0 million is driven by an increase in headcount to support growth in research and development and increased public company regulation;
- $4.0 million of increased professional service fees primarily attributable to recruiting efforts and increased intellectual property legal fees;
- $1.7 million of increased facilities expenses primarily attributable to the expansion of our mixed-use office space; and
- $0.4 million of increased other general and administrative expense primarily attributable to increased software licensing fees and corporate expenses.

Other Income, Net

Total other income, net increased by $2.4 million from $1.0 million for the year ended December 31, 2022 to $3.4 million for the year ended December 31, 2023. The increase in other income, net was primarily attributable to an increase of $2.4 million in interest income earned on money market fund accounts driven by rising interest rates and higher cash and cash equivalent balance.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have generated recurring net losses. We have not yet commercialized any product and we do not expect to generate revenue from sales of any products for several years, if at all. Since inception, we have funded our operations primarily through proceeds from the issuance of convertible preferred stock and common stock. To date, we have raised approximately $223.6 million of aggregate gross proceeds from the sale of convertible preferred stock and $117.3 million from the Pre-Closing Financing. As of December 31, 2023, we had cash and cash equivalents of $166.1 million.

Since inception, we have incurred significant operating losses and, as of December 31, 2023, had an accumulated deficit of $183.0 million. We expect to continue to incur significant expenses, operating losses, and negative operating cash flows for the foreseeable future. In addition, we have not yet commercialized any product and we do not expect to generate revenue from sales of any products for several years, if at all.

As of December 31, 2023, we had cash and cash equivalents of $166.1 million. We expect that our cash and cash equivalents outstanding as of December 31, 2023, will be sufficient to fund our operating expenses and capital expenditure requirements into 2026. We have based this estimate on assumptions that may prove to be wrong, and we could expend our capital resources sooner than we expect. We may also pursue additional cash resources through public or private equity, collaborations or debt financings. There is no assurance that we will be successful in obtaining sufficient financing on acceptable terms to continue funding our operations.

Funding Requirements

We expect to continue to incur significant expenses, operating losses, and negative operating cash flows for the foreseeable future as we continue our novel genetic medicine discovery efforts, advance our pipeline candidates into the clinic and through clinical trials, seek regulatory approval of our product candidates and pursue commercialization of any approved product candidates. In addition, we expect to continue to incur costs associated with operating as a public company.

Because of the numerous risks and uncertainties associated with research, development and commercialization of our product candidates, we are unable to estimate the exact amount of our working capital requirements.

Our future capital requirements will depend on many factors, including:

- the cost of continuing to build our OPERA platform and discover additional novel genetic medicines;
- the scope, progress, results, and costs of discovery, preclinical development, laboratory testing, manufacturing and clinical trials for the product candidates we may develop;
- the extent to which we partner our programs, acquires or in-licenses other product candidates and technologies or enters into additional collaborations;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, EMA and other regulatory authorities;
- the timing and amount of milestone and royalty payments that we are required to make or eligible to receive under any future collaboration and license agreements;
- Our headcount growth and associated costs as we expand our research and development efforts;
- the cost of expanding, maintaining and enforcing our intellectual property portfolio, including filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us or any of our product candidates;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidates for which we receive marketing approval;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the effect of competing technological and market developments; and
- the costs of operating as a public company.

Until such time, if ever, as we can generate substantial product revenues to support our cost structure, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations and other similar arrangements. To the extent that we raise additional capital through the sale of equity or convertible securities, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through collaborations, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common shares. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product research and development or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

Cash Flows

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our cash flows for the years ended December 31, 2023 and 2022:

	Year Ended December 31,	
(in thousands)	**2023**	**2022**
Net cash used in operating activities	$ (67,283)	$ (53,645)
Net cash provided by investing activities	11,164	11,060
Net cash provided by financing activities	187,761	18
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 131,642	$ (42,567)

Cash Used in Operating Activities

Net cash used in operating activities for the year ended December 31, 2023 was $67.3 million, compared to net cash used in operating activities of $53.6 million for the year ended December 31, 2022. The increase of $13.7 million in cash used in operating activities was primarily due to the overall increase in our operating expenses during that same period, including an increase in payments made for external research and development activities.

Cash Provided by Investing Activities

Net cash provided by investing activities for the year ended December 31, 2023 was $11.2 million, compared to net cash provided by investing activities of $11.0 million for the year ended December 31, 2022. Net cash provided by investing activities primarily consists of proceeds from maturities of investments partially offset by cash used to purchase property and equipment.

Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2023 was $187.8 million, compared to less than $0.1 million for the year ended December 31, 2022. The increase of $187.8 million in cash provided by financing activities is primarily due to $117.3 million of gross proceeds from the Pre-Closing Financing, $45.5 million of net cash proceeds received from the sale of Legacy Korro's Series B-2 convertible preferred stock and $24.4 million net cash acquired from completion of the Merger, which was accounted for as a reverse recapitalization. No comparable proceeds were received from financing activities during the year ended December 31, 2022.

Critical Accounting Policies and Estimates

Our consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimate in light of changes in circumstances, facts, and experience. The effects of material

revisions in estimates, if any, will be reflected in the consolidated financial statements prospectively from the date of change in estimates.

While our significant accounting policies are described in more detail in Note 2 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued and Prepaid Research and Development Expenses

As part of the process of preparing the consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with internal personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers require advance payments; however, some providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and make adjustments if necessary.

We recognize expenses related to preclinical studies and other studies on our estimates of the services performed pursuant to contracts with research institutions and clinical research organizations that conduct and manage preclinical studies and other studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of accrued research and development expenses.

Contractual Obligations and Other Commitments

Our contractual obligations and commitments relate primarily to our operating leases and non-cancelable purchase obligations under agreements with various research and development organizations and suppliers in the ordinary course of business. See Note 14, "Commitments and Contingencies" to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

Recent Accounting Pronouncements

A description of recently issued and recently adopted accounting pronouncements applicable to our financial position and results of operations is included in Note 2 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

Emerging Growth Company and Smaller Reporting Company Status

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. We may take advantage of these exemptions until we are no longer an emerging growth company under Section 107 of the JOBS Act, which provides that an emerging growth company can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. We have elected to avail ourselves of the extended transition period and, therefore, while we are an emerging growth company, we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not emerging growth companies, unless we choose to early adopt a new or revised accounting standard. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the closing of our initial public offering, (b) in which we have total annual gross revenue of at least $1.235 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by

non-affiliates exceeds $700 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Additionally, we are a "smaller reporting company" as defined in Item 10(f)(1) of Regulation S-K. Smaller reporting companies may take advantage of certain reduced disclosure obligations, including, among other things, providing only two years of audited financial statements. We will remain a smaller reporting company until the last day of the fiscal year in which (i) the market value of our common stock held by non-affiliates exceeds $250 million as of the prior June 30, or (ii) our annual revenues exceed $100 million during such completed fiscal year and the market value of our common stock held by non-affiliates exceeds $700 million as of the prior June 30.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

As of December 31, 2023, we had cash and cash equivalents of $166.1 million, which consist of bank deposits and money market funds. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, an immediate 10% change in market interest rates would not have a material effect on the fair market value of our cash or cash equivalents.

Our employees and operations are primarily located in the United States. We have, from time to time, engaged in contracts with contractors or other vendors in a currency other than the U.S. dollar. To date, we have had minimal exposure to fluctuations in foreign currency exchange rates as the time period between the date that transactions are initiated, and the date of payment or receipt of payment is generally of short duration. Accordingly, we believe it does not have a material exposure to foreign currency risk.

Item 8. Financial Statements and Supplementary Data

Korro Bio, Inc

INDEX TO FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Korro Bio, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Korro Bio, Inc. (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the two years in the period ended December 31, 2023, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2023, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2020.
Boston, Massachusetts
March 26, 2024

Korro Bio, Inc.
Consolidated Balance Sheets
(amounts in thousands, except share and par value amounts)

	December 31, 2023	December 31, 2022
Assets:		
Current assets:		
Cash and cash equivalents	$ 166,150	$ 36,333
Short-term investments	—	18,915
Restricted cash	3,563	603
Prepaid expenses and other current assets	3,015	1,232
Total current assets	172,728	57,083
Property and equipment, net	15,665	9,866
Operating lease right-of-use assets	27,150	2,024
Restricted Cash, net of current portion	3,406	4,541
Other non-current assets	2,714	228
Total assets	$ 221,663	$ 73,742
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 7,280	$ 2,605
Accrued expenses and other current liabilities	10,212	3,175
Operating lease liabilities, current portion	1,991	2,921
Total current liabilities	19,483	8,701
Operating lease liabilities, net of current portion	31,216	209
Other non-current liabilities	1,053	—
Total liabilities	51,752	8,910
Commitments and contingencies (Note 13)		
Series Seed convertible preferred stock, $0.001 par value; no shares and 684,739 shares authorized, issued and outstanding at December 31, 2023 and 2022 (aggregate liquidation preference of $0 and $16,115 at December 31, 2023 and 2022)	—	15,924
Series A convertible preferred stock, $0.001 par value; no shares and 2,029,666 shares authorized, issued and outstanding at December 31, 2023 and 2022 (aggregate liquidation preference of $0 and $91,500 at December 31, 2023 and 2022)	—	77,736
Series B-1 convertible preferred stock, $0.001 par value; no shares and 1,104,178 shares authorized, issued and outstanding at December 31, 2023 and 2022 (aggregate liquidation preference of $0 and $58,000 at December 31, 2023 and 2022)	—	57,703
Series B-2 convertible preferred stock, $0.001 par value; no shares and 1,036,656 shares authorized at December 31, 2023 and 2022; no shares and 223,417 shares issued and outstanding at December 31, 2023 and 2022 (aggregate liquidation preference of $0 and $12,500 at December 31, 2023 and 2022)	—	12,500
Stockholders' equity (deficit)		
Preferred stock, $0.001 par value; 10,000,000 shares and no shares authorized at December 31, 2023 and 2022; no shares issued and outstanding at December 31, 2023 and 2022	—	—
Common stock, $0.001 par value; 200,000,000 and 5,755,759 shares authorized at December 31, 2023 and 2022; 8,016,516 and 271,371 shares issued at December 31, 2023 and 2022, respectively; 8,016,516 and 268,468 shares outstanding at December 31, 2023 and 2022	8	—
Additional paid-in capital	352,908	2,807
Accumulated other comprehensive loss	—	(5)
Accumulated deficit	(183,005)	(101,833)
Total stockholders' equity (deficit)	169,911	(99,031)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 221,663	$ 73,742

The accompanying notes are an integral part of these consolidated financial statements.

Korro Bio, Inc.
Consolidated Statements of Operations and Comprehensive Loss
(amounts in thousands, except share and per share amounts)

	Years Ended December 31,	
	2023	2022
Operating expenses:		
Research and development	$ 57,250	$ 42,201
General and administrative	27,284	16,797
Total operating expenses	84,534	58,998
Loss from operations	(84,534)	(58,998)
Other income:		
Other income, net	3,389	976
Total other income, net	3,389	976
Loss before provision for income taxes	(81,145)	(58,022)
Provision for income taxes	27	10
Net loss	$ (81,172)	$ (58,032)
Other comprehensive income		
Unrealized gain on available-for-sale investments	—	2
Comprehensive loss	$ (81,172)	$ (58,030)
Net loss per share, basic and diluted	$ (53.08)	$ (227.42)
Weighted-average shares used in computing net loss per share, basic and diluted	1,529,321	255,175

The accompanying notes are an integral part of these consolidated financial statements.

Korro Bio, Inc.
Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(amounts in thousands, except share amounts)

	Series Seed Convertible Preferred Stock		Series A Convertible Preferred Stock		Series B-1 Convertible Preferred Stock		Series B-2 Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2021	684,739	$ 15,924	2,029,666	$ 77,736	1,104,178	$ 57,703	223,417	$ 12,500	237,803	—	$ 1,600	$ (7)	$ (43,801)	$ (42,208)
Issuance of common stock for services rendered	—	—	—	—	—	—	—	—	497	—	11	—	—	11
Exercises of stock options	—	—	—	—	—	—	—	—	8,722	—	62	—	—	62
Vesting of restricted common stock	—	—	—	—	—	—	—	—	21,377	—	5	—	—	5
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	1,129	—	—	1,129
Other comprehensive income	—	—	—	—	—	—	—	—	—	—	—	2	—	2
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(58,032)	(58,032)
Balance at December 31, 2022	684,739	$ 15,924	2,029,666	$ 77,736	1,104,178	$ 57,703	223,417	$ 12,500	268,399	—	$ 2,807	$ (5)	$ (101,833)	$ (99,031)
Issuance of Series B-2 convertible preferred stock, net of issuance costs	—	—	—	—	—	—	813,239	45,458	—	—	—	—	—	—
Issuance of common stock for services rendered	—	—	—	—	—	—	—	—	292	—	6	—	—	6
Exercises of stock options	—	—	—	—	—	—	—	—	73,394	—	702	—	—	702
Vesting of restricted common stock	—	—	—	—	—	—	—	—	2,902	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	2,375	—	—	2,375
Issuance of common stock for cash in PIPE financing	—	—	—	—	—	—	—	—	2,077,864	2	117,250	—	—	117,252
Conversion of preferred stock to common stock	(684,739)	(15,924)	(2,029,666)	(77,736)	(1,104,178)	(57,703)	(1,036,656)	(57,958)	4,855,208	5	209,315	—	—	209,320
Issuance of common stock to Frequency shareholders in reverse recapitalization									738,457	1	32,700	—	—	32,701
Reverse recapitalization transaction costs	—	—	—	—	—	—	—	—	—	—	(12,247)	—	—	(12,247)
Other comprehensive income	—	—	—	—	—	—	—	—	—	—	—	5	—	5
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(81,172)	(81,172)
Balance at December 31, 2023	—	—	—	—	—	—	—	—	8,016,516	$ 8	$ 352,908	—	$ (183,005)	$ 169,911

The accompanying notes are an integral part of these consolidated financial statements.

Korro Bio, Inc.
Consolidated Statements of Cash Flows
(amounts in thousands)

	Years Ended December 31,	
	2023	2022
Operating Activities:		
Net loss	$ (81,172)	$ (58,032)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash lease expense	3,610	1,396
Stock-based compensation expense	2,381	1,140
Depreciation expense	3,630	2,511
Non-cash interest expense	—	118
Net amortization of premiums and discounts on investments	(80)	(145)
Loss on disposal of property and equipment	65	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	559	(28)
Accounts payable	3,100	1,755
Accrued expenses	1,270	(39)
Operating lease liabilities	1,341	(2,198)
Other non-current assets	(1,987)	(123)
Net cash used in operating activities	(67,283)	(53,645)
Investing Activities:		
Purchases of investments	—	(37,213)
Proceeds from maturities of investments	19,000	53,485
Purchases of property and equipment	(7,836)	(5,136)
Advance payments for property and equipment not yet received	—	(76)
Net cash provided by investing activities	11,164	11,060
Financing Activities:		
Proceeds from Series B-2 convertible preferred stock, net of issuance costs	45,458	—
Proceeds from exercises of stock options	702	62
Proceeds from the issuance of common stock in pre-closing financing	117,250	—
Cash acquired in connection with the reverse recapitalization	36,600	—
Payment of reverse recapitalization transaction costs	(12,247)	—
Other financing activities, net	(2)	(44)
Net cash provided by financing activities	187,761	18
Net increase (decrease) in cash, cash equivalents and restricted cash	131,642	(42,567)
Cash, cash equivalents and restricted cash, beginning of period	41,477	84,044
Cash, cash equivalents and restricted cash, end of period	$ 173,119	$ 41,477
Non-cash investing and financing activities:		
Property and equipment capitalized under tenant improvement allowance	$ 2,271	$ —
Purchases of property and equipment in accounts payable and accrued expenses	$ 1,983	$ 402
Operating lease liabilities arising from right-of-use assets	$ 26,777	$ 5,629
Operating lease liabilities arising from lease modification	$ 1,959	$ —
Supplemental cash flow information:		
Cash paid for income taxes	$ 27	$ 11
Cash paid for operating lease liabilities	$ 2,978	$ 2,335

The accompanying notes are an integral part of these consolidated financial statements.

1. The Company and Liquidity

Nature of Business

Korro Bio, Inc. (together with its subsidiaries, the "Company") is a biopharmaceutical company with a mission to discover, develop and commercialize a new class of genetic medicines based on editing RNA, enabling the treatment of both rare and highly prevalent diseases. The Company was incorporated in November 2014 as a Delaware corporation. The Company's principal offices are in Cambridge, Massachusetts.

Risks and Uncertainties

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive pre-clinical and clinical testing and regulatory approval, prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Reverse Merger with Legacy Korro

On July 14, 2023, Frequency Merger Sub, Inc.("Merger Sub") a Delaware corporation, a wholly-owned subsidiary of Frequency Therapeutics, Inc. ("Frequency"), a Delaware corporation and Korro Bio, Inc ("Legacy Korro"), a Delaware corporation, entered into an Agreement and Plan of Merger (the "Merger Agreement"). The merger was completed on November 3, 2023 (the "Merger" or the "Transaction"). In accordance with the Merger Agreement, the Merger Sub merged with and into Legacy Korro, with Legacy Korro surviving as a wholly-owned subsidiary of the Company. In connection with the completion of the Merger, the Company changed its name from Frequency Therapeutics, Inc. to Korro Bio, Inc. On November 6, 2023, the combined company's common stock began trading on The Nasdaq Capital Market under the ticker symbol "KRRO".

Except as otherwise indicated, references herein to the "Company," or the "combined company", refer to Korro Bio, Inc. on a post-merger basis, and the term "Legacy Korro" refers to the business of privately held Korro Bio, Inc., prior to completion of the merger. References to Frequency refer to Frequency Therapeutics, Inc. prior to completion of the merger.

Concurrently with the execution and delivery of the Merger Agreement, Legacy Korro entered into a subscription agreement with a number of accredited investors. Immediately prior to consummation of the Merger, Legacy Korro issued and sold an aggregate of 42,176,255 shares of its common stock at a purchase price of approximately $2.78 per share, for an aggregate purchase price of approximately $117.3 million. Shares of Legacy Korro common stock issued pursuant to the Pre-Closing Financing were converted into shares of the Company's common stock based on an exchange ratio (as defined below).

Pursuant to the terms of the Merger Agreement, immediately prior to the effective time of the Merger, each share of Legacy Korro preferred stock was converted into a share of Legacy Korro common stock, and then exchanged in the Merger for shares of Frequency common stock using an exchange ratio of 0.049688 (the "Exchange Ratio").

At the effective time of the Merger, the Company issued (or reserved for issuance upon exercise of options assumed in the Merger) an aggregate of approximately 7,848,776 shares of its common stock to Legacy Korro securityholders (before eliminating fractions), calculated as provided in the Merger Agreement, (the "Exchange"), resulting in approximately 8,623,645 shares of its common stock being issued and outstanding on a fully diluted basis immediately following the effective time of the Merger. This number includes shares of the Company's common stock that was issued upon vesting and settlement of certain outstanding equity awards at the effective time of the Merger. Immediately following the completion of the Merger, Frequency securityholders prior to the Merger owned approximately 9% of the outstanding shares of common stock on a fully diluted basis and Legacy Korro's securityholders, including those securityholders who purchased shares in Legacy Korro's pre-closing financing, owned approximately 91% of the outstanding shares on a fully diluted basis. The Merger was intended to qualify for federal income tax purposes as a tax-free reorganization under the provisions of Section 368(a) of the Internal Revenue Code of 1986, as amended (the "IRC").

Upon closing of the Merger, the Company assumed the Legacy Korro 2019 Stock Incentive Plan, and each outstanding and unexercised option to purchase Legacy Korro shares at such time, each of which converted into an option to purchase shares of the Company's common stock, with necessary adjustments to the number of shares and exercise price to reflect the Exchange Ratio. In addition, upon the closing of the Merger, the Company assumed each outstanding and unexercised warrant to purchase

Legacy Korro shares at such time, each of which converted into a warrant to purchase shares of the Company's common stock, with necessary adjustments to the number of shares and exercise price to reflect the Exchange Ratio.

At the effective time of the Merger, the Company entered into a contingent value rights agreement (the "CVR Agreement") with Computershare Trust Company, N.A. and Computershare Inc., collectively as rights agent providing for the payment of certain contingent cash payments equal to the net amount (calculated in accordance with GAAP consistently applied) of proceeds actually received by the Company or its subsidiaries after the end of each fiscal quarter following the first anniversary of the closing of the Transaction related to the disposition of assets related to Frequency's former multiple sclerosis programs, with the time periods and subject to deductions as provided therein. Concurrently, the Company entered into an asset purchase agreement with Progentos Therapeutics ("Progentos"), whereby Progentos acquired the rights, title and interest in certain assets related to Freqeuncy's MS program ("MS APA"). The MS APA included initial consideration of $0.5 million in proceeds that were settled through net cash at the merger closing and will be entitled to future milestone payments of up to $17.5 million as well as a $0.7 million payment in the event of Progentos closing of an equity financing at or above a specified amount.

The Merger was accounted for as a reverse recapitalization in accordance with U.S. GAAP. For accounting purposes, Legacy Korro was considered to be acquiring the assets and liabilities of Frequency in the Merger based on the terms of the Merger Agreement and other factors, including: (i) Legacy Korro controlling the majority of outstanding voting shares; (ii) Legacy Korro controlling the Board of Directors; (iii) Legacy Korro's executive management team became the management of the combined company; and (iv) the pre-combination assets of Frequency were primarily cash and cash equivalents and other non-operating assets. Accordingly, the Merger was treated as the equivalent of Legacy Korro issuing stock to acquire the net assets of Frequency. As a result of Legacy Korro being treated as the accounting acquirer, Legacy Korro's assets and liabilities were recorded at their pre-combination carrying amounts. Frequency's assets and liabilities were measured and recognized at their fair values, which approximated their carrying values as of the effective date of the Merger, and combined with the assets, liabilities, and results of operations of Legacy Korro after the consummation of the Merger. As a result, upon consummation the historical financial statements of Legacy Korro became the historical consolidated financial statements of the combined company.

Reverse Stock Split and Exchange Ratio

In connection with, and prior to the completion of, the merger, Frequency effected a one-for-fifty reverse stock split of its then outstanding common stock (the "Reverse Stock Split"). The par value and the authorized shares of the common stock were not adjusted as a result of the Reverse Stock Split. All issued and outstanding Legacy Korro common stock, convertible preferred stock and options prior to the effective date of the merger have been retroactively adjusted to reflect the merger 0.049688 Exchange Ratio, which reflects the impact of the reverse stock split, for all periods presented.

Liquidity and Capital Resources

The Company's consolidated financial statements have been prepared on the basis of the Company continuing as a going concern. The Company expects that its existing cash and cash equivalents as of December 31, 2023 of $166.1 million will enable the Company to fund its planned operating expense and capital expenditure requirements for at least twelve months from the date of issuance of these consolidated financial statements. The Company has incurred recurring losses and negative cash flows from operations since inception. As of December 31, 2023, the Company had an accumulated deficit of $183.0 million. The Company expects its operating losses and negative operating cash flows to continue into the foreseeable future. The future viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. There can be no assurance that the Company will ever earn revenues or achieve profitability, or if achieved, that the revenues or profitability will be sustained on a continuing basis. In addition, the Company's preclinical and clinical development activities, manufacturing and commercialization of the Company's product candidates, if approved, will require significant additional financing. However, if the Company is unable to obtain additional financing, the Company would be forced to delay, reduce or eliminate its research and development programs and/or relinquish valuable rights to its technology and product candidates. There is no assurance that the Company will be successful in obtaining sufficient financing on acceptable terms to continue funding its operations.

2. Basis of Presentation and Summary of Significant Accounting Policies

Basis of Presentation and Consolidation

The accompanying consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the United States of America ("GAAP"). These consolidated financial statements have been prepared on the going concern basis of accounting, which assumes continuity of operations, realization of assets and satisfaction of liabilities in the ordinary course of business.

The consolidated financial statements include the accounts of Korro Bio, Inc. and its wholly-owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision-maker in deciding how to allocate resources and assess performance. The Company and the Company's chief operating decision maker, the Company's chief executive officer, views the Company's operations and manages its business as a single operating segment. The Company operates only in the United States.

Use of Estimates

The preparation of the Company's consolidated financial statements requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to the fair value of the common stock prior to the effective date of the merger; the fair value of the CVR liability and the incremental borrowing rate for determining lease liabilities and right-of-use assets. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it has concluded to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates as there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results may differ materially from those estimates or assumptions.

Fair Value of Financial Instruments

ASC Topic 820, *Fair Value Measurement*, ("ASC 820") establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances.

ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

- Level 1 inputs are quoted prices (unadjusted) in active markets for identical assets or liabilities.
- Level 2 inputs are inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly.
- Level 3 inputs are unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability. Financial assets and liabilities are classified in their entirety based on the lowest level of input that is significant to the fair value measurement.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Cash Equivalents

Cash equivalents are highly-liquid investments that are readily convertible into cash with original maturities of three months or less when purchased. These assets include investments in money market funds that invest in U.S. Treasury obligations. Cash equivalents are reflected at fair value based on quoted market prices, as further described in Note 3, "Fair Value Measurements".

Investments

Investments consist of securities with original maturities greater than three months when purchased. Short-term investments consist of investments that are available for use in current operations. Long-term investments consist of investments with maturities of greater than one year that are not available for use in current operations. The Company did not maintain any long-term investments as of December 31, 2023 or 2022.

The Company classifies all of its investments as available-for-sale securities. Accordingly, these investments are recorded at fair value. Realized gains and losses and amortization and accretion of discounts and premiums are included in "Other income, net". Unrealized gains and losses on available-for-sale securities are included in "Accumulated other comprehensive loss" as a component of stockholders' deficit until realized.

The Company reviews its investment portfolio to identify and evaluate investments that have indicators of possible other-than-temporary impairment. Factors considered in determining whether a loss is other-than-temporary include the length of time and extent to which fair value has been less than the cost basis, the financial condition of the issuer and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value.

Property and Equipment

Property and equipment are recorded at cost and consists of laboratory equipment, furniture and office equipment, computer equipment, leasehold improvements, and construction in progress. The Company capitalizes property and equipment that is acquired for research and development activities and that has alternative future use. Expenditures for repairs and maintenance are recorded to expense as incurred, whereas major betterments are capitalized as additions to property and equipment. Property and equipment not yet placed into service is capitalized as construction in progress and is depreciated once placed into service. Leasehold improvements are depreciated over the lesser of their useful lives or the term of the lease. Depreciation, including depreciation for assets recorded under finance leases, is calculated over the estimated useful lives of the assets using the straight-line method.

Impairment of Long-lived Assets

The Company reviews long-lived assets when events or changes in circumstances indicate that the carrying value of an asset may not be recoverable. Recoverability is measured by comparing the carrying value of the asset to the future undiscounted cash flows from the use and eventual disposition of the asset. If an asset is considered to be impaired, the impairment loss to be recognized is measured as the amount by which the carrying value of the asset exceeds its fair value.

Leases

Under ASC Topic 842, *Leases* ("ASC 842"), the Company determines whether an arrangement is or contains a lease at inception. For leases with a term of 12 months or less, the Company does not recognize a right-of-use asset or lease liability. The Company's operating leases are recognized on its consolidated balance sheets as noncurrent assets, current liabilities and noncurrent liabilities. The Company does not have any finance leases.

Right-of-use assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease right-of-use assets and liabilities are recognized at the lease commencement date based on the present value of lease payments over the lease term. As the Company's leases typically do not provide an implicit rate, the Company uses an estimate of its incremental borrowing rate based on the information available at the lease commencement date in determining the present value of lease payments. Lease expense is recognized on a straight-line basis over the lease term. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew or options to terminate a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will exercise such options, respectively.

The Company has elected to account for the lease and non-lease components together for existing classes of underlying assets.

Preferred Stock

The Company applies the guidance of ASC Topic 480, Distinguishing Liabilities from Equity ("ASC 480"), when determining the classification and measurement of its preferred stock. Preferred stock subject to mandatory redemption (if any) are classified as liability instruments and are measured at fair value. The Company classifies contingently redeemable preferred stock (if any), which includes preferred stock that features redemption rights that are either within the control of the holder or subject to redemption upon the occurrence of uncertain events not solely within the Company's control, as temporary equity. At all other times, the Company classifies its preferred stock in stockholders' equity (deficit).

Prior to the merger, Legacy Korro classified its convertible preferred stock as temporary equity due to terms that allowed for redemption of the shares upon the occurrence of a contingent event that was not solely within the Legacy Korro's control. Legacy Korro did not accrete the carrying values of the preferred stock to the redemption values since the contingent event was not considered probable.

Research and Development Expenses

Expenditures relating to research and development are expensed as incurred. Research and development expenses include external expenses incurred under arrangements with third parties, academic and non-profit institutions and consultants; salaries and personnel-related costs, including non-cash stock-based compensation expense; license fees to acquire in-process technology and other expenses, which include direct and allocated expenses for laboratory, facilities and other costs. Non-refundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

As part of the process of preparing the consolidated financial statements, the Company is required to estimate its accrued research and development expenses as of each balance sheet date. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. This process involves reviewing open contracts and purchase orders, communicating with internal personnel to identify services that have been performed on the Company's behalf and estimating the level of service performed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of the actual cost. The Company periodically confirms the accuracy of its estimates with its service providers and makes adjustments if necessary. The majority of the Company's service providers invoice monthly in arrears for services performed or when contractual milestones are met. The financial terms of agreements with these service providers are subject to negotiation, vary from contract-to-contract and may result in uneven payment flows. In circumstances where amounts have been paid in excess of costs incurred, the Company records a prepaid expense.

Intellectual Property Expenses

The Company expenses legal costs related to patent applications as they are incurred. Such costs are classified as general and administrative expenses within the consolidated statements of operations and comprehensive loss.

Stock-based Compensation

The Company accounts for stock-based payments in accordance with ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). This guidance requires all stock-based payments, including grants of stock options and restricted common stock, to be recognized as expense in the consolidated statements of operations and comprehensive loss based on their grant date fair values. For stock options granted to employees, non-employees and members of the Company's Board of Directors for their services on the Board of Directors, the Company estimates the grant date fair value of each stock option using the Black-Scholes option-pricing model. For restricted common stock granted to employees and non-employees, the Company estimates the grant date fair value of each award using the intrinsic value, which is based on the value of the underlying common stock less any purchase price. For stock-based payments subject to service-based vesting conditions, the Company recognizes stock-based compensation expense equal to the grant date fair value of stock-based payment on a straight-line basis over the requisite service period.

Prior to the merger, due to the absence of an active market for Legacy Korro's common stock, Legacy Korro and its Board were required to determine the fair value of Legacy Korro's common stock at the time of each grant of a stock-based award. Legacy Korro estimated the grant date fair value of its common stock using an appropriate valuation methodology, in accordance with the framework of the American Institute of Certified Public Accountants' Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*. Historically, the Company has utilized a market approach to determine its total equity value and the option pricing method ("OPM") to allocate this equity value among various classes of securities. The OPM treats common stock and convertible preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under the OPM, the common stock has value only if the funds available for distribution to stockholders exceeds the value of the

convertible preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock.

Each valuation methodology includes estimates and assumptions that required the Legacy Korro's judgment. These estimates and assumptions include a number of objective and subjective factors, including external market conditions, guideline public company information, the prices at which Legacy Korro sold convertible preferred stock to third parties in arms' length transactions, the rights and preferences of securities senior to Legacy Korro's common stock at the time and the likelihood of achieving a liquidity event such as an initial public offering or sale. Significant changes to the assumptions used in the valuations could result in different fair values of stock options and restricted stock at each valuation date, as applicable.

In addition to the grant date fair value of the Company's common stock, the Black-Scholes option pricing model requires the input of certain subjective assumptions, including (i) the calculation of expected term of the stock-based payment, (ii) the risk-free interest rate, (iii) the expected stock price volatility and (iv) the expected dividend yield. The Company uses the simplified method as proscribed by SEC Staff Accounting Bulletin No. 107 to calculate the expected term for stock options granted to employees as the Company does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. The Company determines the risk-free interest rate based on a treasury instrument whose term is consistent with the expected term of the stock options. Due to the lack of Company-specific historical and implied volatility data, the Company bases its estimates of expected volatility on the historical volatility of a group of publicly-traded companies with similar characteristics to itself, including stage of product development and therapeutic focus within the life sciences industry. The expected volatility has been determined using a weighted-average of the historical volatility measures of this group of companies. The Company expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. Historical volatility is calculated over a period of time commensurate with the expected term of the stock-based payment. The Company uses an assumed dividend yield of zero as the Company has never paid dividends on its common stock, nor does it expect to pay dividends on its common stock in the foreseeable future.

The Company accounts for forfeitures of all stock-based payments when such forfeitures occur.

Income Taxes

Income taxes are recorded in accordance with ASC Topic 740, *Income Taxes*, which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the consolidated financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance against deferred tax assets is recorded if, based on the weight of the available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company accounts for uncertain tax positions using a more-likely-than-not threshold for recognizing and resolving uncertain tax positions. The evaluation of uncertain tax positions is based on factors, including, but not limited to, changes in the law, the measurement of tax positions taken or expected to be taken in tax returns, the effective settlement of matters subject to audit, new audit activity and changes in facts or circumstances related to a tax position.

Interest and penalty charges, if any, related to income taxes would be classified as a component of the "Provision for income taxes" in the consolidated statements of operations and comprehensive loss.

Net Loss per Share

Net loss per share attributable to common stockholders is calculated using the two-class method, which is an earnings allocation formula that determines net loss per share for the holders of the Company's common shares and participating securities. The Company's Preferred Stock contains participation rights in any dividend paid by the Company and is deemed to be a participating security. Net income attributable to common stockholders and participating preferred shares are allocated to each share as if all of the earnings for the period had been distributed. The participating securities do not include a contractual obligation to share in losses of the Company and are not included in the calculation of net loss per share in the periods in which a net loss is recorded. Net loss attributable to common stockholders is equal to the net loss for the period.

Diluted net loss per share is computed using the more dilutive of (a) the two-class method or (b) the treasury stock method and if-converted method. The Company allocates earnings first to preferred stockholders based on dividend rights and then to common and preferred stockholders based on ownership interests. The weighted-average number of common shares included in the computation of diluted net loss gives effect to all potentially dilutive common equivalent shares, including outstanding stock

options and Preferred Stock. Common stock equivalent shares are excluded from the computation of diluted net loss per share if their effect is antidilutive. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is generally the same as basic net loss per share attributable to common stockholders since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

Concentration of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially expose the Company to concentrations of credit risk primarily consist of cash, cash equivalents and investments. Cash balances are deposited with federally-insured financial institutions in the United States and may, at times, exceed federally-insured limits. The Company maintains its cash, cash equivalents and investments with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company's cash equivalents are comprised of money market funds that are invested in U.S. Treasury and government agency obligations. The Company's investments are comprised of commercial paper and government securities. Credit risk in these securities is reduced as a result of the Company's investment policy to limit the amount invested in any single issuer and to only invest in securities of a high credit quality.

The Company relies, and expects to continue to rely, on a small number of vendors to manufacture supplies and to process its product candidates for its development programs. These programs could be adversely affected by a significant interruption in the manufacturing process or supply chain.

The Company has no significant off-balance sheet risk such as foreign exchange contracts, option contracts or other foreign hedging arrangements.

Emerging growth company status

The Company qualifies as an "emerging growth company" ("EGC"), as defined in the Jumpstart Our Business Startups Act ("JOBS Act"), and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not EGCs. The Company may take advantage of these exemptions until it is no longer an EGC under Section 107 of the JOBS Act, which provides that an EGC can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. The Company has elected to avail itself of the extended transition period and, therefore, while the Company is an EGC it will not be subject to new or revised accounting standards the same time that they become applicable to other public companies that are not EGCs, unless it chooses to early adopt a new or revised accounting standard. As a result of this election, the consolidated financial statements may not be comparable to companies that comply with public company FASB standards' effective dates.

Recently Adopted Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*, which has been subsequently amended by ASU 2018-19, ASU 2019-04, ASU 2019-05, ASU 2019-10, ASU 2019-11, ASU 2020-03, and ASU 2022-02 ("ASU 2016-13"). This standard requires that credit losses be recorded using an expected losses model rather than the incurred losses model that was previously used and establishes additional credit risk disclosures associated with financial assets. The amendments in this standard should be applied on a modified retrospective basis to all periods presented. The Company adopted ASU 2016-13 on January 1, 2023 using the modified retrospective approach. The adoption of this standard did not have a material effect on the Company's financial position, results of operations or disclosures.

Recent Accounting Pronouncements—Yet to be Adopted

Other accounting standards that have been issued or proposed by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on the Company's consolidated financial statements upon adoption.

In November 2023, the FASB issued Accounting Standards Update ("ASU") 2023-07, *Segment Reporting (Topic 280), Improvements to Reportable Segment Disclosures*, which is intended to provide enhanced segment disclosures. The standard will require disclosures about significant segment expenses and other segment items and identifying the Chief Operating Decision Maker and how they use the reported segment profitability measures to assess segment performance and allocate resources. These enhanced disclosures are required for all public entities on an interim and annual basis, even if they have only a single reportable segment. The standard is effective for years beginning after December 15, 2023, and interim periods within annual periods beginning after December 15, 2024. Early adoption is permitted. The Company is evaluating this standard to determine if adoption will have a material impact on the Company's consolidated financial statements.

3. Reverse Merger

As described in Note 1, Legacy Korro merged with Frequency on November 3, 2023. The merger was accounted for as a reverse recapitalization with Legacy Korro as the accounting acquirer. The primary pre-combination assets of Frequency were cash and cash equivalents. Under reverse recapitalization accounting, the assets and liabilities of Frequency were recorded at their fair value which approximated book value due to the short-term nature of the accounts. No goodwill or intangible assets were recognized. Consequently, the consolidated financial statements of the Company reflect the operations of Legacy Korro for accounting purposes, together with a deemed issuance of shares equivalent to the shares held by the former stockholders of Frequency, the legal acquirer, and a recapitalization of the equity of Legacy Korro, the accounting acquirer.

As part of the reverse recapitalization, the Company acquired $36.6 million of cash and cash equivalents. The Company also obtained prepaids and other assets of $1.9 million and assumed accounts payable and accrued expenses of $5.4 million. Frequency's development programs had ceased prior to the merger and were deemed to be de minimis in value at the transaction date.

In addition, the Company recognized $0.3 million in share-based compensation expense as a result of the acceleration of vesting of stock options, performance stock units and restricted stock units at the time of merger. This amount was recorded in general and administrative expense in the accompanying consolidated statements of operations and comprehensive loss for the year ended December 31, 2023. The company recognized $2.5 million in personnel cost related to the severance payment to Frequency employees and this amount was recorded in general and administrative expense in the accompanying consolidated statements of operations and comprehensive loss for the year ended December 31, 2023. The Company also incurred transaction costs of $12.2 million and this amount is recorded in additional paid-in capital in the accompanying consolidated statements of convertible preferred stock and stockholders' equity (deficit) for the year ended December 31, 2023.

4. Fair Value Measurements

The Company measures the fair value of money market funds based on quoted prices in active markets for identical securities. Investments also include commercial paper and government securities that are valued either based on recent trades of securities in inactive markets or based on quoted market prices of similar instruments and other significant inputs derived from or corroborated by observable market data. The carrying amounts reflected in the consolidated balance sheets for cash, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values, due to their short-term nature.

Assets measured at fair value on a recurring basis as of December 31, 2023 were as follows (in thousands):

	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds, included in cash and cash equivalents	$ 158,706	$ 158,706	$ —	$ —
MS APA asset	$ 1,448	$ —	$ —	$ 1,448
Total	$ 160,154	$ 158,706	$ —	$ 1,448

Assets measured at fair value on a recurring basis as of December 31, 2022 were as follows (in thousands):

	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds, included in cash and cash equivalents	$ 14,904	$ 14,904	$ —	$ —
Short-term investments:				
Commercial paper	$ 14,935	—	14,935	—
Government securities	$ 3,980	—	3,980	—
Total	$ 33,819	$ 14,904	$ 18,915	$ —

As noted previously in Note 1, at the effective time of the Transaction, the Company entered into the CVR Agreement providing for the payment of certain contingent cash payments equal to the net amount of proceeds actually received by the Company or its subsidiaries after the end of each fiscal quarter following the first anniversary of the closing of the Transaction related to the disposition of assets related to the Company's former multiple sclerosis programs, with the time periods and subject to deductions as provided therein. The Company concluded that the CVR is a derivative liability and is accounted for at fair value, which was $1.4 million as of December 31, 2023 of which $0.4 million is included in accrued expenses and other current liabilities and the remaining $1.0 million in other non-current liabilities in the consolidated balance sheet. Concurrently, the Company entered into the MS APA with Progentos, whereby Progentos acquired the rights, title and interest in certain assets related to the Company's MS program. The MS APA included initial consideration of $0.5 million in proceeds that were settled through net cash at the merger closing and will be entitled to future milestone payments of up to $17.5 million as well as a $0.7 million payment in the event of Progentos closing of an equity financing at or above a specified amount. The Company concluded that the MS APA is a derivative asset and is accounted for at fair value, which was $1.4 million as of December 31, 2023 of which $0.4 million is included in prepaid and other current assets and the remaining $1.0 million in other long-term assets in the consolidated balance sheet.

The fair value of the CVR liability and the MS APA are based on significant unobservable inputs, which represent Level 3 measurements within the fair value hierarchy. In determining the fair value of the CVR liability and the MS APA asset, the Company used the income approach, primarily discounted cash flow models. The discounted cash flow models require the use of significant judgment, estimates and assumptions, including the probability of technical and regulatory success, and discount rates. For the year ended December 31, 2023, the aggregate change in fair value of the CVR liability and MS APA asset was $0.08 million.

There were no liabilities measured at fair value on a recurring basis as of December 31, 2022.

There were no changes in valuation techniques, nor were there any transfers among the fair value hierarchy levels during the years ended December 31, 2023 or 2022.

5. Investments

The Company did not have investments as of December 31, 2023.

Short-term investments as of December 31, 2022 were comprised as follows (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Commercial paper	$ 14,935	$ —	$ —	$ 14,935
Government securities	3,985	—	(5)	3,980
Total	$ 18,920	$ —	$ (5)	$ 18,915

As of December 31, 2022, the aggregate fair value of securities that were in an unrealized loss position for less than twelve months was $4.0 million. As of December 31, 2022, the Company held no securities that were in an unrealized loss position for more than twelve months. As of December 31, 2022, the Company did not intend to sell, and would not be more likely than not required to sell, the securities in an unrealized loss position before recovery of their amortized cost bases. Furthermore, the Company determined that there was no material change in the credit risk of these securities. As a result, the Company determined it did not hold any securities with any other-than-temporary impairment as of December 31, 2022.

6. Restricted Cash

As of December 31, 2023, the Company maintained current restricted cash of $3.6 million and non-current restricted cash of $3.4 million. As of December 31, 2022, the Company maintained current restricted cash of $0.6 million and non-current restricted cash of $4.5 million. All restricted cash amounts are comprised solely of letters of credit required pursuant to the Company's facility leases.

The following table provides a reconciliation of cash, cash equivalents and restricted cash as of December 31, 2023 and 2022 that sums to the total of the same amounts shown in the consolidated statements of cash flows (in thousands):

	December 31,	
	2023	2022
Cash and cash equivalents	$ 166,150	$ 36,333
Restricted cash	6,969	5,144
Cash, cash equivalents and restricted cash	$ 173,119	$ 41,477

7. Property and Equipment, Net

Property and equipment, net, as of December 31, 2023 and 2022 was comprised as follows (in thousands):

		December 31,	
	Estimated Useful Life (in Years)	2023	2022
Laboratory equipment	5	$ 11,187	$ 8,441
Furniture and office equipment	4	481	477
Computer equipment	3	241	213
Leasehold improvements	Shorter of useful life or remaining lease term	3,356	2,941
Construction in progress		8,242	2,049
Total property and equipment, gross		23,507	14,121
Less: accumulated depreciation		(7,842)	(4,255)
Total property and equipment, net		$ 15,665	$ 9,866

As of December 31, 2023 and 2022, the Company had construction in progress of $8.2 million and $2.0 million, predominately related to laboratory equipment received but not yet installed and capitalizable costs related to the Company's future corporate headquarters.

Depreciation expense for the years ended December 31, 2023 and 2022 was $3.6 million and $2.5 million, respectively.

8. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities as of December 31, 2023 and 2022 were comprised as follows (in thousands):

	December 31,	
	2023	2022
Employee compensation and benefits	$ 7,896	$ 2,624
External research and development services	1,255	274
Other operating expenses	447	207
CVR liability, current	395	—
Professional fees	219	70
Total accrued expenses and other current liabilities	$ 10,212	$ 3,175

9. Common Stock

At the closing of the merger, the shares of Legacy Korro common stock were converted into shares of the Company's common stock based on the exchange ratio determined in the Merger Agreement.

As of December 31, 2023, the Company was authorized to issue 200,000,000 shares of common stock, $0.001 per value per share. Prior to the merger, the holders of Legacy Korro's common stock were subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth in Note 10. Holders of common stock are entitled to one vote per share. In addition, holders of common stock are entitled to receive dividends, if and when declared by the Company's Board of Directors. As of December 31, 2023, no dividends had been declared.

As of December 31, 2023 and 2022, the Company had reserved for future issuance the following number of shares of common stock:

	December 31,	
	2023	2022
Conversion of outstanding Legacy Korro Series Seed Preferred Stock	—	684,739
Conversion of outstanding Legacy Korro Series A Preferred Stock	—	2,029,666
Conversion of outstanding Legacy Korro Series B-1 Preferred Stock	—	1,104,178
Conversion of outstanding Legacy Korro Series B-2 Preferred Stock	—	223,417
Future issuances of Legacy Korro Series B-2 Preferred Stock	—	813,239
Vesting of restricted common stock	—	2,902
Exercises of outstanding stock options	1,323,151	461,128
Exercise of outstanding warrant	8,049	8,049
Future issuances under 2019 Stock Incentive Plan	—	159,890
Future issuances under 2023 Stock Incentive Plan	255,694	—
Future issuances under 2023 ESPP Plan	88,502	—
Total reserved for future issuance	1,675,396	5,487,208

10. Preferred Stock

As of December 31, 2023, the Company was authorized to issue up to 10,000,000 shares of preferred stock at a par value of $0.001 with no preferred stock shares issued or outstanding.

Pursuant to the terms of the Merger Agreement, immediately prior to closing of the Merger, each share of Legacy Korro's Preferred Stock issued and outstanding immediately prior to the Closing of the Merger was converted into shares of the Legacy Korro's common stock, and then exchanged in the Merger for shares of the Company's common stock using an exchange ratio of 0.049688. The conversion was approved by greater than 66% of the then-outstanding shares of Preferred Stock, voting as a single class on an as-converted to common stock basis.

As of December 31, 2022, Legacy Korro was authorized to issue up to 10,000,000 shares of preferred stock at a par value of $0.001 with no preferred stock shares issued or outstanding. As of December 31, 2022, Legacy Korro Preferred Stock authorized, issued and outstanding consisted of the following (in thousands, except share amounts):

	December 31, 2022				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Value	Common Stock Issuable Upon Conversion
Legacy Korro Series Seed Preferred Stock	684,739	684,739	15,924	$ 16,115	684,739
Legacy Korro Series A Preferred Stock	2,029,666	2,029,666	77,736	91,500	2,029,666
Legacy Korro Series B-1 Preferred Stock	1,104,178	1,104,178	57,703	58,000	1,104,178
Legacy Korro Series B-2 Preferred Stock	1,036,656	223,417	12,500	12,500	223,417
Total	4,855,239	4,042,000	$ 163,863	$ 178,115	4,042,000

Rights, Preferences and Privileges of Legacy Korro Preferred Stock

The Legacy Korro Preferred Stock as of December 31, 2022 had the following rights and preferences. In the discussion below, the Legacy Korro Series Seed Preferred Stock and the Legacy Korro Series A Preferred Stock, the Legacy Korro Series B-1 Preferred Stock and the Legacy Korro Series B-2 Preferred Stock are collectively referred to as the " Legacy Korro Preferred Stock" unless specifically noted.

Conversion

Each share of Legacy Korro Preferred Stock were convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and non-assessable shares of common stock as is determined by dividing the original issuance price by the conversion price in effect at the time of conversion. The original conversion price of the Legacy Korro Series Seed 1 Preferred Stock and Legacy Korro Series Seed 2 Preferred Stock was $20.13, the original conversion price of the Legacy Korro Series Seed 3 Preferred Stock was $26.16, the original conversion price of the Legacy Korro Series A Preferred Stock was $45.08, the original conversion price of the Legacy Korro Series B-1 Preferred Stock was $52.53 and the original conversion price of the Legacy Korro Series B-2 Preferred Stock was $55.95. Shares of Legacy Korro preferred stock are subject to adjustments to reflect the issuance of common stock,

options, warrants, or other rights to subscribe for or to purchase common stock for a consideration per share, less than the conversion price then in effect and subsequent stock dividends, stock splits, combinations, or recapitalizations.

The Legacy Korro Preferred Stock was subject to mandatory conversion upon the closing of a sale of common stock to the public at a price of at least $111.90 per share (subject to appropriate adjustment in the event of a stock dividend, stock split, combination or other similar recapitalization) in a firm-commitment underwritten public offering resulting in at least $75.0 million of gross proceeds to Legacy Korro. The Legacy Korro Preferred Stock was also subject to mandatory conversion upon the vote or written consent of the holders of at least 66% of the then-outstanding shares of Legacy Korro Preferred Stock, voting as a single class on an as-converted to common stock basis.

Dividends

The holders of Legacy Korro Preferred Stock, in preference to common stockholders, were entitled to receive, when, as and if declared by the Company's Board of Directors, dividends at a rate of 8% annually. Dividends on Legacy Korro Preferred Stock were non-cumulative and were payable only when and if declared by Legacy Korro's Board of Directors. The holders of Legacy Korro Preferred Stock were entitled to participate in dividends on common stock on an as-converted basis when and if declared by Legacy Korro's Board of Directors. Since Legacy Korro's inception, no dividends have been declared.

Liquidation Preference

In the event of any voluntary or involuntary liquidation, dissolution, winding up or deemed liquidation event of Legacy Korro, the holders of shares of Legacy Korro Preferred Stock then outstanding were entitled to be paid out of the assets of Legacy Korro available for distribution to its stockholders before any payment shall be made to the holders of common stock, an amount equal to the original issue price per share plus any dividends declared but unpaid thereon. For clarity, the original issue price of the Legacy Korro Series Seed 1 Preferred Stock and Legacy Korro Series Seed 2 Preferred Stock was $20.13 per share, the original issue price of the Legacy Korro Series Seed 3 Preferred Stock was $26.16 per share, the original issue price of the Legacy Korro Series A Preferred Stock was $45.08 per share, the original issue price of the Legacy Korro Series B-1 Preferred Stock was $52.53 per share and the original issue price of the Legacy Korro Series B-2 Preferred Stock was $55.95 per share.

If upon any voluntary or involuntary liquidation, dissolution, winding up or deemed liquidation event of Legacy Korro, the assets of Legacy Korro available for distribution were insufficient to pay the holders of Legacy Korro Preferred Stock the full amount to which they were entitled, the holders of shares of Legacy Korro Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts that would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

Redemption

The Legacy Korro Preferred Stock was contingently redeemable upon the occurrence of a deemed liquidation event, which included a merger or a sale of substantially all of the assets of Legacy Korro. As of December 31, 2022, a deemed liquidation event was not considered to be probable.

Voting Rights

The holders of Legacy Korro Preferred Stock were entitled to vote based on the number of common shares that their preferred shares converted into on as-converted basis at the time of such vote. Except in specific circumstances, holders of Legacy Korro Preferred Stock shall vote as a single class with the common stockholders.

The holders of record of the shares of the Legacy Korro Series Seed Preferred Stock, voting exclusively and as a separate class on an as-converted to common stock basis, were entitled to elect two members to Legacy Korro's Board of Directors. The holders of record of the shares of the Legacy Korro Series A Preferred Stock, voting exclusively and as a separate class on an as-converted to common stock basis, were entitled to elect two members to the Legacy Korro's Board of Directors. The holders of record of the shares of the Legacy Korro Series B Preferred Stock, voting exclusively and as a separate class on an as-converted to common stock basis, were entitled to elect two members to the Legacy Korro's Board of Directors.

11. Stock-based Compensation

2023 Stock Option and Incentive Plan

In November 2023, the Company's board of directors adopted the Company's 2023 Stock Option and Incentive Plan (the "2023 Plan"). The 2023 Plan was approved by the Company's stockholders in November 2023 and became effective in November 2023

in connection with completion of the Merger. The 2023 Plan initially reserved 885,028 shares for the issuance of stock awards. In addition, the number of shares of common stock available for issuance under the 2023 Plan will be automatically increased on the first day of each calendar year during the ten-year term of the 2023 Plan, beginning with January 1, 2024 and ending with January 1, 2033, by the amount equal to 5% of the outstanding number of shares of the Company's common stock on December 31 of the preceding calendar year or such lesser amount as determined by the Company's board of directors. Awards granted under the 2023 Plan expire no later than ten years from the date of grant. For stock options, the option price shall generally not be less than 100% of the fair market value on the day of grant. As of December 31, 2023, there were in aggregate 889,922 shares of common stock reserved for issuance and 255,694 shares available to grant under the 2023 Plan.

2019 Stock Incentive Plan

In January 2019, the Legacy Korro's Board of Directors adopted the 2019 Stock Incentive Plan (as amended from time to time, the "Legacy Korro Plan"). The Legacy Korro Plan provides for the grant of stock options, stock awards and restricted stock units to employees, members of the Company's Board of Directors and non-employee consultants and advisors. The Legacy Korro Plan initially provided for the issuance of up to 110,285 shares of common stock. The Legacy Korro Plan was subsequently amended in May 2019, June 2020, October 2020, April 2021, November 2021 and March 2023 to modify the number of shares of common stock issuable under the Legacy Korro Plan. Subsequent to the March 2023 amendment to the Legacy Korro Plan, Legacy Korro could issue up to 747,752 shares of common stock under the Legacy Korro Plan.

In connection with the merger, each option to purchase shares of Legacy Korro common stock that was outstanding and unexercised under the Legacy Korro Plan immediately prior to the Effective Time, whether or not vested, was converted into an option to purchase shares of the Company's common stock and became eligible to be registered on Form S-8. The Company assumed each outstanding option to purchase shares of Legacy Korro common stock in accordance with the terms (as in effect as of the date of the Merger Agreement) of the Legacy Korro Plan and the terms of the stock option agreement by which such option to purchase shares of Legacy Korro common stock is evidenced. The number of shares under the Legacy Korro Plan subject to outstanding awards as of the effective date of the 2023 Plan that are subsequently canceled, forfeited or repurchased by the Company will be added to the shares reserved under the 2023 Plan.

The number of shares of common stock reserved for issuance pursuant to outstanding awards under the Legacy Korro Plan as of December 31, 2023 and 2022 was 600,977 shares and 683,156 shares, respectively. The number of shares available for grant under the Legacy Korro Plan was 0 and 159,890 at December 31, 2023 and 2022, respectively. Upon completion of the Merger and effectiveness of the 2023 Plan, the Company ceased granting awards under the Legacy Korro Plan.

2014 Stock Incentive Plan

On November 13, 2014, Frequency adopted the 2014 Stock Incentive Plan (as amended from time to time,the "2014 Plan"). All of Frequency's employees, officers, directors, and consultants were eligible to be granted options to purchase common shares, restricted stock units and restricted stock under the terms of the 2014 Plan. Frequency, reserved an aggregate of 424,853 shares of common stock for issuance under the 2014 Plan. The number of shares under the 2014 Plan subject to outstanding awards as of the effective date of the 2023 Plan that are subsequently canceled, forfeited or repurchased by the Company will be added to the shares reserved under the 2023 Plan. As of December 31, 2023, there were no shares of common stock available for future grants under the 2014 Plan and there were 36,455 shares subject to outstanding option awards under the 2014 Plan.

2019 Incentive Award Plan

On September 17, 2019, Frequency's board of directors and on September 19, 2019, its stockholders approved and adopted the 2019 Incentive Award Plan (the "2019 Plan"). Under the 2019 Plan, Frequency may grant stock options, stock appreciation rights, restricted stock, restricted stock units, and other stock and cash-based awards to individuals who are then employees, officers, directors or consultants of the Company, and employees and consultants of the Company's subsidiaries. A total of 154,032 shares of common stock were approved to be initially reserved for issuance under the 2019 plan. The number of shares under the 2019 Plan subject to outstanding awards as of the effective date of the 2023 Plan that are subsequently canceled, forfeited or repurchased by the Company will be added to the shares reserved under the 2023 Plan. Upon completion of the Merger and effectiveness of the 2023 Plan, the Company ceased granting awards under the 2019 Plan. Accordingly, as of December 31, 2023, there were no shares of common stock available for future grants under the 2019 Plan and there were 51,491 shares subject to outstanding option awards under the 2019 Plan.

All stock option grants are non-statutory stock options except option grants to employees (including officers and directors) intended to qualify as incentive stock options under the IRC. Incentive stock options may not be granted at less than the fair

market value of the Company's common stock on the date of grant. Nonqualified stock options may be granted at an exercise price established by the Board of Directors at its sole discretion (which has not been less than fair market value on the date of grant) and the vesting periods may vary. Vesting periods are generally four years and are determined by the Board of Directors. Stock options generally become exercisable as they vest. Options granted under the Legacy Korro Plan, 2014 Plan and the 2019 Plan expire no more than ten years from the date of grant.

Stock-based Compensation Expense

Total stock-based compensation expense recognized in the consolidated statements of operations and comprehensive loss for the years ended December 31, 2023 and 2022 was as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Research and development	$ 759	$ 243
General and administrative	1,622	897
Total stock-based compensation expense	$ 2,381	$ 1,140

Restricted Common Stock Activity

Prior to the adoption of the 2019 Plan, Legacy Korro issued shares of restricted common stock to its founders as well as to certain employees. The restrictions on the common stock generally lapse over two to four years. In the event that a recipient ceases to provide service to Legacy Korro, Legacy Korro has the right to repurchase any unvested shares of restricted common stock at their original purchase price. As a result of this repurchase right, Legacy Korro recorded the issuance of such restricted common stock as a liability in the consolidated balance sheets. Amounts are reclassified to common stock at par and additional paid-in capital as the restricted common stock vests and restrictions lapse.

The following table summarizes Legacy Korro restricted common stock activity during the year ended December 31, 2023:

	Shares	Weighted-Average Grant Date Fair Value per Share
Unvested as of December 31, 2022	2,902	$ 0.59
Granted	—	$ —
Vested	2,902	$ 0.23
Repurchased	—	$ —
Unvested as of December 31, 2023	—	$ —

The aggregate fair value of Legacy Korro restricted common stock that vested during the years ended December 31, 2023 and 2022, based upon the fair value of the underlying restricted common stock on the day of vesting, was $0.1 million and $0.5 million, respectively.

Stock Option Activity

The fair value of stock options granted during the years ended December 31, 2023 and 2022 was calculated on the date of grant using the following weighted-average assumptions:

	Year Ended December 31,	
	2023	2022
Risk-free interest rate	4.3%	2.5%
Expected dividend yield	—%	—%
Expected term (in years)	6.0	6.0
Expected volatility	72.8%	72.9%

Using the Black-Scholes option pricing model, the weighted-average grant date fair value of stock options granted during the years ended December 31, 2023 and 2022 was $23.49 and $14.95 per share, respectively.

The following table summarizes changes in stock option activity during the year ended December 31, 2023 (in thousands, except per share amounts):

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2022	461,100	$ 14.95	8.0	$ 2,953
Assumed in reverse recapitalization	93,153	$ 121.91		
Granted	835,839	$ 17.33		
Exercised	(39,395)	$ 16.41		
Forfeited	(20,091)	$ 20.07		
Cancelled	(7,455)	$ 18.85		
Outstanding as of December 31, 2023	1,323,151	$ 23.49	8.4	$ 36,310
Exercisable at December 31, 2023	368,036	$ 34.60	6.7	$ 8,457

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the common stock as of the end of the period. The aggregate intrinsic value of stock options exercised during the years ended December 31, 2023 and 2022 was $0.3 million and $0.1 million, respectively.

As of December 31, 2023, there was unrecognized stock-based compensation expense related to unvested stock options of $10.1 million, which the Company expects to recognize over a weighted-average period of approximately 3.1 years.

12. Income Taxes

The provision for income taxes for the years ended December 31, 2023 and 2022 was comprised as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Current taxes:		
Federal	$ —	$ —
State	27	10
Total current taxes	27	10
Deferred taxes:		
Federal	—	—
State	—	—
Total deferred taxes	—	—
Total provision for income taxes	$ 27	$ 10

A reconciliation of the federal statutory income tax rate to the Company's effective tax rate is as follows:

	December 31,	
	2023	2022
Income tax computed at federal statutory rate	21.0%	21.0%
State taxes, net of federal benefit	7.1%	6.1%
Tax credit carryforwards	3.6%	6.2%
Permanent items	(1.0)%	(0.2)%
Change in valuation allowance	(30.8)%	(32.7)%
Other	0.1%	(0.4)%
Effective tax rate	—%	—%

The principal components of the Company's deferred tax assets and liabilities as of December 31, 2023 and 2022 were comprised as follows (in thousands):

	December 31,	
	2023	2022
Deferred tax assets:		
Net operating loss carryforwards	$ 80,385	$ 19,580
Tax credit carryforwards	22,474	5,477
Capitalized research and development	34,395	10,279
Stock-based compensation	4,494	319
Amortization	1,138	—
Operating lease liability	9,052	855
Accrued expenses and other temporary differences	1,543	707
Total deferred tax assets	153,481	37,217
Less: valuation allowance	(145,766)	(36,094)
Net deferred tax assets	7,715	1,123
Deferred tax liabilities:		
Operating right-of-use asset	(7,401)	(552)
Depreciation	(314)	(571)
Total deferred tax liabilities	(7,715)	(1,123)
Net deferred taxes	$ —	$ —

As of December 31, 2023, the Company had federal and state net operating loss ("NOL") carryforwards of $302.5 million and $266.3 million, respectively. Under current law, the Company's federal NOLs generated in taxable years ending after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOLs is limited to 80% of the Company's taxable income annually for tax years beginning after December 31, 2018. Federal NOLs generated in taxable years ending on or prior to December 31, 2017, however, have a 20-year carryforward period, but are not subject to the 80% limitation. The Company has federal NOLs of $22.4 million that are subject to expiration between 2036 and 2037 and has $280.1 million of federal NOLs that do not expire.

State NOLs expire at various dates from 2035 through 2043. As of December 31, 2023, the Company had federal research and development tax credit carryforwards of $15.6 million that expire at various dates from 2036 through 2043. In addition, as of December 31, 2023, the Company had state research and development tax credit carryforwards of $8.7 million that expire at various dates from 2032 through 2038.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which primarily pertain to NOL carryforwards, tax credit carryforwards and capitalized research and development. The Company has determined that it is more likely than not that it will not realize the benefits of its deferred tax assets, and as a result, a valuation allowance of $145.8 million has been established at December 31, 2023. The increase in the valuation allowance of $109.7 million during the year ended December 31, 2023 was primarily due to the additional operating loss generated by the Company and the combination of deferred tax assets as a result of the Merger.

NOL and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50% as defined under Sections 382 and 383 in the IRC. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the Company's value immediately prior to the ownership change. As a result of ownership changes in the Company from its inception through December 31, 2023, the Company's NOL and tax credit carryforwards allocable to the periods preceding each such ownership change could be subject to limitations under IRC Section 382, however the Company has not yet completed an IRC Section 382 study.

The Company had no unrecognized tax benefits as of either December 31, 2023 or 2022. The Company has not conducted a study of its research and development credit carryforwards generated during any year. This study, once completed, may result in an adjustment to the Company's research and development credit carryforwards.

However, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credit carryforwards, and if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the consolidated statements of operations and comprehensive loss if an adjustment were required.

The Company files income tax returns in the United States federal tax jurisdiction and the Connecticut and Massachusetts state tax jurisdictions. Because the Company is in a loss carryforward position, it is generally subject to examination by federal and state tax authorities for all tax years in which a loss carryforward is available.

As of December 31, 2023, the Company has not incurred any material interest or penalty charges.

13. Genevant Agreement

In March 2023, Legacy Korro entered into a collaboration and license agreement (the "Genevant Agreement") with Genevant Sciences GmbH ("Genevant"). Key financial terms under the Genevant Agreement are as follows:

- The Company made a $2.5 million payment to Genevant in March 2023 upon execution of the Genevant Agreement and recorded the payment within research and development expense in the consolidated statement of operations for the year ended December 31, 2023.
- The Company will reimburse Genevant for certain out-of-pocket and full-time equivalent costs incurred as a result of research and development activities performed under the Genevant Agreement.
- Genevant is entitled to receive payments from the Company upon the achievement of certain milestones, including potential clinical milestone payments of up to $13.5 million, potential regulatory and development milestone payments of up to $27.0 million, and potential commercial milestone payments up to an aggregate total of $57.0 million.
- Genevant is eligible to receive royalties at percentage rates in the mid-single-digits, based on future annual net sales of licensed products within the scope of the Genevant Agreement.

As of December 31, 2023, no milestones have been achieved and the Company has recorded payments to Genevant of $1.2 million within research and development expense in the consolidated statement of operations during the year ended December 31, 2023.

14. Commitments and Contingencies

Leases

The Company is party to an operating lease at One Kendall Square, Cambridge, Massachusetts and occupies 22,561 square feet of laboratory and office space (the "OKS Facility"). On October 20, 2023, the Company entered into an amendment to the Lease Agreement that extended the lease expiration date from December 31, 2023 to September 30, 2024 and provided the Company with no option to further extend the lease expiration date. The Company is also party to an operating sublease agreement at Cummings Park in Woburn, Massachusetts and occupies 18,148 square feet of laboratory and office space (the "Cummings Park Sublease") which expires on July 31, 2024.

The Company is party to an operating lease for 50,453 square feet of office and laboratory space at 60 First Street, Cambridge, Massachusetts (the "60 First Street Lease"). In May 2023, the Company obtained control over the space and the Company recognized the operating lease right-of-use asset and the operating lease liability of $26.8 million on the commencement date of the lease. The total rental payments over the 11 year lease are expected to be $62.1 million, including rent credits and other lease incentives per the terms of the lease. Specifically, the 60 First Street Lease provides the Company with a tenant improvement allowance of $13.1 million. The Company utilized $2.3 million of the $13.1 million tenant improvement allowance as of December 31, 2023. The lease has remaining term approximately 10 years. The Company has an option to extend the lease for an additional period of five years with the rent during the option period being the then fair market rent.

Future minimum lease payments for all three leases, net of $10.8 million expected to be received and intended to be used related to the remaining tenant improvement allowance and rent credits associated with the 60 First Street Lease, as of December 31, 2023 were as follows (in thousands):

	As of December 31, 2023
2024	(7,227)
2025	6,247
2026	7,341
2027	7,557
Thereafter	52,498
Total Future Minimum Leases Payments	66,416
Less: Interest	33,209
Present Value of Operating Lease Liabilities	33,207

As of December 31, 2023, the weighted average remaining lease term was 10.0 years and the weighted average incremental borrowing rate used to determine the operating lease liability was 11.2%.

The Company combines the lease and non-lease components of fixed costs in its lease arrangements as a single lease component. Variable costs, such as utilities and maintenance costs, are not included in the measurement of right-of-use assets and lease liabilities, but rather are expensed when the event determining the amount of variable consideration to be paid occurs.

The following table summarizes the effect of lease costs in the Company's consolidated statement of operations and comprehensive loss of its operating leases (in thousands):

	Year Ended December 31,	
	2023	2022
Operating lease costs	$ 5,948	$ 1,675
Variable Lease Costs	1,211	732
Short-term Lease Costs	921	626
Total Lease Costs	$ 8,080	$ 3,033

Legal Contingencies

The Company accrues a liability for legal contingencies when it believes that it is both probable that a liability has been incurred and that the Company can reasonably estimate the amount of the loss. The Company reviews these accruals and adjusts them to reflect ongoing negotiations, settlements, rulings, advice of legal counsel and other relevant information. To the extent new information is obtained and the views on the probable outcomes of claims, suits, assessments, investigations or legal proceedings change, changes in the Company's accrued liabilities would be recorded in the period in which such determination is made.

In addition, in accordance with the relevant authoritative guidance, for any matters in which the likelihood of material loss is at least reasonably possible, the Company will provide disclosure of the possible loss or range of loss. If a reasonable estimate cannot be made, however, the Company will provide disclosure to that effect. The Company expenses legal costs as they are incurred.

On June 3, 2021 and June 22, 2021, purported stockholders of the Company filed putative class action lawsuits in the U.S. District Court for the District of Massachusetts against the Frequency and the Frequency's Chief Executive Officer, President, and Director, David Lucchino. On March 21, 2022, the two lawsuits were consolidated into a single lawsuit, Quinones et al. v. Frequency Therapeutics, Inc. et al. and on May 16, 2022, the Company's Chief Development Officer, Dr. Carl Le Bel, was added as a defendant. The plaintiffs alleged violations of Sections 10(b), 20(a) and Rule 10b5 of the Securities Exchange Act of 1934, as amended (the Exchange Act), due to allegedly false and misleading statements and omissions about the Company's Phase 2a clinical trial (FX-322-202) for its product candidate FX-322 in the Company's public disclosures between October 29, 2020 and March 22,2021. The lawsuit sought, among other things, damages in connection with the Company's allegedly artificially inflated stock price between October 29,2020 and March 22, 2021 as a result of those allegedly false and misleading statements and omissions, as well as interest, attorneys' fees and costs. The Company filed a motion to dismiss the Amended Complaint on July 15, 2022. On March 29, 2023, the Company's motion to dismiss was granted and the lawsuit was dismissed in its entirety. On April 27, 2023, Plaintiff filed a notice of appeal to the United States Court of Appeals for the First Circuit from the order dismissing the lawsuit. On August 2, 2023, Plaintiff-Appellant submitted its opening brief to the First Circuit. The Company filed its response brief on October 27, 2023. The First Circuit heard oral argument on January 8, 2024, and has not yet issued a decision on plaintiff's appeal. This matter is at the very early stages of the legal process, and as a result, the Company is not able to estimate a range of possible loss. Since an estimate of the possible loss or range of loss cannot be made at this time, no accruals have been recorded as of December 31, 2023.

Indemnifications

The Company indemnifies each of its officers and directors for certain events or occurrences, subject to certain limits, while the officer or director is or was serving at the Company's request in such capacity, as permitted under Delaware law and in accordance with the Company's amended and restated certificate of incorporation and bylaws. The term of the indemnification period lasts as long as an officer or director may be subject to any proceeding arising out of acts or omissions of such officer or director in such capacity.

The maximum amount of potential future indemnification is unlimited; however, the Company currently holds director and officer liability insurance. This insurance allows the transfer of risk associated with the Company's exposure and may enable the Company to recover a portion of any future amounts paid. The Company believes that the fair value of these potential indemnification obligations is minimal. Accordingly, the Company has not recognized any liabilities relating to these obligations for any period presented.

15. 401(k) Savings Plan

The Company has a defined-contribution savings plan under Section 401(k) of the IRC (the "401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation, subject to statutory limitations. Beginning on April 1, 2022, the Company matches 100% of an employee's 401(k) contributions up to a maximum of 3% of the participant's salary, subject to employer match limitations under the IRC. As such, the Company made $0.5 million and $0.2 million of matching contributions to the 401(k) Plan during the year ended December 31, 2023 and December 31, 2022, respectively.

16. Related Party Transactions

As a result of Atlas' ownership of the Legacy Korro's Series Seed Preferred Stock, Series A Preferred Stock and Series B Preferred Stock which was exchanged in the Merger for the Company's common stock, Atlas represents an affiliate of the Company. During the years ended December 31, 2023 and 2022, the Company incurred no expense and expenses of less than $0.1 million, respectively, related to consulting services provided by an affiliate of Atlas. As of December 31, 2023 and 2022, the Company had no amounts due to this affiliate.

17. Net Loss per Share

The following common stock equivalents have been excluded from the calculation of diluted net loss per share because their effect would be anti-dilutive:

	Year Ended December 31,	
	2023	2022
Legacy Korro Series Seed Preferred Stock	—	684,739
Legacy Korro Series A Preferred Stock	—	2,029,666
Legacy Korro Series B-1 Preferred Stock	—	1,104,178
Legacy Korro Series B-2 Preferred Stock	—	223,417
Unvested Legacy Korro restricted common stock	—	2,902
Outstanding Legacy Korro stock options	1,323,151	461,100
Outstanding Legacy Korro warrant	8,049	8,049
Total	1,331,200	4,514,051

Basic and diluted loss per share is computed by dividing net loss by the weighted-average common shares outstanding. The following table sets forth the computation of the Company's basic and diluted net loss per share (in thousands, except share and per share data):

	Years Ended December 31,	
	2023	2022
Numerator:		
Net loss	$ (81,172)	$ (58,032)
Denominator:		
Weighted-average number of shares outstanding, basic and diluted	1,529,321	255,175
Net loss per share, basic and diluted	$ (53.08)	$ (227.42)

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures" as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act that are designed to ensure that information required to be disclosed in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, who serve as our principal executive officer and principal financial officer, respectively, has evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2023. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2023.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. We maintain a system of internal control that is designed to provide reasonable assurance regarding the reliability of our financial reporting and the preparation of consolidated financial statements for external purposes in accordance with generally accepted accounting principles.

Notwithstanding that we do not qualify for the relief afforded by Instruction 1 to Item 308 of Regulation S-K to newly public companies, management has not assessed nor attested to our internal control over financial reporting as is set forth in Item 308 of Regulation S-K promulgated under the Exchange Act, and Section 404 of the Sarbanes-Oxley Act as of December 31, 2023, the end of our last fiscal year. We will do so initially as of December 31, 2024.

We were unable to conduct the required assessment primarily due to the Merger occurring on November 3, 2023 and the resulting substantial change in operational focus, management and the internal control environment. Following the Merger, Legacy Korro's historical operations, and not that of our predecessor business, represent virtually the entirety of the combined business. In addition, following the Merger, our accounting and financial systems, as well as personnel, were replaced by those of Legacy Korro. Due to the extensive changes to our internal control environment, it was impractical for us to develop, implement, and assess our system of internal control over financial reporting, and conduct management's assessment of internal control over financial reporting as of December 31, 2023.

Changes in Internal Control over Financial Reporting

Other than changes due to the Merger, there was no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during our most recently completed fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

(a) On March 21, 2024, our board of directors established that our 2024 Annual Meeting of Stockholders will be held on Tuesday, June 11, 2024. The record date for the determination of stockholders entitled to receive notice of and to vote at our 2024 Annual Meeting will be the close of business on Wednesday, April 17, 2024. Because the date of our 2024 Annual Meeting differs by more than thirty (30) days from the anniversary date of our 2023 Special Meeting in lieu of an Annual Meeting of Stockholders, which was held on November 3, 2023, we ares hereby providing notice, pursuant to Rule 14a-5(f) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, of the deadlines for any stockholder proposals pursuant to Rule 14a-8 under the Exchange Act and for any stockholder nomination or proposal outside of Rule 14a-8 via this Form 10-K.

To be considered for inclusion in this year's proxy materials for our 2024 Annual Meeting, stockholder proposals must be submitted in writing by the close of business on Tuesday, April 9, 2024, to our Corporate Secretary at our principal executive offices at One Kendall Square, Building 600-700, Suite 6-401, Cambridge, MA 02139. In addition to complying with this deadline, stockholder proposals intended to be considered for inclusion in the proxy materials for our 2024 Annual Meeting must also comply with our bylaws and all applicable rules and regulations promulgated by the Securities and Exchange Commission under the Exchange Act. Additionally, any stockholder who intends to submit a proposal regarding a director nomination or any other matter of business at our 2024 Annual Meeting not to be included in our proxy materials for our 2024 Annual Meeting, must also ensure that notice of any such proposal (including any additional information specified in our bylaws) is received by our Corporate Secretary at our principal executive offices by the close of business on April 9, 2024.

(b) None.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Executive Officers and Directors

Our directors and executive officers and their ages as of the date of this Annual Report are listed below:

Name	Age	Position
Executive Officers:		
Ram Aiyar	46	President, Chief Executive Officer and Director
Vineet Agarwal	42	Chief Financial Officer
Todd Chappell	51	Chief Operating Officer
Steve Colletti	58	Chief Scientific Officer
Shelby Walker	49	General Counsel
Non-Employee Directors:		
Ali Behbahani (2)(3)	47	Director
Nessan Bermingham (1)(2)	51	Director
Jean-Francois Formela (1)(3)	67	Director
Rachel Meyers(3)	60	Director
Timothy Pearson (1)(2)	56	Director
David L. Lucchino	55	Director

(1) Audit Committee member
(2) Compensation Committee member
(3) Nominating and Corporate Governance Committee member

Executive Officers

Ram Aiyar, Ph.D., M.B.A. Dr. Aiyar has served as our Chief Executive Officer and a director since completion of the Merger. Dr. Aiyar previously served as Chief Executive Officer and as a director of Legacy Korro since November 2020, and has served as its President since November 2021. Prior to joining Legacy Korro, Dr. Aiyar co-founded Corvidia Therapeutics, Inc. and most recently served its as Chief Financial Officer from January 2020 to November 2020 and Executive Vice President, Corporate and Business Development from February 2016 to November 2020. Prior to that, Dr. Aiyar held leadership roles in corporate development, product development, management, research, finance and strategy at BeneVir BioPharma, Inc., BioHealth Innovation, Inc., FlowMetric, Inc., Sofinnova Partners, J.P. Morgan Chase and Johnson & Johnson Pharmaceuticals (NYSE:JNJ). Dr. Aiyar is a co-founder and director of Protean Bio, Inc., a director of Triveni Bio, Inc. and a past director of Avidea Technologies, Inc. Dr. Aiyar holds an M.B.A. in finance and business strategy from INSEAD (France/Singapore), an M.S. in computer engineering and a Ph.D. in electrical and computer engineering from Drexel University, and a B.E. in electronics engineering from Mumbai University. We believe Dr. Aiyar is qualified to serve on the Board because of his significant operational and senior management experience in the biopharmaceutical industry.

Vineet Agarwal, M.B.A. Mr. Agarwal served as Chief Financial Officer since the Merger and previously served as Chief Financial Officer of Legacy Korro since May 2021. Prior to joining Korro Bio, Mr. Agarwal joined J.P. Morgan Chase & Co. In 2007 and advised healthcare companies on merger & acquisitions, capital raising and strategic initiatives. Mr. Agarwal served as Executive Director, Biotech Investment Banking at J.P. Morgan Chase & Co. from January 2019 until May 2021 and as Vice President, Biotech Investment Banking from January 2016 until January 2019. Mr. Agarwal previously served in numerous leadership roles at J.P. Morgan Chase & Co. across different countries. Mr. Agarwal holds an M.B.A. from the Institute of Management Technology, India, and a Bachelor's degree in finance from Shri Ram College of Commerce, India.

Todd Chappell, M.B.A. Mr. Chappell has served as Chief Operating Officer since the Merger and previously served as Chief Operating Officer of Legacy Korro since August 2023 and previously served as Senior Vice President, Strategy and Portfolio Planning of Korro Bio from March 2021. Before joining Korro Bio, Mr. Chappell served as Chief Executive Officer of Rasio Therapeutics, Inc. from June 2019 until March 2021. Prior to this role, he served as Chief Executive Officer of Perceptive Navigation, LLC from June 2015 until May 2019. Mr. Chappell previously managed a portfolio of start-up pharmaceutical and medical device companies as an entrepreneur-in-residence at BioHealth Innovation, Inc. Prior to that, Mr. Chappell was a Vice President of Operations at Shape Pharmaceuticals, Inc., a portfolio company of HealthCare Ventures, LLC, where he oversaw all day-to-day operations for the development of a novel HDAC inhibitor for cutaneous t-cell lymphoma. Prior to this role, Mr. Chappell was an Executive Director of New Products at CombinatoRx, Inc., where he led the advancement of three programs

from assay stage into human clinical studies. Mr. Chappell holds an M.B.A. from Boston University and a B.S. in biology from the University of California, Los Angeles.

Steve Colletti, Ph.D. Dr. Colletti served as Chief Scientific Officer since the Merger and previously served as Chief Scientific Officer of Legacy Korro since February 2023. Dr. Colletti most recently served as Senior Vice President of Drug Discovery Research and Development at Zymergen, Inc. from May 2021 to January 2023. Prior to this role, he served as Chief Scientific Officer of Lodo Therapeutics from March 2020 to May 2021 and as Senior Vice President, Head of Research and Development from September 2018 to March 2020. He previously held multiple leadership roles at Merck (NYSE:MRK), including in small molecule, natural products, oligonucleotide, peptide and fusion protein bioconjugate drug discovery, targeting programs in cardiovascular and respiratory disease, diabetes and obesity, immunological disorders, infectious diseases, neuroscience and oncology. Also at Merck, Dr. Colletti built and led the RNA Therapeutics Medicinal Chemistry department and was a core member of multiple development teams responsible for discovering more than a dozen preclinical candidates and advancing them to clinical development. Dr. Colletti is an inventor and author of over 130 patents and publications. Dr. Colletti holds a Ph.D. in chemistry from Boston University and a B.S. in chemistry from Loyola University, and was a National Institutes of Health postdoctoral fellow in chemistry at the Scripps Research Institute.

Shelby J. Walker, M.S., J.D. Ms. Walker has served as Senior Vice President, General Counsel and Corporate Secretary since the Merger and previously served as Senior Vice President, General Counsel and Corporate Secretary of Legacy Korro since May 2023. Ms. Walker most recently served as Senior Vice President and Head of Intellectual Property at CRISPR Therapeutics (Nasdaq:CRSP) from March 2018 to April 2023. She previously served as General Counsel at Ginkgo Bioworks, a synthetic biology company, from May 2016 to March 2018. Prior to this role, she served as Vice President, Associate General Counsel and Chief Intellectual Property Counsel at Dyax Corporation, and previously held intellectual property leadership roles at Novo Nordisk (NYSE:NVO) and ZymoGenetics. Ms. Walker holds a J.D. and L.L.M. in intellectual property law from the University of New Hampshire School of Law, master's degrees in biotechnology and regulatory science from Johns Hopkins University, and a B.S. in biotechnology from Worcester Polytechnic Institute.

Non-Employee Directors

Nessan Bermingham, Ph.D. Dr. Bermingham, one of Legacy Korro's co-founders, has served as Chairperson of the Board since completion of the Merger, and previously served as Legacy Korro's Chairman and on its board of directors since November 2021, and previously served as its President and Executive Chairman from November 2018 to November 2021. Dr. Bermingham has been an Operating Partner at Khosla Ventures since December 2021 and has served as Interim Chief Executive Officer of Everyone Medicines since October 2022. Previously, he co-founded and served as President and Chief Executive Officer of Triplet Therapeutics from November 2018 until July 2021. Dr. Bermingham was also a Venture Partner at Atlas Venture from February 2018 until July 2021. Dr. Bermingham also served as Interim Chief Executive Officer of Liberate Bio from October 2022 until February 2023. Prior to that role, Dr. Bermingham co-founded and served as President and Chief Executive Officer of Intellia Therapeutics (Nasdaq:NTLA) from 2014 to 2017. Dr. Bermingham currently serves on the boards of directors of a number of private companies and previously served on the board of Xilio Therapeutics (Nasdaq:XLO). He also previously served as the chair of the board of F-Star Therapeutics prior to its reverse merger and subsequent to its acquisition as a public company, and served on the boards of several private companies. Dr. Bermingham holds a bachelor's degree in genetics from Queen's University Belfast and a Ph.D. in molecular biology from Imperial College London, and was a Howard Hughes Associate Fellow at Baylor College of Medicine. We believe Dr. Bermingham is qualified to serve on the Board because of his significant leadership and investment experience in the biotech industry.

Ali Behbahani, M.D., M.B.A. Dr. Behbahani has served on the Board since completion of the Merger, and previously served as a member of Legacy Korro's board of directors since August 2019. Dr. Behbahani joined New Enterprise Associates, Inc., or NEA, in 2007 and is a General Partner on the healthcare team. He previously held positions at The Medicines Company, Morgan Stanley Venture Partners and Lehman Brothers. Dr. Behbahani has served as a member of the board of directors of Monte Rosa Therapeutics, Inc. (Nasdaq:GLUE) since April 2020, Black Diamond Therapeutics (Nasdaq:BDTX) since December 2018, Nkarta, Inc. (Nasdaq:NKTX) since August 2015, CRISPR Therapeutics AG (Nasdaq:CRSP) since April 2015, Arcellx, Inc. (Nasdaq:ACLX) since February 2015, Adaptimmune Therapeutics Plc (Nasdaq:ADAP) since September 2014, CVRx, Inc. (Nasdaq:CVRX) since July 2013, Minerva Surgical, Inc. (Nasdaq:UTRS) since May 2011, and was on the board of Nevro Corp. (NYSE:NVRO) from August 2014 to March 2019, Genocea Biosciences (Nasdaq:GNCA) from February 2018 to May 2022, and Oyster Point Pharma (Nasdaq:OYST) from July 2017 to January 2023. He also serves on a number of private company boards. Dr. Behbahani holds a B.S. in biomedical engineering, electrical engineering and chemistry from Duke University, an M.B.A. from the Wharton School of the University of Pennsylvania and an M.D. from the University of Pennsylvania School of Medicine. We believe Dr. Behbahani is qualified to serve on the Board because of his extensive experience as a public company director and investor in the biotech industry.

Jean-Francois Formela, M.D., M.B.A. Dr. Formela, one of Legacy Korro's co-founders, has served as a member of the Board since completion of the Merger, and previously served on Legacy Korro's board of directors since November 2018. Dr. Formela is currently a partner at Atlas Venture, a life sciences-focused venture capital firm, which he joined in 1993. Dr. Formela is a co-founder and director of IFM Therapeutics, and serves as a director of Ikena Oncology, Inc. (Nasdaq:IKNA), as well as a director of the following private companies: Scorpion Therapeutics, Inc., Sail Bio, Inc., Triveni Bio, Inc. and Travin Bio, Inc. Dr. Formela also previously served as a director of Intellia Therapeutics, Inc. (Nasdaq:NTLA), Spero Therapeutics (Nasdaq:SPRO) and several private companies. Dr. Formela is a member of the Mass General Brigham Innovation Advisory Board and a former trustee of the Boston Institute of Contemporary Art. Dr. Formela began his career as a physician practicing emergency medicine at Necker University Hospital in Paris. He holds an M.D. from the Paris University School of Medicine and an M.B.A. from Columbia University. We believe Dr. Formela's experience as an investor and board member in the life sciences industry, as well as his scientific and medical knowledge, provides him with the qualifications and skills to serve on the Board.

David L. Lucchino previously served as our President and Chief Executive Officer through completion of the Merger and has also served on the Board, each since November 2014 and was a co-founder of Frequency with Dr. Robert S. Langer and Dr. Christopher R. Loose. From December 2014 until June 2016, Mr. Lucchino served as the President of Entrega Bio, a biotechnology company focused on oral drug delivery technology. Prior to that, Mr. Lucchino cofounded Semprus BioSciences, or Semprus, a biotechnology company, and served as its President and Chief Executive Officer from June 2007 to June 2012. Mr. Lucchino oversaw the development of Semprus' lead medical product, which received FDA clearance in 2012. Semprus was acquired by Teleflex, Inc., or Teleflex, in June 2012. Prior to Semprus, Mr. Lucchino worked at the investment firm Polaris Partners. He started his biotech career by Co-Founding LaunchCyte, an investment firm where he was also a Managing Director. Mr. Lucchino is the past chairman of the board of directors of MassBio, a nonprofit organization that represents over 1,500 life science firms and provides services and support for the biotechnology industry in Massachusetts. He is a member of the College of Fellows of the American Institute for Medical and Biological Engineering and was appointed by Massachusetts' Governor Charlie Baker as a member of the Commonwealth's STEM Advisory Council. Mr. Lucchino also served as a trustee of Mt. Auburn Hospital, a Harvard Medical School facility for fifteen years, a trustee of the Multiple Myeloma Research Foundation, and a member of the Board of NOLS (The National Outdoor Leadership School). Mr. Lucchino holds an MBA from the Massachusetts Institute of Technology's Sloan School of Management, an M.S. from the Newhouse School of Journalism at Syracuse University, and a B.A. in Philosophy and Religious Studies from Denison University. We believe Mr. Lucchino is qualified to continue to serve on the Board because of his extensive management experience in the biotechnology and pharmaceutical industry.

Rachel Meyers, Ph.D. Dr. Meyers has served as a member of the Board since November 2023. Dr. Meyers previously served as the Founder and Chief Scientific Officer at Faze Medicines, Inc. from June 2020 to January 2023. Prior to Faze Medicines, Dr. Meyers served as Entrepreneur-in-Residence at Third Rock Ventures. She also spent over 13 years at Alynlam Pharmaceuticals, Inc. from April 2003 to November 2016 and remains an active member of the scientific advisory board. Dr. Meyers also serves on several scientific advisory boards, including the National Advisory Board on Innovation and Entrepreneurship through the Department of Commerce. Rachel is listed as an inventor on many patents and patent applications, and has numerous peer-reviewed publications. Dr. Meyers completed her postdoctoral training at Harvard Medical School in the field of signal transduction and received her Ph.D. at the Massachusetts Institute of Technology in the field of in vitro transcription. We believe Dr. Meyers' extensive experience in the biotech industry and her expertise in drug discovery and development, including in RNA-based medicines, provide her with the qualifications and skills to serve on the Board.

Timothy R. Pearson. Mr. Pearson has served on the Board since completion of the Merger, and has served as the Chief Executive Officer of Carrick Therapeutics, a privately held oncology company, since July 2019. Mr. Pearson served as an Executive Vice President and the Chief Financial Officer of TESARO, Inc., an oncology-focused biopharmaceutical company, from 2014 until its acquisition by GlaxoSmithKline in February 2019. He served as an Executive Vice President, Chief Financial Officer and Treasurer of Catalyst Health Solutions, a publicly held pharmacy benefit management company, from 2011 until its acquisition by SXC Health Solutions in 2012. Prior to joining Catalyst Health Solutions, Mr. Pearson served as the Chief Financial Officer and Executive Vice President of MedImmune, Inc. Mr. Pearson has served on the board of directors of GlycoMimetics, Inc. (Nasdaq:GLYC) since 2014 and as its chairperson since 2019. He previously served on the board of directors of Ra Pharmaceuticals, Inc., a publicly held biopharmaceutical company until its acquisition by UCB in April 2020. Mr. Pearson is a Certified Public Accountant and holds dual B.S. degrees in business administration from the University of Delaware and in accounting from the University of Maryland, University College, as well as an M.S. degree in finance from Loyola College. We believe Mr. Pearson is qualified to serve on the Board because of his experience in the biopharmaceutical industry and his expertise in accounting and finance, strategic planning and leadership of complex organizations, and human capital management.

Election of Officers

Our executive officers are appointed by, and serve at the discretion of, our board of directors. There are no family relationships among any of our directors or executive officers.

Our Board of Directors

Our board of directors currently consists of seven directors divided into three staggered classes, with one class to be elected at each annual meeting to serve for a three-year term.

There are no family relationships among any of our directors and officers.

Committees of Our Board of Directors

The standing committees of our board of directors of are the following: audit committee, compensation committee and a nominating and corporate governance committee, and each operate pursuant to a charter. Our board of directors may establish other committees from time to time to assist us and our board of directors.

Audit Committee

Our audit committee oversees our corporate accounting and financial reporting process. Among other matters, the audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of, our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending, based upon the audit committee's review and discussions with management and our independent registered public accounting firm, whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

The audit committee consists of Timothy Pearson, Nessan Bermingham and Jean-Francois Formela. Timothy Pearson is the chair of the audit committee and is a financial expert under the rules of the SEC. To qualify as independent to serve on our audit committee, listing standards of Nasdaq and the applicable SEC rules require that a director not accept any consulting, advisory or other compensatory fee from us, other than for service as a director, or be an affiliated person of us. We believe that the composition of the audit committee complies with the applicable requirements of the rules and regulations of Nasdaq and the SEC.

Compensation Committee

Our compensation committee will oversee policies relating to compensation and benefits of our officers and employees. Among other matters, the compensation committee's responsibilities include:

- annually reviewing and recommending to the board of directors the corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and, based on such evaluation, recommending to the board of directors the cash compensation of our Chief Executive Officer;
- determining the cash compensation of our other executive officers;
- overseeing and administering our compensation and similar plans;
- reviewing and approving the retention or termination of any consulting firm or outside advisor to assist in the evaluation of compensation matters and evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- retaining and approving the compensation of any compensation advisors;
- reviewing and approving the grant of equity-based awards;
- reviewing and recommending to the board of directors the compensation of our directors; and
- preparing the compensation committee report required by SEC rules, if and when required, to be included in our annual proxy statement.

The compensation committee consists of Nessan Bermingham, Ali Behbahani and Timothy Pearson. Nessan Bermingham is the chair of the compensation committee. Each member of our compensation committee is a "non-employee" director within the meaning of Rule 16b-3 of the rules promulgated under the Exchange Act and independent within the meaning of the independent director guidelines of Nasdaq. We believe that the composition of the compensation committee complies with the applicable requirements of the rules and regulations of Nasdaq.

Nominating and Corporate Governance Committee

The nominating and corporate governance responsibilities include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees;
- reviewing and recommending to the board of directors appropriate corporate governance guidelines; and
- overseeing the evaluation of the board of directors.

The nominating and corporate governance committee consists of Ali Behbahani, Jean-Francois Formela and Rachel Meyers. Ali Behbahani is the chair of the nominating and corporate governance committee. We believe that the composition of the nominating and corporate governance committee meets the requirements for independence under, and the functioning of such nominating and corporate governance committee complies with, any applicable requirements of the rules and regulations of Nasdaq.

Delinquent Section 16(a) Reports

Section 16(a) of the Exchange Act requires our directors and executive officers, and persons who beneficially own more than 10% of a registered class of our equity securities, to file with the SEC initial reports of ownership and reports of changes in ownership of our common stock and other equity securities. Officers, directors and greater than 10% beneficial owners are required by SEC regulations to furnish us with copies of all Section 16(a) forms they file.

To our knowledge, based solely on our review of Forms 3, 4 and 5, and any amendments thereto, filed by such reporting persons and/ or written representations that no Form 5 was required, we believe that during the fiscal year ended December 31, 2023, all filing requirements applicable to our executive officers, directors and persons who beneficially own more than 10%

percent of a registered class of our equity securities under the Exchange Act were met in a timely manner except for (i) two late Form 4 filings for David L. Lucchino with respect to the vesting of his restricted stock units, or RSUs, and two sales to cover tax withholding obligations in connection with the vesting of the RSUs and (ii) one late Form 4 filing for Richard J. Mitrano with respect to one sale required to cover tax withholding obligations in connection with the vesting of RSUs pursuant to a Rule 10b5-1 trading plan.

Code of Business Conduct and Ethics

We have adopted an amended and restated code of business conduct and ethics for directors, officers, and employees, known as the Code of Business Conduct and Ethics. The Code of Business Conduct and Ethics is available on our website at https://ir.korrobio.com/corporate-governance/governance-overview. We will promptly disclose on our website (i) the nature of any amendment to the policy that applies to our principal executive officer, principal financial officer, or controller, or persons performing similar functions and (ii) the nature of any waiver, including an implicit waiver, from a provision of the policy that is granted to one of these specified individuals, the name of such person who is granted the waiver and the date of the waiver. Stockholders may request a free copy of the Code of Business Conduct and Ethics from our Corporate Secretary, c/o Korro Bio, Inc., One Kendall Square, Building 600-700, Suite 6-401 Cambridge, MA 02139.

Compensation Recovery Policy

In accordance with the requirements of the SEC and Nasdaq listing rules, our board of directors adopted a compensation recovery policy on November 3, 2023, effective as of October 2, 2023. The compensation recovery policy provides that in the event we are required to prepare a restatement of financial statements due to material noncompliance with any financial reporting requirement under securities laws, we will seek to recover any incentive-based compensation that was based upon the attainment of a financial reporting measure and that was received by any current or former executive officer during the three-year period preceding the date that the restatement was required if such compensation exceeds the amount that the executive officers would have received based on the restated financial statements. We have filed this policy as an Exhibit to this Annual Report on Form 10-K.

Item 11. Executive Compensation.

On November 3, 2023, Frequency completed the Merger with Legacy Korro. At the effective time of the Merger, the management of Frequency was replaced with the management of Legacy Korro. Unless otherwise indicated, the disclosures in this section regarding Frequency's common stock or securities convertible into common stock for periods or as of a date that precedes the closing of the Merger have been adjusted to give effect to the Reverse Stock Split, and the disclosures in this section regarding Legacy Korro's common stock or securities convertible into Legacy Korro's common stock for periods or as of a date that precedes the closing of the Merger have been adjusted to give effect to the Exchange Ratio and the Reverse Stock Split. This discussion may contain forward-looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. Actual compensation programs that we adopt in the future could vary significantly from our historical practices and currently planned programs summarized in this discussion.

Executive Compensation Overview

This section discusses the material components of the executive compensation program for our named executive officers, which consists of (i) any person who served as our principal executive officer during any part of 2023, (ii) our two most highly compensated executive officers (other than our principal executive officers) who were serving as executive officers on December 31, 2023, and (iii) one additional individual who would have been under clause (ii) but for the fact that he was not serving as an executive officer on December 31, 2023. In 2023, our named executive officers were:

- Ram Aiyar, Ph.D., our Chief Executive Officer and President;
- David L. Lucchino, our former President and Chief Executive Officer;
- Vineet Agarwal, our Chief Financial Officer;
- Steven Colletti, Ph.D., our Chief Scientific Officer; and
- Christopher R. Loose, Ph.D., our former Chief Scientific Officer.

2023 Summary Compensation Table

The following table presents information regarding the total compensation awarded to, earned by and paid to our named executive officers for services during 2023.

Name and Principal Position	Year	Salary ($)	Bonus ($)	Stock Awards ($)(1)	Option Awards ($)(2)	Nonequity Incentive Plan Compensation ($)(3)	All Other Compensation ($)	Total ($)
Ram Aiyar	2023	498,487	-	-	2,013,364	336,479	9,900(4)	2,858,230
Chief Executive Officer and President	2022	472,500	-	-	546,835	170,100	7,110	1,196,545
David L. Lucchino	2023	558,767(6)	-	1,338,400	-	-	1,981,098(7)	3,878,265
Former President and Chief Executive Officer (5)	2022	630,000	-	1,209,000	927,028	378,000	15,250	3,159,278
Vineet Agarwal	2023	426,484	250,000(8)	-	511,121	125,386	9,900(4)	1,322,891
Chief Financial Officer	2022	404,250	-	-	88,676	127,339	8,743	629,008
Steven Colletti *Chief Scientific Officer(9)*	2023	361,667(6)	-	-	1,087,385	151,269	47,047(10)	1,647,368
Christopher R. Loose	2023	294,211(6)	-	533,358(12)	-	-	752,905(13)	1,580,474
Former Chief Scientific Officer (11)	2022	480,344	-	604,500	226,580	192,138	15,250	1,518,812

1. Amounts represent the aggregate grant date value of the RSUs granted to our named executive officers during the applicable year, computed in accordance with FASB ASC Topic 718, rather than the amounts paid to or realized by the named executive officer. Such grant date fair values do not take into account any estimated forfeitures. The assumptions used in calculating the grant date fair values of the RSUs reported in this column are set forth in Note 11 of our financial statements included elsewhere in this Annual Report on Form 10-K. These amounts reported in this column reflect the accounting cost for these RSUs and do not correspond to the economic value that may be received by our named executive officers upon vesting and settlement of such awards or any sale of the shares of our common stock received.

2. Amounts represent the aggregate grant date fair value of the stock option awards granted to our named executive officers during the applicable fiscal year, computed in accordance with FASB ASC Topic 718, rather than the amounts paid to or realized by the named executive officer. Such grant date fair values do not take into account any estimated forfeitures. The assumptions used in calculating the grant date fair value of the stock option awards reported in this column are set forth in Note 11 of our financial statements included elsewhere in this Annual Report on Form 10-K. The amounts reported in this column reflect the accounting cost for these stock option awards and do not correspond to the actual economic value that may be received by our named executive officers upon the exercise of the stock option awards or any sale of the underlying shares of our common stock.

3. Amounts represent cash incentive compensation awarded in recognition of individual and/or company performance under our annual incentive compensation program. Refer to "—2023 Bonuses" below for additional information.

4. Amount reported represents matching contributions to the named executive officer's 401(k) account.

5. Mr. Lucchino's employment terminated in connection with the Merger but he continues to provide services to us as a non-employee member of our board.

6. Amount reported reflects the prorated base salary for the named executive officer's partial year of service with us during the applicable year.

7. The amount reported represents severance paid to Mr. Lucchino in connection with his termination of employment in the amount of $1,958,224, comprised of $992,250, which represents 18 months of Mr. Lucchino's 2023 base salary, $333,833, which represents Mr. Lucchino's prorated annual bonus for the 2023 fiscal year, $396,900, which

represents Mr. Lucchino's target annual bonus for the 2023 fiscal year, $61,473 in company-paid COBRA premiums, and $173,768, which represents the value of the accelerated vesting of Mr. Lucchino's equity awards in connection with his termination of employment as well as $16,500 in matching contributions made by us under the Frequency 401(k) plan and $6,374, the compensation earned by Mr. Lucchino for services on our board of directors after the Merger.

8. Amount represents a transaction bonus awarded in connection with the completion of the Merger.

9. Dr. Colletti was not a named executive officer for 2022.

10. Amount represents $9,713 in matching contributions made by us under our 401(k) retirement plan and reimbursement of $37,334 for expenses incurred in connection with travel to work at our offices and housing in the greater Boston, Massachusetts area.

11. Dr. Loose departed our company in connection with the Merger.

12. Amount reported includes $31,458, which represents the incremental fair value, computed in accordance with FASB ASC Topic 718, related to the amendment of Dr. Loose's RSUs in 2023 to accelerate the vesting of such RSUs in connection with termination of Dr. Loose's employment.

13. The amount reported represents severance paid to Dr. Loose in connection with his termination of employment in the amount of $736,405, comprised of $504,361 of continued base salary payments, $201,744, which represents Dr. Loose's target annual bonus for the 2023 fiscal year, and $30,300 in company-paid COBRA premiums as well as $16,500 in matching contributions made by us under the Frequency 401(k) plan.

Narrative Disclosure to the 2023 Summary Compensation Table

Prior to the Merger, our compensation committee reviewed compensation annually for all employees, including our executive officers. Following the Merger, our compensation committee anticipates annually reviewing the compensation of our employees, including our executive officers. In setting executive base salaries and bonuses and granting equity incentive awards, the compensation committee considers compensation for comparable positions in the market, the historical compensation levels of our executive officers, individual performance as compared to our expectations and objectives, internal equity, our desire to motivate our employees to achieve short- and long-term results that are in the best interests of our stockholders, and a long-term commitment to us. We target a general competitive position and consider independent third-party benchmark analytics to determine the mix of compensation of base salary, bonus and long-term incentives.

Our compensation committee is primarily responsible for determining the compensation for our executive officers. Our compensation committee typically reviews and discusses management's proposed compensation with our Chief Executive Officer for all executives other than the Chief Executive Officer. Based on those discussions and its discretion, taking into account the factors noted above, the compensation committee then sets the compensation for each executive officer other than the Chief Executive Officer. For the Chief Executive Officer, our compensation committee determines and approves the compensation, or upon request of the board of directors, recommends our Chief Executive Officer's compensation for approval by our board of directors. Our compensation committee may delegate certain authorities to an officer of our company and has delegated to our Chief Executive Officer the authority to make certain equity award grants to employees (other than our executive officers), within specified limits approved by the compensation committee. Our compensation committee has the authority to engage the services of a consulting firm or other outside advisor to assist it in designing our executive compensation programs and in making compensation decisions. During 2023, the compensation committee retained the services of Alpine Rewards LLC, or Alpine, as its external compensation consultant to advise on executive compensation matters including our overall compensation program design and collection of market data to inform our compensation programs for our executive officers and members of our board of directors. Alpine reports directly to our compensation committee. Our compensation committee annually assesses its independence consistent with Nasdaq listing standards and concluded that the engagement of such consultant did not raise any conflict of interest.

2023 Base Salaries

Each of the named executive officers' base salary is a fixed component of annual compensation for performing specific duties and functions. Base salaries are adjusted from time to time to realign salaries with market levels after taking into account individual responsibilities, performance and experience. For the fiscal year ended December 31, 2023, the annual base salaries for Dr. Aiyar, Mr. Lucchino (prior to termination of his employment), Mr. Agarwal, Dr. Colletti, and Dr. Loose (prior to termination of his employment) were $498,487, $661,500, $426,484, $420,000, and $504,361, respectively.

2023 Annual Bonuses

During the year ended December 31, 2023, Dr. Aiyar, Mr. Lucchino (prior to termination of his employment), Mr. Agarwal, Dr. Colletti, and Dr. Loose (prior to termination of his employment), were each eligible to earn an annual bonus based on their individual performance and/or our performance as a company. For the year ended December 31, 2023, the target annual

bonuses for Dr. Aiyar, Mr. Lucchino (prior to termination of his employment), Mr. Agarwal, Dr. Colletti, and Dr. Loose (prior to termination of his employment) were equal to 45%, 60%, 35%, 35%, and 40%, respectively, of their applicable annual base salaries.

Equity Incentive Compensation

Although neither Legacy Korro nor Frequency had a formal policy with respect to the grant of equity incentive awards to its executive officers, we believe that equity awards provide our executive officers with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and stockholders. In addition, we believe that equity awards with a time-based vesting feature promote executive retention because this feature incentivizes our executive officers to remain in our employment during the applicable vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our named executive officers and from time to time may grant equity incentive awards to them. In 2023, we granted options and RSUs to our named executive officers, as described in more detail in the "—Outstanding Equity Awards at Fiscal 2023 Year-End" table below.

Perquisites

We generally do not provide perquisites to our employees, other than certain de minimis perquisites available to all of our employees, including our named executive officers. However, pursuant to his employment agreement with us, Dr. Colletti is also entitled to reimbursement (i) for reasonable expenses incurred in connection with travel to work at our offices and (ii) up to $4,000 per month for housing in the greater Boston, Massachusetts area.

401(k) Plan

We maintain the Korro Bio 401K Retirement Plan, a tax-qualified retirement plan that provides eligible employees, including the named executive officers, with an opportunity to save for retirement on a tax-advantaged basis. Plan participants are able to defer eligible compensation subject to applicable annual limits under the Code. Participants' pre-tax or Roth contributions are allocated to each participant's individual account and are then invested in selected investment alternatives according to the participants' directions. Participants are immediately and fully vested in their contributions. We match each participant's contribution up to a maximum of 3% of his or her eligible compensation with participants vesting immediately and fully in such matching contributions. Our 401(k) plan is intended to be qualified under Section 401(a) of the Code with the 401(k) plan's related trust intended to be tax exempt under Section 501(a) of the Code.

Prior to the consummation of the Merger, Frequency maintained a 401(k) retirement savings plan, or the Frequency 401(k) Plan, for Frequency employees. Prior to their respective terminations, Mr. Lucchino and Dr. Loose were eligible to participate in the Frequency 401(k) Plan on the same terms as other full-time Frequency employees. Under this plan, Frequency matched 100% of the first 5% of participants' contributions.

Prior Offer Letters and Employment Agreements

Legacy Korro previously entered into offer letters or employment agreements with Dr. Aiyar, Mr. Agarwal and Dr. Colletti and Frequency previously entered into employment agreements with Mr. Lucchino and Dr. Loose, which were in effect in 2023 prior to the Merger and are described below. We entered new agreements with certain of our named executive officers following the Merger, as described further below.

Ram Aiyar

On October 13, 2020, Legacy Korro entered into an offer letter with Dr. Aiyar, or the Aiyar Offer Letter. The Aiyar Offer Letter sets forth his initial annual base salary of $450,000, initial target bonus opportunity equal to 40% of Dr. Aiyar's annual base salary, initial equity grant, and his eligibility to participate in our employee benefit plans generally.

The Aiyar Offer Letter provided that in the event that Dr. Aiyar's employment was terminated by Legacy Korro without "cause" or by him for "good reason" (as such terms are defined in the Aiyar Offer Letter), in either case within three months before or 12 months following a change in control, or the Aiyar Change in Control Period, subject to Dr. Aiyar's signing and complying with a separation agreement and release, Dr. Aiyar would be entitled to the following severance benefits: (i) a lump sum cash payment equal to sum of (x) 12 months of his then-current base salary (or the base salary in effect immediately prior to the change in control, if higher), plus (y) 100% of Dr. Aiyar's target annual bonus for the year of termination, without regard to whether the metrics had been established or achieved for such year; (ii) if Dr. Aiyar elected COBRA health continuation, a monthly payment to the group health plan provider or the COBRA provider for up to 12 months, and (iii) 100% of all equity awards held by Dr. Aiyar would have immediately accelerated in vesting and become fully exercisable or non-forfeitable.

In addition, in the event that Dr. Aiyar's employment were terminated by Legacy Korro without "cause" or by him for "good reason", in either case outside the Aiyar Change in Control Period, subject to Dr. Aiyar's signing and complying with a separation agreement and release, Dr. Aiyar would have been entitled to the severance benefits as described in the preceding paragraph, payable in substantially equal installments over a 12-month period, provided that Dr. Aiyar would not have been

entitled to any acceleration of vesting of his equity awards and the target annual bonus described above would have been prorated based on the date of termination.

David Lucchino

Frequency entered into a second amended and restated executive employment agreement with Mr. Lucchino on September 20, 2019, pursuant to which Frequency employed Mr. Lucchino as its President and Chief Executive Officer. The employment agreement also provided for Mr. Lucchino to serve as a member of the Frequency's board of directors for as long as he was employed as its Chief Executive Officer. The employment agreement had an indefinite term.

The employment agreement provided for an initial annual base salary of $525,000, which was increased to $630,000 effective January 1, 2022, and for an initial target annual performance bonus equal to 55%, which was increased to 60% effective January 1, 2021, of Mr. Lucchino's annual base salary based on the attainment of predetermined performance objectives agreed upon between Mr. Lucchino and Frequency's board of directors. In the event of certain corporate transactions, including a spin-off of assets or a restructuring, Mr. Lucchino would have been entitled to the same relative ownership percentage in the resulting entity or entities as he had in the company immediately before the corporate transaction. If a "change in control" (as such term is defined in his employment agreement) occurred, all of Mr. Lucchino's time-based equity awards would accelerate and vest.

On November 1, 2023 and in connection with Mr. Lucchino's departure from Frequency in connection with the Merger, Mr. Lucchino signed a separation agreement pursuant to which he was entitled to receive the following termination payments, subject to his non-revocation of a release in our favor: (i) eighteen months' base salary, (ii) 100% of his target annual bonus and (iii) a pro-rated portion of his annual target bonus based on the portion of the year he was employed. The payments under clauses (i)-(iii) were payable in a single lump sum on the first payroll date following the 60th day after his termination of employment. Mr. Lucchino also received up to twelve months' continued coverage, at our expense, under COBRA, if he elected such continued coverage. In addition to the termination payments, all equity awards granted to Mr. Lucchino accelerated in vesting (including any awards subject to performance-based vesting).

Mr. Lucchino was also party to restrictive covenant agreements, pursuant to which he agreed to refrain from competing with us or soliciting its customers or employees during his employment and for one year following termination of his employment and from disclosing our proprietary information during or at any time following his employment.

Vineet Agarwal

On March 12, 2021, Legacy Korro entered into an employment agreement with Mr. Agarwal, or the Prior Agarwal Employment Agreement. The Prior Agarwal Employment Agreement set forth his initial annual base salary of $385,000, initial target bonus opportunity equal to 35% of Mr. Agarwal's base salary, initial equity grant, and his eligibility to participate in our employee benefit plans generally.

The Prior Agarwal Employment Agreement provided that in the event that Mr. Agarwal's employment were terminated by Legacy Korro without "cause" or by him for "good reason" (as such terms are defined in the Prior Agarwal Employment Agreement), in either case within 12 months following a change in control, or the Agarwal Change in Control Period, subject to Mr. Agarwal's signing and complying with a separation agreement and release, Mr. Agarwal would have been entitled to the following severance benefits: (i) a lump sum cash payment equal to sum of (x) nine months of his then-current base salary (or the base salary in effect immediately prior to the change in control, if higher), plus (y) 100% of Mr. Agarwal's target annual bonus for the year of termination, without regard to whether the metrics had been established or achieved for such year; (ii) if Mr. Agarwal elected COBRA health continuation, a monthly payment to the group health plan provider or the COBRA provider for up to nine months, and (iii) 100% of all equity awards subject to time-based vesting held by Mr. Agarwal would have immediately accelerated in vesting and become fully exercisable or non-forfeitable.

In addition, in the event that Mr. Agarwal's employment were terminated by Legacy Korro without "cause" or by him for "good reason", in each case outside the Agarwal Change in Control Period, subject to Mr. Agarwal's signing and complying with a separation agreement and release, Mr. Agarwal would have been entitled to the severance benefits as described in the preceding paragraph, payable in substantially equal installments over a nine-month period, provided that Mr. Agarwal would not have been entitled to any acceleration of vesting of his equity awards and the target annual bonus described above would have been prorated based on the date of termination.

Steven Colletti

On January 20, 2023, Legacy Korro entered into an employment agreement with Dr. Colletti, or the Prior Colletti Employment Agreement. The Prior Colletti Employment Agreement set forth his initial annual base salary of $420,000, initial target bonus opportunity equal to 35% of Dr. Colletti's base salary, initial equity grant, and his eligibility to participate in our employee benefit plans generally.

The Prior Colletti Employment Agreement provided that in the event that Dr. Colletti's employment were terminated by Legacy Korro without "cause" or by him for "good reason" (as such terms are defined in the Prior Colletti Employment Agreement), in either case within 12 months following a change in control, or the Colletti Change in Control Period, subject to

Dr. Colletti's signing and complying with a separation agreement and release, Dr. Colletti would have been entitled to the following severance benefits: (i) a lump sum cash payment equal to sum of (x) nine months of his then-current base salary (or the base salary in effect immediately prior to the change in control, if higher), plus (y) 100% of Dr. Colletti's target annual bonus for the year of termination, without regard to whether the metrics had been established or achieved for such year; (ii) if Dr. Colletti elected COBRA health continuation, a monthly payment to the group health plan provider or the COBRA provider for up to nine months, and (iii) 100% of all equity awards subject to time-based vesting held by Dr. Colletti would have immediately accelerated in vesting and become fully exercisable or non-forfeitable.

In addition, in the event that Dr. Colletti's employment were terminated by Legacy Korro without "cause" or by him for "good reason", in each case outside the Colletti Change in Control Period, subject to Dr. Colletti's signing and complying with a separation agreement and release, Dr. Colletti would have been entitled to the severance benefits as described in the preceding paragraph, payable in substantially equal installments over a nine-month period, provided that Dr. Colletti would not have been entitled to any acceleration of vesting of his equity awards and the target annual bonus described above would have been prorated based on the date of termination.

Christopher R. Loose

Frequency entered into an amended and restated employment agreement with Dr. Loose on September 20, 2019, pursuant to which Frequency employed Dr. Loose as its Chief Scientific Officer. Dr. Loose's employment agreement had an indefinite term.

Dr. Loose's employment agreement provided for an annual base salary of $425,000, which was increased to $480,344 effective January 1, 2022, and a target annual performance bonus equal to 40% of his base salary, and was based on the attainment of predetermined individual and company performance objectives agreed upon between Dr. Loose and Frequency.

In the event Frequency terminated Dr. Loose's employment without "cause" or he resigned for "good reason" (as such terms are defined in his employment agreement), subject to his execution and non-revocation of a release in our favor, he was entitled to receive the following termination payments: (i) 12 months' continued base salary in equal installments following his termination, (ii) 100% of his target annual bonus paid in a lump sum within 14 days following his execution of the release and (iii) if he made an election, up to twelve months continued coverage under COBRA, with us paying the same portion of the COBRA premiums as we pays for active employees. If such a qualifying termination occurs within twelve months following a "change in control" (as defined in his employment agreement), Dr. Loose's equity awards would have accelerated and vested.

Dr. Loose was a party to restrictive covenant agreements, pursuant to which he has agreed to refrain from competing with us or soliciting our customers or employees during his employment and for one year following termination of his employment and from disclosing our proprietary information during or at any time following his employment.

On July 28, 2023, Frequency entered into a separation agreement with Dr. Loose pursuant to which, in exchange for a general release of claims in our favor, he was entitled to receive (i) continued payment of his base salary for a period of 12 months from July 28, 2023, (ii) continued group health plan coverage under COBRA for up to 12 months, with Frequency paying the portion of the premium that it would pay for active and similarly situated employees, (iii) $201,744.35, which was equal to 100% of his 2023 target bonus opportunity, and (iv) accelerated vesting of all of his unvested stock options and RSUs, subject to the closing of the Merger prior to or on December 31, 2023.

Current Offer Letters and Employment Agreements

We entered new employment agreements with certain of our executive officers following the closing of the Merger, the terms of which are described below.

Ram Aiyar

On November 10, 2023, we entered into a new employment agreement with Dr. Ram Aiyar, our President and Chief Executive Officer, or the Aiyar Employment Agreement. Under the Aiyar Employment Agreement, Dr. Aiyar has an initial annual base salary of $498,487 and an initial target bonus opportunity equal to 45% of his annual base salary, he continues to remain eligible for equity grants under our equity incentive plans, and he continues to be eligible to participate in our employee benefit plans generally.

The Aiyar Employment Agreement provides that in the event Dr. Aiyar is terminated without "cause" or he resigns for "good reason" (as such terms are defined therein) outside of the change in control period (which extends from three months prior to a change in control to 12 months following a change in control, as "change in control" is defined therein), subject to signing and complying with a separation agreement and release, which shall include, without limitation, a release of claims, a reaffirmation of restrictive covenants, and in our sole discretion, a one year post-employment noncompetition agreement, then Dr. Aiyar will be entitled to the following severance benefits: (i) 12 months of his then-current base salary, (ii) a pro rata target bonus for the year of termination, without regard to whether the metrics have been established or achieved for such year, and (iii) subject to his copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA

health coverage, up to 12 months of payment of the portion of the premium equal to the amount we would have paid to provide health insurance had he remained employed by us.

The Aiyar Employment Agreement provides enhanced severance pay and benefits in the event Dr. Aiyar's employment is terminated by us without cause or he resigns for good reason, in each case, within the change in control period. Such enhanced severance pay and benefits consist of (i) a lump sum cash payment equal to the sum of (A) 18 months of Dr. Aiyar's then-current base salary (or the base salary in effect immediately prior to the change in control, if higher) plus (B) 1.5 times Dr. Aiyar's target annual bonus for the then-current year, without regard to whether the metrics have been established or achieved for such year, (ii) subject to his copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, up to 18 months of payment of the portion of the premium equal to the amount we would have paid to provide health insurance had he remained employed by us, and (iii) accelerated vesting of the then-outstanding and unvested portion of Dr. Aiyar's stock options and other stock-based awards that are subject solely to time-based vesting and any stock options and other stock-based awards that were granted to him prior to the effective date of the Aiyar Employment Agreement and that are subject to performance-based vesting. The severance pay and benefits described in this paragraph are subject to Dr. Aiyar's delivery of and compliance with a fully effective release of claims.

The payments and benefits under the Aiyar Employment Agreement in connection with a change in control may not be eligible for federal income tax deduction by us pursuant to Section 280G of the Code. These payments and benefits may also be subject to an excise tax under Section 4999 of the Code. If the payments or benefits payable to Dr. Aiyar in connection with a change in control would be subject to the excise tax imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to him.

Dr. Aiyar entered into an Employee Proprietary Information and Inventions Assignment Agreement that contains, among other provisions, nondisclosure of confidential information, invention assignment and nonsolicitation provisions.

Vineet Agarwal

On November 8, 2023, we entered into a new employment agreement with Vineet Agarwal, our Treasurer and Chief Financial Officer, or the Agarwal Employment Agreement. Under Mr. Agarwal's new employment agreement, he has an initial annual base salary of $426,483, an initial target bonus opportunity equal to 35% of his annual base salary and continues to remain eligible for equity grants under our equity incentive plans, and continues to be eligible to participate in our employee benefit plans generally.

The Agarwal Employment Agreement provides that in the event Mr. Agarwal is terminated without "cause" or he resigns for "good reason" (as such terms are defined therein) outside of the change in control period (which extends from three months prior to a change in control to 12 months following a change in control, as "change in control" is defined therein), subject to him signing and complying with a separation agreement and release, which shall include, without limitation, a release of claims, a reaffirmation of restrictive covenants, and in our sole discretion, a one year post-employment noncompetition agreement, then Mr. Agarwal will be entitled to the following severance benefits: (i) nine months of his then-current base salary, (ii) a pro rata target bonus for the year of termination, without regard to whether the metrics have been established or achieved for such year, and (iii) subject to his copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, up to nine months of payment of the portion of the premium equal to the amount we would have paid to provide health insurance had he remained employed by us.

The Agarwal Employment Agreement provides enhanced severance pay and benefits in the event Mr. Agarwal's employment is terminated by us without cause or Mr. Agarwal resigns for good reason, in each case, within the change in control period. Such enhanced severance pay and benefits consist of (i) a lump sum cash payment equal to the sum of (A) 12 months of Mr. Agarwal's then-current base salary (or the base salary in effect immediately prior to the change in control, if higher) plus (B) 1.0 times Mr. Agarwal's target annual bonus for the then current year, without regard to whether the metrics have been established or achieved for such year, (ii) subject to his copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, up to 12 months of payment of the portion of the premium equal to the amount we would have paid to provide health insurance had he remained employed by us, and (iii) accelerated vesting of the then-outstanding and unvested portion of Mr. Agarwal's stock options and other stock-based awards that are subject solely to time-based vesting. The severance pay and benefits described in this paragraph are subject to Mr. Agarwal's delivery of and compliance with a fully effective release of claims.

The payments and benefits under the Agarwal Employment Agreement in connection with a change in control may not be eligible for federal income tax deduction by us pursuant to Section 280G of Code. These payments and benefits may also be subject to an excise tax under Section 4999 of the Code. If the payments or benefits payable to Mr. Agarwal in connection with a change in control would be subject to the excise tax imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to him.

Mr. Agarwal entered into an Employee Proprietary Information and Inventions Assignment Agreement that contains, among other provisions, nondisclosure of confidential information, invention assignment and nonsolicitation provisions.

Steven Colletti

On November 8, 2023, we entered into a new employment agreement with Steven Colletti, our Chief Scientific Officer, or the Colletti Employment Agreement. Under Dr. Colletti's new employment agreement, he has an initial annual base salary of $420,000, an initial target bonus opportunity equal to 35% of his annual base salary (with any incentive bonus for 2023 to be prorated based on Dr. Colletti's days of employment with our company during 2023) and continues to remain eligible for equity grants under our equity incentive plans, and continues to be eligible to participate in our employee benefit plans generally.

The Colletti Employment Agreement provides that in the event Dr. Colletti is terminated without "cause" or he resigns for "good reason" (as such terms are defined therein) outside of the change in control period (which extends from three months prior to a change in control to 12 months following a change in control, as "change in control" is defined therein), subject to signing and complying with a separation agreement and release, which shall include, without limitation, a release of claims, a reaffirmation of restrictive covenants, and in our sole discretion, a one year post-employment noncompetition agreement, then Dr. Colletti will be entitled to the following severance benefits: (i) nine months of his then-current base salary, plus (ii) a pro rata target bonus for the year of termination, without regard to whether the metrics have been established or achieved for such year, and (iii) subject to his copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, up to nine months of payment of the portion of the premium equal to the amount we would have paid to provide health insurance had he remained employed by us.

The Colletti Employment Agreement provides enhanced severance pay and benefits in the event Dr. Colletti's employment is terminated by us without cause or Dr. Colletti resigns for good reason, in each case, within the change in control period. Such enhanced severance pay and benefits consist of (i) a lump sum cash payment equal to the sum of (A) 12 months of Dr. Colletti's then-current base salary (or the base salary in effect immediately prior to the change in control, if higher) plus (B) 1.0 times Dr. Colletti's target annual bonus for the then-current year, without regard to whether the metrics have been established or achieved for such year, (ii) subject to his copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, up to 12 months of payment of the portion of the premium equal to the amount we would have paid to provide health insurance had he remained employed by us, and (iii) accelerated vesting of the then-outstanding and unvested portion of Dr. Colletti's stock options and other stock-based awards that are subject solely to time-based vesting. The severance pay and benefits described in this paragraph are subject to Dr. Colletti's delivery of and compliance with a fully effective release of claims.

The payments and benefits under the Colletti Employment Agreement in connection with a change in control may not be eligible for federal income tax deduction by us pursuant to Section 280G of the Code. These payments and benefits may also be subject to an excise tax under Section 4999 of the Code. If the payments or benefits payable to Dr. Colletti in connection with a change in control would be subject to the excise tax imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to him.

Dr. Colletti entered into an Employee Proprietary Information and Inventions Assignment Agreement that contains, among other provisions, nondisclosure of confidential information, invention assignment and nonsolicitation provisions.

Outstanding Equity Awards at 2023 Fiscal Year-End

The following table sets forth information concerning outstanding equity awards held by each of the named executive officers as of December 31, 2023. For Messrs. Aiyar, Agarwal and Colletti, each equity award granted prior to the Merger were under the terms of the Legacy Korro Plan and each equity award granted following the Merger were under the terms of the 2023 Plan. For Mr. Lucchino and Dr. Loose, each equity award granted prior to the Merger were under the terms of the 2019 Plan or the 2014 Plan and each equity award granted following the Merger were under the terms of the 2023 Plan.

	Option Awards				
Name	Vesting Commencement Date	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price ($)	Option Expiration Date
Ram Aiyar	11/2/2020 (1)	105,007	31,226	11.68	12/1/2030
	1/27/2022 (1)	17,614	19,154	22.75	1/26/2032
	1/1/2023 (1)	—	30,130	20.94	2/8/2033
	11/3/2023 (2)	3,265	153,495	14.98	11/2/2033
David L. Lucchino	— (3)	8,887	—	168.50	4/16/2029
	— (3)	1,927	—	168.50	4/16/2029
	— (3)	1,927	—	168.50	4/16/2029
	— (3)	14,287	—	107.00	10/1/2029
	— (3)	4,849	—	107.00	2/11/2030
	— (3)	3,999	—	107.00	1/14/2031
Vineet Agarwal	5/11/2021 (1)	26,385	14,484	11.68	5/29/2031
	1/27/2022 (1)	2,854	3,107	22.75	1/26/2032
	1/1/2023 (1)	—	2,590	20.94	1/23/2033
	11/3/2023 (2)	969	45,578	14.98	11/2/2033
Steven Colletti	2/21/2023 (1)	—	51,895	21.94	4/10/2033
	11/3/2023 (2)	718	33,760	14.98	11/2/2033
Christopher R. Loose	— (3)	7,211	—	168.50	2/3/2024 (4)
	— (3)	1,284	—	168.50	2/3/2024 (4)
	— (3)	1,284	—	168.50	2/3/2024 (4)
	— (3)	2,935	—	107.00	2/3/2024 (4)
	— (3)	1,149	—	107.00	2/3/2024 (4)
	— (3)	1,399	—	107.00	2/3/2024 (4)

1. 1/4 of the shares subject to the stock option vest on the first anniversary of the vesting commencement date, and 1/48 of the shares subject to the stock option vest each month thereafter, in each case, subject to the named executive officer's continuous service relationship with us through each applicable vesting date. The stock option is also subject to certain acceleration of vesting provisions as provided in the applicable named executive officer's offer letter or employment agreement, as applicable.
2. 1/48 of the shares subject to the stock option vest each month following the vesting commencement date, in each case, subject to the named executive officer's continuous service relationship with us through each applicable vesting date. The stock option is also subject to certain acceleration of vesting provisions as provided in the applicable named executive officer's offer letter or employment agreement, as applicable.
3. All of the shares subject to the stock option were vested as of December 31, 2023.
4. Dr. Loose's service relationship with Frequency terminated on November 3, 2023. The date reported represents the expiration of the post-termination exercise period of the stock option.

Non-Employee Director Compensation

On November 3, 2023, Frequency completed the Merger with Legacy Korro. At the effective time of the Merger, a majority of the Frequency directors resigned, with the exception of David Lucchino, and the remaining director vacancies were replaced by new directors designated by Legacy Korro. Accordingly, we have provided the compensation disclosure with respect to all directors of Frequency and Legacy Korro that served during 2023 and for those non-employee directors of Legacy Korro that were appointed to our Board in connection with the Merger.

We have designed and implemented our compensation program for our non-employee directors to attract, motivate and retain individuals who are committed to our values and goals and who have the expertise and experience that we need to achieve those goals.

2023 Director Compensation Table

The following table presents the total compensation for each person who served as a non-employee director on our board of directors during 2023. The non-employee directors included in the following table under the heading Current Non-Employee Directors were our non-employee directors as of December 31, 2023. Dr. Aiyar, our Chief Executive Officer and President, did not receive any additional compensation from us for services on our board of directors. Mr. Lucchino, the Chief Executive Officer and President of Frequency prior to the Merger, did not receive any additional compensation from Frequency for services on Frequency's board of directors prior to the Merger or from us for services on our board of directors following the Merger. The compensation received by Dr. Aiyar and Mr. Lucchino, respectively, as our named executive officers is set forth above in "— *2023 Summary Compensation Table*."

Name	Fees Paid or Earned in Cash ($)	Option Awards ($) (1)	All Other Compensation ($)	Total ($)
Current Non-Employee Directors				
Ali Behbahani (2)	8,445	163,589	-	172,034
Nessan Bermingham (3)	202,137	332,390	-	534,527
Jean-Francois Formela (4)	8,206	163,589	-	171,795
Rachel Meyers (5)	4,231	299,994	-	304,225
Timothy R. Pearson (6)	9,560	163,589	-	173,149
Former Non-Employee Directors				
Timothy J. Barberich (7)	65,285	-	-	65,285
Jordan Baumhardt (8)	-	-	-	-
Hannah Chang (9)	-	-	-	-
Cynthia L. Feldmann (10)	42,120	-	-	42,120
Michael Huang (11)	43,383	-	-	43,383
Omar Khwaja (12)	21,016	-	-	21,016
Robert S. Langer (13)	37,065	-	50,000 (14)	87,065
Joel S. Marcus (15)	17,535	-	-	17,535
Alex Silverstein (16)	-	-	-	-
Colin Walsh (17)	-	-	-	-

1. The amounts reported represent the aggregate grant date fair value of the stock option awards granted to our non-employee directors during fiscal year 2023, computed in accordance with FASB ASC Topic 718, rather than the amounts paid to or realized by the non-employee directors. Such grant date fair values do not take into account any estimated forfeitures. The assumptions used in calculating the grant date fair value of the stock option awards reported in this column are set forth in Note 11 of our financial statements included elsewhere in this Annual Report on Form 10-K. The amounts reported in this column reflect the accounting cost for these stock option awards and do not correspond to the actual economic value that may be received by our non-employee directors upon the exercise of the stock option awards or any sale of the underlying shares of our common stock.
2. As of December 31, 2023, Dr. Behbahani held options to purchase an aggregate of 16,000 shares of our common stock.
3. As of December 31, 2023, Dr. Bermingham held options to purchase an aggregate of 91,773 shares of our common stock.
4. As of December 31, 2023, Dr. Formela held options to purchase an aggregate of 16,000 shares of our common stock.
5. As of December 31, 2023, Dr. Meyers held options to purchase an aggregate of 12,551 shares of our common stock.
6. As of December 31, 2023, Mr. Pearson held options to purchase an aggregate of 16,000 shares of our common stock.
7. As of December 31, 2023, Mr. Barberich held options to purchase an aggregate of 2,335 shares of our common stock.
8. As of December 31, 2023, Mr. Baumhardt did not hold any options to purchase shares of our common stock.
9. As of December 31, 2023, Dr. Chang did not hold any outstanding equity awards.
10. As of December 31, 2023, Ms. Feldman held options to purchase an aggregate of 1,185 shares of our common stock.
11. As of December 31, 2023, Mr. Huang held options to purchase an aggregate of 888 shares of our common stock.
12. As of December 31, 2023, Dr. Khwaja held options to purchase an aggregate of 3,726 shares of our common stock.

13. As of December 31, 2023, Dr. Langer held options to purchase an aggregate of 7,737 shares of our Bio common stock.

14. Amounts represent consulting fees paid pursuant to a verbal consulting agreement with us prior to the termination of consulting services upon the closing of the Merger. The agreement entitled Dr. Langer to $5,000 in consulting fees per month.

15. As of December 31, 2023, Mr. Marcus held options to purchase an aggregate of 888 shares of our common stock.

16. As of December 31, 2023, Mr. Silverstein did not hold any outstanding equity awards.

17. As of December 31, 2023, Mr. Walsh did not hold any outstanding equity awards.

Prior to the Merger

Prior to the Merger, Legacy Korro did not have a formal policy to provide any cash or equity compensation to its non-employee directors for their service on the Legacy Korro board of directors or committees of its board of directors nor did any non-employee director receive any compensation for serving on Legacy Korro's board of directors, except for Dr. Bermingham who received an annual payment of $187,500 for services on Legacy Korro's board of directors prior to the Merger.

Prior to the Merger, Frequency maintained a compensation program for Frequency non-employee directors, or the Frequency Director Program under which each Frequency non-employee director received the following amounts for their services on our board of directors:

Annual Retainer for Board Membership		
$35,000 for general availability and participation in meetings and conference calls of our Board of Directors		
Additional Annual Retainer for Committee Membership		
Audit Committee Chairperson:	$	15,000
Audit Committee member (other than Chairperson):	$	7,500
Compensation Committee Chairperson:	$	10,000
Compensation Committee member (other than Chairperson):	$	5,000
Nominating and Corporate Governance Committee Chairperson:	$	8,000
Nominating and Corporate Governance Committee member (other than Chairperson):	$	4,000
Additional Retainer for Chairperson of the Board, Vice Chairperson of the Board, or Lead Independent Director:	$	30,000

Under the Frequency Director Plan, Frequency non-employee directors were granted an option to purchase 29,693 (prior to the Reverse Stock Split) shares of our common stock upon the director's initial election or appointment to our board of directors and, if the director had served on our board of directors for at least six months as of the date of an annual meeting of stockholders, an option to purchase 14,846 (prior to the Reverse Stock Split) shares of our common stock on the date of the annual meeting. The stock options granted to Frequency non-employee directors under the program had an exercise price equal to the fair market value of our common stock on the date of grant and expired not later than ten years after the date of grant. The stock options granted upon a director's initial election or appointment vested in 36 substantially equal monthly installments following the date of grant. The stock options granted annually to directors vested in a single installment on the earlier of the day before the following annual meeting or the first anniversary of the date of grant. In addition, all unvested stock options vested in full upon the occurrence of a change in control.

Director fees under the Frequency Director Program were payable in arrears in four equal quarterly installments not later than the fifteenth day following the final day of each calendar quarter, provided that the amount of each payment is prorated for any portion of a quarter that a director was not serving as a non-employee member of our board of directors.

Following the Merger

In connection with the Merger, we adopted a non-employee director compensation policy, or the Korro Director Policy. Under the Korro Director Policy, our non-employee directors are eligible to receive cash retainers (which are be payable quarterly in arrears and prorated for partial years of service) and equity awards as set forth below:

Annual Retainer for Board Membership		
$40,000 for general availability and participation in meetings and conference calls of our Board of Directors		
Additional Annual Retainer for Committee Membership		
Audit Committee Chairperson:	$	15,000
Audit Committee member (other than Chairperson):	$	7,500
Compensation Committee Chairperson:	$	10,000
Compensation Committee member (other than Chairperson):	$	5,000
Nominating and Corporate Governance Committee Chairperson:	$	8,000
Nominating and Corporate Governance Committee member (other than Chairperson):	$	4,000
Additional Retainer for Non-Executive Chairperson or Lead Director of the Board:	$	30,000

In addition, the Korro Director Policy provides that, upon initial election or appointment to the board, each new non-employee director will be granted a non-statutory stock option with a value of $300,000 (as determined in accordance with the policy and subject to a 16,000 share maximum), or the Director Initial Grant. The Director Initial Grant will vest in substantially equal annual installments over three years, subject to continued service as a non-employee director through the applicable vesting date. On the date of each annual meeting of our stockholders, each non-employee director who has been serving as a non-employee director for at least six months as of such date and will continue as a non-employee director following such meeting will be granted an annual award of a non-statutory stock option with a value of $150,000 (subject to a 8,000 share maximum), or the Director Annual Grant. The Director Annual Grant will vest in full on the earlier of the one-year anniversary of the grant date or on the date of our next annual meeting of stockholders, subject to continued service as a non-employee director through the applicable vesting date. The Director Initial Grant and Director Annual Grants are subject to full accelerated vesting upon the sale of our company. All of the foregoing stock options will be granted with a per share exercise price equal to the fair market value of a share of our common stock on the date of grant and will have a 10 year term.

The aggregate amount of compensation, including both equity compensation and cash compensation, paid to any of our non-employee directors for services as a director in a calendar year period will not exceed $1,000,000 in the first calendar year such individual becomes a non-employee director and $750,000 in any other calendar year.

We reimburse all reasonable out-of-pocket expenses incurred by directors for their attendance at meetings of the board or any committee thereof.

Employee directors receive no additional compensation for their service as a director.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth certain information regarding beneficial ownership of our common stock as of February 29, 2024.

Beneficial ownership is determined in accordance with the rules of the SEC and generally includes voting or investment power with respect to securities. Under those rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power with respect to the securities as well as any shares of common stock that the individual or entity has the right to acquire within 60 days of February 29, 2024 the exercise of stock options or other rights. These shares are deemed to be outstanding and beneficially owned by the person holding those options for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. Except as noted by footnote, and subject to community property laws where applicable, we believe, based on the information provided to them, that the persons and entities named in the table below have sole voting and investment power with respect to all common stock shown as beneficially owned by them.

The table lists applicable percentage ownership based on 8,020,703 shares of common stock outstanding as of February 29, 2024. The number of shares beneficially owned includes shares of common stock that each person has the right to acquire within 60 days, including upon the exercise of stock options and the vesting of RSUs. These stock options and RSUs shall be deemed to be outstanding for the purpose of computing the percentage of outstanding shares of our common stock expected to be owned by such person but shall not be deemed to be outstanding for the purpose of computing the percentage of outstanding shares of the combined organization’s common stock expected to be owned by any other person.

Name of Beneficial Owner	Beneficial Ownership	
	Number	Percent
5% or Greater Stockholders:		
Entities affiliated with Atlas Venture(1)	1,119,292	14.0%
Entities affiliated with New Enterprise Associates(2)	1,074,273	13.4%
FMR LLC(3)	727,205	9.1%
Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund(4)	546,325	6.8%
Entities affiliated with Point72 Asset Management(5)	542,657	6.8%
Platanus Investment LLC(6)	540,165	6.7%
Entities affiliated with Citadel(7)	529,251	6.6%
Entities affiliated with Cormorant Asset Management LP(8)	529,170	6.6%
Invus Public Equities, L.P.(9)	419,226	5.2%
Directors and Named Executive Officers:		
Vineet Agarwal(10)	38,794	*
Ram Aiyar(11)	167,394	2.0%
Ali Behbahani	—	*
Nessan Bermingham(12)	112,455	1.4%
Jean-François Formela	—	*
David L. Lucchino(13)	58,026	*
Rachel Meyers	—	*
Timothy R. Pearson.	—	*
Steve Colletti (14)	18,726	*
Christopher R. Loose	—	*
All directors and executive officers as a group (11 persons)(15)	419,816	5.0%

* Represents beneficial ownership of less than 1%.

(1) Consists of (i) 942,075 shares of our common stock held by Atlas Venture Fund XI, L.P., or AVF XI, and (ii) 177,217 shares of our common stock held by Atlas Venture Opportunity Fund II, L.P., or AVO II. Atlas Venture Associates XI, L.P. is the general partner of AVF XI and Atlas Venture Associates XI, LLC is the general partner of Atlas Venture Associates XI, L.P. The members of Atlas Venture Associates XI, LLC collectively make investment decisions on behalf of Atlas Venture Fund XI, LLC. Jean-Francois Formela is a member of Atlas Venture Associates XI, LLC and a member of Korro Bio's board of directors. Each of AVF XI, Atlas Venture Associates XI, L.P., and Atlas Venture Associates XI, LLC may be deemed to beneficially own the shares held by AVF XI. Dr. Formela expressly disclaim beneficial ownership of the shares owned by AVF XI, except to the extent of his pecuniary interest therein, if any. Atlas Venture Associates Opportunity II, L.P. is the general partner of AVO II, and Atlas Venture Associates Opportunity II, LLC is the general partner of Atlas Venture Associates Opportunity II, L.P. The members of Atlas Venture Associates Opportunity II, LLC collectively make investment decisions on behalf of Atlas Venture Associates Opportunity II, LLC. Dr. Formela is a member of Atlas Venture Associates Opportunity II, LLC. Each of AVO II, Atlas Venture Associates Opportunity II, L.P., and Atlas Venture Associates Opportunity II, LLC may be deemed to beneficially own the shares held by AVO II. Dr. Formela expressly disclaim beneficial ownership of the shares owned by AVO II, except to the extent of his pecuniary interest therein, if any. The mailing address of Atlas is 300 Technology Square, 8th Floor, Cambridge, MA 02139.

(2) Consists of 1,072,936 shares of our common stock held by New Enterprise Associates 17, L.P., or NEA 17, and 1,337 shares of our common stock held by NEA Ventures 2019, L.P. The general partner of NEA 17 is NEA Partners 17, L.P., or NEA Partners 17, and the general partner of NEA Partners 17 is NEA 17 GP, LLC, or NEA 17 LLC. The managers of NEA 17 LLC are Forest Baskett, Ali Behbahani, M.D., Carmen Chang, Anthony A. Florence, Jr., Mohamad H. Makhzoumi, Edward T. Mathers, Scott D. Sandell, Paul Walker and Rick Yang. Dr. Behbahani is a member of NEA 17 LLC and a member of Korro Bio's board of directors. Each of NEA Partners 17 and NEA 17 LLC may be deemed to beneficially own the shares held by NEA 17. The general partner of NEA Ventures 2019, L.P., or Ven 2019, is Karen Welsh. The address of the principal business office of NEA 17, NEA Partners 17, NEA 17 LLC, Ven 2019, and Mr. Sandell is New Enterprise Associates, 1954 Greenspring Drive, Suite 600, Timonium, MD 21093. The address of the principal business office of Dr. Behbahani, Mr. Baskett, Ms. Chang, Mr. Makhzoumi, Mr. Walker and Mr. Yang is New Enterprise Associates, 2855 Sand Hill Road, Menlo Park, California 94025. The address of the principal business office of Mr. Florence and Mr. Mathers is New Enterprise Associates, 104 5th Avenue, 19th Floor, New York, NY 10001.

(3) Consists of (i) 180,884 shares of our common stock held by Fidelity Select Portfolios: Biotechnology Portfolio, (ii) 40,253 shares of our common stock held by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund, (iii) 197,128 shares of our common stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund, (iv) 250,912 shares of our common stock held by Fidelity Growth Company Commingled Pool and (v) 58,028 shares of our common stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company K6 Fund. All of the foregoing securities are beneficially owned, or may be deemed to be beneficially owned, by FMR LLC, certain of its subsidiaries and affiliates, and other companies. Abigail P. Johnson is a Director, the Chairman and the Chief Executive Officer of FMR LLC. Members of the Johnson family, including Abigail P. Johnson, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. The address of FMR LLC is 245 Summer Street, Boston, MA 02210.

(4) Consists of 546,325 shares of our common stock held by Mutual Fund Series Trust, on behalf of Eventide. Eventide Asset Management, LLC is the investment advisor to Eventide. Robin C. John is the Chief Executive Officer of Eventide Asset Management, LLC and Finny Kuruvilla, M.D., Ph.D. is a Co-Chief Investment Officer and Managing Director of Eventide Asset Management, LLC. In their corporate capacity for Eventide Asset Management, LLC, Mr. John and Dr. Kuruvilla hold voting and/or dispositive power over the shares held by Eventide. Mr. John and Dr. Kuruvilla disclaim beneficial ownership of such shares except to the extent of their pecuniary interest therein. The address for Eventide, Mr. John and Dr. Kuruvilla is U.S. Bank Trust Services, Physical Processing, MK-WI S302, 1555 N RiverCenter Drive, Suite 302, Milwaukee, WI 53212.

(5) Consists of 276,831 shares of our common stock held by Point72 Biotech Private Investments, LLC, or Point72 Biotech, and 265,826 shares of our common stock held by Point72 Associates, LLC, or Point72 Associates. Differentiated Ventures Investments, LLC, or Differentiated Ventures, is the managing member of Point72 Biotech and may be deemed to share beneficial ownership of the shares held by Point72 Biotech. 72 Investment Holdings, LLC, or 72 Investment Holdings, is the sole member of Differentiated Ventures and may be deemed to share beneficial ownership of the shares of which Differentiated Ventures may be deemed to share beneficial ownership. Steven A. Cohen, or Mr. Cohen, is the sole member of 72 Investment Holdings and may be deemed to share beneficial ownership of the shares of which 72 Investment Holdings may be deemed to share beneficial ownership. Each of Differentiated Ventures, 72 Investment Holdings and Mr. Cohen disclaims beneficial ownership of the shares held by Point72 Biotech. Pursuant to an investment management agreement, Point72 Asset Management, L.P., or Point72 Asset Management, maintains investment and voting power with respect to the shares held by Point72 Associates and therefore may be deemed to share beneficial ownership of such shares. Point72 Capital Advisors, Inc., or Point72 Capital Advisors, is the general partner of Point72 Asset Management and may be deemed to share beneficial ownership of the shares of which Point72 Asset Management may be deemed to share beneficial ownership. Mr. Cohen is the sole member of Point72 Capital Advisors and may be deemed to share beneficial ownership of the shares of which Point72 Capital Advisors may be deemed to share beneficial ownership. Each of Point72 Asset Management, Point72 Capital Advisors and Mr. Cohen disclaims beneficial ownership of the shares held by Point72 Associates. The address for Point72 Biotech and Point72 Associates is c/o Point72, L.P., 72 Cummings Point Road, Stamford, CT 06902.

(6) Consists of 540,165 shares of our common stock held by Platanus Investment LLC, or Platanus. Xinyi Cai is the director of Platanus and holds voting and dispositive power over the securities owned by Platanus. The address for Platanus is 3 E 3rd Ave, Suite 200, San Mateo, CA 94401.

(7) Based solely on Schedule 13G jointly filed by Citadel Advisors LLC, or Citadel Advisors, Citadel Advisors Holdings LP, or CAH, Citadel GP LLC, or CGP, Citadel Securities LLC, or Citadel Securities, Citadel Securities Group LP, or CALC4, Citadel Securities GP LLC, or CSGP, and Mr. Kenneth Griffin, or collectively with Citadel Advisors, CAH, CGP, Citadel Securities, CALC4 and CSGP, the Citadel Reporting Persons, with respect to shares owned by Citadel Multi-Strategy Equities Master Fund Ltd., a Cayman Islands company, or CM, Citadel CEMF Investments Ltd., a Cayman Islands limited company, or CCIL, Citadel Securities and CRBU Holdings LLC, a Delaware limited liability company, or CRBH. Citadel Advisors is the portfolio manager for CM and CCIL. CAH is the sole member of Citadel Advisors. CGP is the general partner of CAH. CALC4 is the non-member manager of Citadel Securities and CRBH. CSGP is the general partner of CALC4. Mr. Griffin is the President and Chief Executive Officer of CGP, and owns a controlling interest in CGP and CSGP. The address of each of the Citadel Reporting Persons is Southeast Financial Center, 200 S. Biscayne Blvd., Suite 3300, Miami, FL 33131.

(8) Consists of (i) 312,052 shares of our common stock held by Cormorant Global Healthcare Master Fund, LP, or Master Fund, and (ii) 217,118 shares of our common stock held by Cormorant Private Healthcare Fund II, LP, or Fund II. Cormorant Global Healthcare GP, LLC, or Global GP, and Cormorant Private Healthcare GP II, LLC, or Private GP II, serve as the general partner of Master Fund and Fund II, respectively. Cormorant Asset Management, LP serves as the investment manager to Master Fund and Fund II. Bihua Chen serves as the managing member of Global GP, Private GP II and Cormorant Asset Management, LP. Each of Global GP, Private GP II, Cormorant Asset Management, LP and Ms.

Chen disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. The principal address for the Cormorant Asset Management LP entities is 200 Clarendon Street 52nd Floor, Boston, Massachusetts 02116.

(9) Consists of 419,226 shares of our common stock held by Invus Public Equities L.P., or Invus. Invus Public Equities Advisors, LLC, or Invus PE Advisors, controls Invus, as its general partner and accordingly, may be deemed to beneficially own the shares held by Invus. The Geneva branch of Artal International S.C.A., or Artal International, controls Invus PE Advisors, as its managing member and accordingly, may be deemed to beneficially own the shares held by Invus. Artal International Management S.A., or Artal International Management, as the managing partner of Artal International, controls Artal International and accordingly, may be deemed to beneficially own the shares that Artal International may be deemed to beneficially own. Artal Group S.A., or Artal Group, as the sole stockholder of Artal International Management, controls Artal International Management and accordingly, may be deemed to beneficially own the shares that Artal International Management may be deemed to beneficially own. Westend S.A., or Westend, as the parent company of Artal Group, controls Artal Group and accordingly, may be deemed to beneficially own the shares that Artal Group may be deemed to beneficially own. Stichting Administratiekantoor Westend, or the Stichting, as majority shareholder of Westend, controls Westend and accordingly, may be deemed to beneficially own the shares that Westend may be deemed to beneficially own. Mr. Amaury Wittouck, as the sole member of the board of the Stichting, controls the Stichting and accordingly, may be deemed to beneficially own the shares that the Stichting may be deemed to beneficially own. The address for Invus and Invus PE Advisors is 750 Lexington Avenue, 30th Floor, New York, NY 10022. The address for Artal International, Artal International Management, Artal Group, Westend and Mr. Wittouck is Valley Park, 44, Rue de la Vallée, L-2661, Luxembourg. The address for the Stichting is Claude Debussylaan, 46, 1082 MD Amsterdam, The Netherlands. The address for Invus is 750 Lexington Avenue, New York, NY 10022.

(10) Consists of 38,794 shares of our common stock underlying options that are exercisable or will become exercisable within 60 days of February 29, 2024.

(11) Consists of (i) 4,613 shares of our common stock held by The Ram Aiyar Irrevocable Trust, or the Trust, and (ii) 162,781 shares of our common stock underlying options that are exercisable or will become exercisable within 60 days of February 29, 2024. The address of the Trust is c/o Steven M. Burke, P.O. Box 326, Manchester, NH 03105.

(12) Consists of (i) 35,114 shares of our common stock and (ii) 77,341 shares of our common stock underlying options that are exercisable or will become exercisable within 60 days of February 29, 2024.

(13) Consists of (i) 22,150 shares of our common stock and (ii) 35,876 shares of our common stock underlying options that are exercisable or will become exercisable within 60 days of February 29, 2024.

(14) Consists of 18,726 shares of our common stock underlying options that are exercisable or will become exercisable within 60 days of February 29, 2024.

(15) Consists of (i) 61,877 shares of our common stock and (ii) 357,939 shares of our common stock underlying options that are exercisable or will become exercisable within 60 days of February 29, 2024.

Securities Authorized for Issuance Under Equity Compensation Plans

The following table provides information as of December 31, 2023 with respect to the shares of our common stock that may be issued under our existing equity compensation plans.

Plan Category	Number of Securities to be Issued Upon Exercise of Outstanding Options, Warrants and Rights	Weighted-Average Exercise Price of Outstanding Options, Warrants and Rights	Number of Securities Remaining Available for Future Issuance Under Equity Compensation Plans (Excluding Securities in First Column)
Equity compensation plans approved by stockholders [1]	1,328,229[2]	$16.48[3]	344,169[4]
Equity compensation plans not approved by stockholders	—	—	—
Total	1,328,229	$ 16.48	344,169

(1) Includes the following plans: the 2023 Plan, the 2019 Plan, the Legacy Korro Plan, the 2014 Plan and the 2023 ESPP.

(2) Includes 1,328,229 shares issuable upon the exercise of outstanding options, of which 600,977 were assumed in connection with the Merger.

(3) The weighted average exercise price is calculated based solely on outstanding stock options.

(4) As of December 31, 2023, a total of 255,694 shares were reserved for issuance pursuant to the 2023 Plan and a total of 88,502 shares were reserved for issuance pursuant to the 2023 ESPP. Following the Merger, we did not grant any awards under the 2019 Plan, the 2014 Plan, or the Legacy Korro Plan, but all outstanding awards under such plans continue to be governed by their existing terms. The 2023 Plan has an evergreen provision that allows for an annual increase in the number of shares available for issuance under the 2023 Plan to be added on the first day of January,

starting with January 1, 2024, in an amount equal to the lesser of (i) 5% of the outstanding number of shares of our common stock on the immediately preceding December 31 or (ii) such number of shares as determined by the administrator of the 2023 Plan in each case subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization. The shares of common stock underlying any awards granted under the Legacy Korro Plan, the 2019 Plan or the 2014 Plan that are forfeited, canceled, reacquired by us prior to vesting, satisfied without the issuance of stock, or otherwise terminated (other than by exercise) and the shares of common stock that are withheld upon exercise of a stock option or settlement of such award to cover the exercise price or tax withholding will be added to the shares of common stock available for issuance under the 2023 Plan. The 2023 ESPP has an evergreen provision that allows for an annual increase in the number of shares available for issuance under the 2023 ESPP to be added on the first day of each January, starting with January 1, 2024, by the lesser of (i) 88,502 shares of our common stock, (ii) 1% of the outstanding number of shares of common stock on the immediately preceding December 31, or (iii) such number of shares of common stock as determined by the administrator of the 2023 ESPP. The number of shares reserved under the 2023 ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization. The number in the table does not include the increases from January 1, 2024.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

Series B Convertible Preferred Stock Financing

In November 2021, December 2021 and March 2023, Legacy Korro sold an aggregate of 43,085,531 shares of its Series B preferred stock at a purchase price of $2.61 and $2.78 per share for aggregate gross proceeds of $116.0 million. The following table summarizes purchases of Legacy Korro's Series B preferred stock by related persons (share amounts have not been updated to reflect the exchange for our common stock in the Merger):

Participant	Shares of Legacy Korro Series B Preferred Stock	Total Purchase Price ($)
Atlas Venture Fund XI, L.P. (1)	3,064,273	8,250,001
New Enterprise Associates 17, L.P. (2)	2,971,416	8,000,000
Platanus Investment LLC (3)	1,764,279	4,750,002
Qiming U.S. Healthcare Fund II, L.P. (4)	1,114,281	3,000,000
Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund (5)	7,428,540	20,000,000
Invus Public Equities, L.P. (6)	5,571,405	15,000,000
Point72 Biotech Private Investments, LLC (7)	5,571,405	15,000,000
The Ram Aiyar Irrevocable Trust (8)	92,857	250,001
FMR LLC (9)	9,285,675	25,000,000
Citadel Multi-Strategy Equities Master Fund Ltd. (10)	835,710	2,249,998
Entities affiliated with Cormorant Asset Management LP (11)	835,710	2,249,998

(1) Atlas beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock. Jean-François Formela is a Partner at Atlas and a member of our board of directors.
(2) NEA beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock. Ali Behbahani is a Partner at NEA and a member of our board of directors.
(3) Platanus beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock. Hannah Chang is a Partner at Platanus and was a member of Legacy Korro's board of directors.
(4) Qiming beneficially owned more than 5% of Legacy Korro's outstanding capital stock and had a designee on Legacy Korro's board of directors.
(5) Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund, or Eventide, beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(6) Invus Public Equities, L.P. beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(7) Point72 Biotech Private Investments, LLC beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock and had a designee on Legacy Korro's board of directors.
(8) Ram Aiyar is the grantor of The Ram Aiyar Irrevocable Trust, is our chief executive officer and member of our board of directors and was chief executive officer of Legacy Korro and a member of its board of directors.
(9) FMR LLC beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.

(10) Citadel Multi-Strategy Equities Master Fund Ltd. beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(11) Entities affiliated with Cormorant Asset Management LP beneficially owned more than 5% of Legacy Korro's capital stock and beneficially own more than 5% of our outstanding capital stock.

Pre-Closing Financing

Legacy Korro entered into a Subscription Agreement in July 2023 with certain investors to consummate the Pre-Closing Financing. Pursuant to the Subscription Agreement, the investors agreed to purchase shares of Legacy Korro common stock, at a price of $2.78 per share, for aggregate gross proceeds of $117.3 million. Seven of the investors or their affiliates were beneficial holders of more than 5% of Legacy Korro's capital stock, and the table below sets forth the number of shares of Legacy Korro common stock purchased by such holders at the closing of the Pre-Closing Financing (share amounts have not been updated to reflect the exchange for our common stock in the Merger):

Participant	Shares of Legacy Korro Common Stock	Total Purchase Price ($)
Atlas Venture Fund XI, L.P. (1)	177,217	9,999,999
New Enterprise Associates 17, L.P. (2)	177,217	9,999,999
FMR LLC (3)	265,826	14,999,999
Citadel CEMF Investments Ltd.(4)	265,826	14,999,999
Platanus Investment LLC (5)	8,860	500,000
Qiming U.S. Healthcare Fund II, L.P. (6)	35,443	1,999,999
Mutual Fund Series Trust, on behalf of Eventide Healthcare & Life Sciences Fund (7)	177,217	9,999,999
Invus Public Equities, L.P (8)	141,774	8,000,000
Point72 Associates, LLC (9)	265,826	14,999,999
Entities affiliated with Cormorant Asset Management LP (10)	265,826	14,999,999

(1) Atlas beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock. Jean-François Formela is a Partner at Atlas and a member of our board of directors.
(2) NEA beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock. Ali Behbahani is a Partner at NEA and a member of our board of directors.
(3) FMR LLC beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(4) Citadel beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(5) Platanus beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock. Hannah Chang is a Partner at Platanus and was a member of Legacy Korro’s board of directors.
(6) Qiming beneficially owned more than 5% of Legacy Korro's outstanding capital stock and had a designee on Legacy Korro’s board of directors.
(7) Eventide beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(8) Invus Public Equities, L.P. beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of our outstanding capital stock.
(9) Point72 Biotech Private Investments, LLC, an affiliate of Point72 Associates, LLC, beneficially owned more than 5% of Legacy Korro's capital stock and beneficially owns more than 5% of Legacy Korro’s outstanding capital stock and had a designee on Legacy Korro’s board of directors.
(10) Entities affiliated with Cormorant Asset Management LP beneficially owned more than 5% of Legacy Korro's capital stock and beneficially own more than 5% of our outstanding capital stock.

Other Agreements with Our Stockholders

In connection with Legacy Korro’s Series B convertible preferred stock financing, Legacy Korro entered into investors’ rights, voting and right of first refusal and co-sale agreements containing registration rights, information rights, voting rights and rights of first refusal, among other things, with certain holders of Legacy Korro preferred stock and certain holders of Legacy Korro common stock. These stockholder agreements terminated upon the closing of the Merger, except for the registration rights granted under Legacy Korro’s investors’ rights agreement.

Indemnification Agreements

We have entered into agreements to indemnify our directors and executive officers. These agreements will, among other things, require us to indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on our behalf or that person's status as a member of our board of directors to the maximum extent allowed under Delaware law.

Registration Rights under Frequency Investors' Rights Agreement

Frequency entered into a second amended and restated investors' rights agreement, or the Investors' Rights Agreement, in July 2019 with each holder of Frequency preferred stock, which included certain holders of more than 5% of Frequency common stock at the time and certain of Frequency's directors and executive officers. The Investors' Rights Agreement grants the parties thereto certain registration rights in respect of the "registrable securities" held by them, which securities include (1) the shares of Frequency common stock issuable or issued upon the conversion of shares of Frequency convertible preferred stock, (2) any shares of Frequency common stock, or any common stock issued or issuable upon conversion and/or exercise of any of Frequency's securities acquired by the parties after the date of the Investors' Rights Agreement, and (3) any shares of Frequency common stock issued as a dividend or other distribution with respect to, or in exchange for, or in replacement of, the shares described in the foregoing clauses (1) and (2). The registration of shares of Frequency common stock pursuant to the exercise of these registration rights would enable the holders thereof to sell such shares under the Securities Act when the applicable registration statement is declared effective. Under the Investors' Rights Agreement, Frequency will pay all expenses relating to such registrations, including the reasonable fees of one special counsel for the participating stockholders, and the stockholders will pay all underwriting discounts and commissions relating to the sale of their shares. The Investors' Rights Agreement also includes customary indemnification and procedural terms.

Form S-1 registration rights

If at any time the holders of at least 40% of the registrable securities request in writing that Frequency effects a registration with respect to at least 25% of such registrable securities then outstanding (or a lesser percent if the anticipated aggregate offering price, net of selling expenses, would exceed $10.0 million), Frequency is obligated to register their shares. Frequency is obligated to effect at most two registrations in response to these demand registration rights. If the holders requesting registration intend to distribute their shares by means of an underwriting, the managing underwriter of such offering will have the right to limit the numbers of shares to be underwritten for reasons related to the marketing of the shares.

Piggyback registration rights

If at any time Frequency proposes to register any shares of Frequency common stock under the Securities Act, subject to certain exceptions, the holders of registrable securities will be entitled to notice of the registration and to include their registrable securities in the registration. If Frequency's proposed registration involves an underwriting, the managing underwriter of such offering will have the right to limit the number of shares to be underwritten for reasons related to the marketing of the shares.

Form S-3 registration rights

If, at any time after Frequency becomes entitled under the Securities Act to register Frequency's shares on a registration statement on Form S-3, the holders of the registrable securities request in writing that Frequency effects a registration with respect to registrable securities at an aggregate price to the public in the offering of at least $1,000,000, net of expenses borne by the holders, Frequency is obligated to effect such registration. Frequency is not obligated to effect more than one S-3 registration in any 12 month period.

Expenses and indemnification

Ordinarily, other than underwriting discounts and commissions, Frequency will be required to pay all expenses incurred by Frequency related to any registration effected pursuant to the exercise of these registration rights. These expenses may include all registration, filing and qualification fees, printing and accounting fees, fees and disbursements of Frequency's counsel, and reasonable fees and disbursements of a counsel for the selling securityholders. Additionally, Frequency has agreed to indemnify selling stockholders for damages, and any legal or other expenses reasonably incurred, arising from or based upon any untrue statement or alleged untrue statement of a material fact contained in any registration statement, an omission or alleged omission to state a material fact required to be stated in any registration statement or necessary to make the statements therein not misleading, or any violation or alleged violation by the indemnifying party of securities laws, subject to certain exceptions.

Termination of registration rights

The registration rights expire on the earlier of (1) the date that is five years after the closing of Frequency's IPO and (2) with respect to each stockholder, at such time as such stockholder can sell all of its shares pursuant to Rule 144 of the Securities Act or another similar exemption under the Securities Act during any three month period without registration.

Registration Rights under Legacy Korro Investors' Rights Agreement

Legacy Korro entered into a third amended and restated investors' rights agreement, or the Legacy Korro Investors' Rights Agreement, in November 2021 with each holder of Legacy Korro's preferred stock. The Legacy Korro Investors' Rights Agreement grants such holders certain registration rights in respect of the "registrable securities" held by them, which securities include (1) the shares of Legacy Korro's common stock issuable or issued upon the conversion of shares of Legacy Korro's preferred stock, (2) any shares of Legacy Korro's common stock, or any common stock issued or issuable upon conversion and/or exercise of any of Legacy Korro's securities acquired by such holders after the date of the Legacy Korro Investors' Rights Agreement, and (3) any shares of Legacy Korro common stock issued as (or issuable upon the conversion or exercise of any warrant, right, or other security that is issued as) a dividend or other distribution with respect to, or in exchange for, or in replacement of, the shares described in the foregoing clauses (1) and (2). The registration of shares of Legacy Korro's common stock pursuant to the exercise of these registration rights would enable the holders thereof to sell such shares under the Securities Act when the applicable registration statement is declared effective. Under the Legacy Korro Investors' Rights Agreement, Legacy Korro agreed to pay all expenses relating to such registrations, including the reasonable fees of one special counsel for the selling holders, and the selling holders agreed to pay all underwriting discounts, selling commissions and stock transfer taxes applicable to the sale of the registrable securities, and fees and disbursement of counsel for the selling holders (except as agreed to be paid by Legacy Korro). The Legacy Korro Investors' Rights Agreement also includes customary indemnification and procedural terms.

Form S-1 registration rights

If at any time the holders of a majority of the registrable securities request in writing that Legacy Korro effects a registration with respect to at least 50% of such registrable securities then outstanding (or a lesser percent if the anticipated aggregate offering price, net of selling expenses, would exceed $15.0 million), Legacy Korro is obligated to register their shares. Legacy Korro is obligated to effect at most two registrations in response to these demand registration rights. If the holders requesting registration intend to distribute their shares by means of an underwriting, the managing underwriter of such offering will have the right to limit the numbers of shares to be underwritten for reasons related to the marketing of the shares.

Piggyback registration rights

If at any time Legacy Korro proposes to register any shares of Legacy Korro's common stock under the Securities Act, subject to certain exceptions, the holders of registrable securities will be entitled to notice of the registration and to include their registrable securities in the registration. If Legacy Korro's proposed registration involves an underwriting, the managing underwriter of such offering will have the right to limit the number of shares to be underwritten for reasons related to the marketing of the shares.

Form S-3 registration rights

If, at any time after Legacy Korro becomes entitled under the Securities Act to register Legacy Korro's shares on a registration statement on Form S-3, the holders of at least 10% of the registrable securities then outstanding request in writing that Legacy Korro effects a registration with respect to registrable securities at an aggregate price, net of selling expenses, to the public in the offering of at least $5,000,000, Legacy Korro is obligated to effect such a registration. Legacy Korro is not obligated to effect more than two S-3 registrations in any 12 month period.

Termination of registration rights

The registration rights expire on the earlier of (1) the date that is five years after the closing of Legacy Korro's IPO, (2) with respect to each stockholder, at such time as such stockholder can sell all of its shares pursuant to Rule 144 of the Securities Act or another similar exemption under the Securities Act during any three month period without limitation during a three-month period, and (3) the closing of a deemed liquidation event.

Pre-Closing Financing Registration Rights

In connection with the Pre-Closing Financing, we entered into a registration rights agreement, or the Registration Rights Agreement, in July 2023 with Legacy Korro and each purchaser in the Pre-Closing Financing. Pursuant to the Registration Rights Agreement, we agreed to register the "registrable securities" held by the purchasers on a registration statement, or registration statements, if necessary, to permit resale of such securities on a continuous basis pursuant to Rule 415. The "registrable securities" include (a) all shares of our common stock issued to the purchasers at the closing of the Merger in respect of the shares of Legacy Korro's common stock purchased by the purchasers in the Pre-Closing Financing, (b) all shares of our common stock issued at the closing of the Merger to the purchasers in respect of all other shares of Legacy Korro's capital stock held by

purchaser as of immediately prior to the closing of the Merger, and (c) any securities issued or then issuable upon any stock split, dividend or other distribution, recapitalization or similar event with respect to the foregoing. Under the Registration Rights Agreement, we will pay all fees and expenses incident to the performance of our obligations, including the reasonable fees of one special counsel for Citadel CEMF Investments Ltd, excluding any underwriting, broker or similar fees or commissions, legal fees and other costs (except as agreed to be paid by us) of any purchaser.

Policies for Approval of Related Party Transactions

Our board of directors reviews and approves transactions with our directors, officers and holders of 5% or more of our voting securities and their affiliates, each a related party. Prior to this offering, the material facts as to the related party's relationship or interest in the transaction are disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party's relationship or interest in the transaction are disclosed to our stockholders, who must approve the transaction in good faith.

Item 14. Principal Accountant Fees and Services.

We incurred the following fees from Ernst & Young LLP for the audit of the consolidated financial statements and for other services related to the years ended December 31, 2023 and December 31, 2022. The following table summarizes the fees for Ernst & Young LLP services to the Company for the last two fiscal years.

Fee Category	Fiscal Year 2023 ($)	Fiscal Year 2022 ($)
Audit Fees (1)	1,341,206	191,007
Audit-Related Fees	-	-
Tax Fees (2)	14,060	13,390
All Other Fees (3)	3,600	3,500
Total Fees	1,358,866	207,897

(1) "Audit fees" consist of fees for the audit of our annual financial statements, review of the interim financial statements included in our quarterly reports on Form 10-Q, registration statements on Form S-4, registration statements on Form S-3, registration statements on Form S-1 and other professional services provided in connection with financings and other regulatory filings.
(2) "Tax fees" consist of fees for professional services, including tax compliance, tax advice and tax planning.
(3) "All other fees" consist of fees paid to access Ernst & Young LLP publications and on-line subscriptions/content.

Audit Committee Pre-approval Policy and Procedures

Our audit committee has adopted policies and procedures relating to the approval of all audit and non-audit services that are to be performed by our independent registered public accounting firm. This policy provides that we will not engage our independent registered public accounting firm to render audit or non-audit services unless the service is specifically approved in advance by our audit committee or the engagement is entered into pursuant to the pre-approval procedure described below.

From time to time, our audit committee may pre-approve specified types of services that are expected to be provided to us by our independent registered public accounting firm during the next 12 months. Any such pre-approval details the particular service or type of services to be provided and is also generally subject to a maximum dollar amount.

PART IV

Item 15. Exhibit and Financial Statement Schedules.

(1) For a list of the financial statements included herein, see the Index to the Consolidated Financial Statements in Item 8 on page 109 of this Annual Report on Form 10-K, incorporated into this Item by reference.

(2) Financial statement schedules have been omitted because they are either not required or not applicable or the information is included in the consolidated financial statements or the notes thereto.

(3) Exhibits:

Exhibit Number	Description
2.1+	Agreement and Plan of Merger and Reorganization, dated as of July 14, 2023, by and among the registrant, Frequency Merger Sub, Inc. and the entity formerly known as Korro Bio, Inc. (incorporated by reference to Exhibit 2.1 to the registrant's Current Report on Form 8-K filed on July 14, 2023).
3.1	Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the registrant's Current Report on Form 8-K filed on October 7, 2019).
3.2	Certificate of Amendment to Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the registrant's Current Report on Form 8-K filed on November 6, 2023).
3.3	Certificate of Amendment to Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.2 to the registrant's Current Report on Form 8-K filed on November 6, 2023).
3.4	Amended and Restated Bylaws (incorporated by reference to Exhibit 3.1 to the registrant's Current Report on Form 8-K filed on September 23, 2020).
4.1	Description of Securities
10.1#	Korro Bio, Inc. 2023 Stock Option and Incentive Plan, and form of award agreements thereunder (incorporated by reference to Exhibit 10.1 to the registrant's Registration Statement on Form S-1/A filed December 20, 2023).
10.2#	Korro Bio, Inc. 2023 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.2 to the registrant's Registration Statement on Form S-1/A filed December 20, 2023).
10.3#	Korro Bio, Inc. 2019 Stock Incentive Plan, and form of award agreements thereunder (incorporated by reference to Exhibit 10.8 to the registrant's Current Report on Form 8-K filed on November 6, 2023).
10.4#	Korro Bio, Inc. Non-Employee Director Compensation Policy (incorporated by reference to Exhibit 10.11 to the Form 8-K, filed November 6, 2023).
10.5#	Frequency Therapeutics, Inc. 2014 Stock Incentive Plan, as amended, and form of option agreements thereunder (incorporated by reference to Exhibit 10.1 to the registrant's Registration Statement on Form S-1 (333-233652) filed on September 6, 2019).
10.6#	Frequency Therapeutics, Inc. 2019 Incentive Award Plan and form of award agreements thereunder (incorporated by reference to Exhibit 10.2 to the registrant's Registration Statement on Form S-1/A (333-233652) filed on September 23, 2019).
10.7#	Frequency Therapeutics, Inc. 2019 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.13 to the registrant's Registration Statement on Form S-1/A (333-233652) filed on September 23, 2019).
10.8#	Korro Bio, Inc. Senior Executive Cash Incentive Bonus Plan (incorporated by reference to Exhibit 10.12 to the Form 8-K, filed November 6, 2023).
10.9#	Employment Agreement, dated as of November 10, 2023, by and between Korro Bio, Inc. and Ram Aiyar, Ph.D. (incorporated by reference to Exhibit 10.1 to the Form 8-K filed November 14, 2023).
10.10#	Employment Agreement, dated November 8, 2023, by and between Korro Bio, Inc. and Vineet Agarwal (incorporated by reference to Exhibit 10.2 to the Form 8-K filed November 14, 2023).
10.11#	Employment Agreement, dated November 8, 2023, by and between Korro Bio, Inc. and Shelby Walker (incorporated by reference to Exhibit 10.11 to the registrant's Registration Statement on Form S-1/A filed December 20, 2023).
10.12#	Employment Agreement, dated November 8, 2023, by and between Korro Bio, Inc. and Steve Colletti (incorporated by reference to Exhibit 10.12 to the registrant's Registration Statement on Form S-1/A filed December 20, 2023).
10.13#	Employment Agreement, dated November 9, 2023, by and between Korro Bio, Inc. and Todd Chappell (incorporated by reference to Exhibit 10.13 to the registrant's Registration Statement on Form S-1/A filed December 20, 2023).
10.14#	Form of Indemnification Agreement for Officers of Korro Bio, Inc. (incorporated by reference to Exhibit 10.6 to the Form 8-K filed November 6, 2023).
10.15#	Form of Indemnification Agreement for Directors of Korro Bio, Inc. (incorporated by reference to Exhibit 10.7 to the Form 8-K filed November 6, 2023).

10.16	Third Amended and Restated Investors' Rights Agreement of Korro Bio, Inc., dated November 8, 2021 (incorporated by reference to Exhibit 10.27 to the Form S-4/A filed September 28, 2023).
10.17	Form of Lock-Up Agreement (incorporated by reference to Exhibit 10.3 to the Form 8-K filed November 6, 2023).
10.18	Contingent Value Rights Agreement dated November 3, 2023 (incorporated by reference to Exhibit 10.4 to the Form 8-K filed November 6, 2023).
10.19	Lease Agreement, by and between Korro Bio, Inc. and ARE-MA Region No. 59, LLC, dated August 10, 2020, as amended on March 2, 2021 (incorporated by reference to Exhibit 10.21 to the Form S-4 filed September 28, 2023).
10.20	Second Amendment to Lease Agreement, by and between Korro Bio, Inc. and ARE-MA Region No. 59, LLC, dated August 31, 2022.
10.21	Third Amendment to Lease Agreement, by and between Korro Bio, Inc. and ARE-MA Region No. 59, LLC, dated October 20, 2023.
10.22	Indenture of Lease, effective as of January 7, 2020 between HCP/KING 75 Hayden LLC and Frequency Therapeutics (incorporated by reference to Exhibit 10.13 to the registrant's Annual Report on Form 10-K filed on March 26, 2020).
10.23	Sublease Agreement, dated July 8, 2022, by and between Frequency Therapeutics, Inc., and SalioGen Therapeutics, Inc. (incorporated by reference to Exhibit 10.1 to the registrant's Current Report on Form 8-K filed on July 12, 2022).
10.24	Subscription Agreement, dated as of July 14, 2023 (incorporated by reference to Exhibit 10.1 to the registrant's Current Report on Form 8-K filed on November 6, 2023).
10.25	Registration Rights Agreement, dated as of July 14, 2023 (incorporated by reference to Exhibit 10.2 to the registrant's Current Report on Form 8-K filed on November 6, 2023).
10.26	Warrant Agreement dated January 22, 2021 (incorporated by reference to Exhibit 10.13 to the Form 8-K, filed November 6, 2023).
21.1	List of Subsidiaries.
23.1	Consent of Ernst & Young LLP.
24.1	Power of Attorney (included on the signature page attached hereto).
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1¥	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97.1#	Korro Bio, Inc. Compensation Recovery Policy.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

# Indicates a management contract or any compensatory plan, contract or arrangement.
+ Schedules and exhibits have been omitted pursuant to Item 601(b)(2) of Regulation S-K. We agree to furnish supplementally a copy of any omitted schedule or exhibit to the SEC upon request.
¥ These certifications will not be deemed "filed" for purposes of Section 18 of the Exchange Act or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act or the Exchange Act except to the extent specifically incorporated by reference into such filing.

Item 16. Form 10-K Summary

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized**.**

Korro Bio, Inc.

Date: March 26, 2024

By: /s/ Ram Aiyar
Ram Aiyar
President and Chief Executive Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Ram Aiyar and Vineet Agarwal, and each of them, as his or her true and lawful attorneys-in-fact and agents, each with the full power of substitution, for him or her and in his or her name, place or stead, in any and all capacities, to sign any and all amendments to this annual report on Form 10-K, and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or their, his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant in the capacities and on the dates indicated.

Name	Title	Date
/s/ Ram Aiyar Ram Aiyar	President, Chief Executive Officer and Director *Principal Executive Officer*	March 26, 2024
/s/Vineet Agarwal Vineet Agarwal	Chief Financial Officer *Principal Financial Officer and Principal Accounting Officer*	March 26, 2024
/s/ Ali Behbahani Ali Behbahani	Director	March 26, 2024
/s/ Nessan Bermingham Nessan Bermingham	Director	March 26, 2024
/s/ Jean-Francois Formela Jean-Francois Formela	Director	March 26, 2024
/s/ David L. Lucchino David L. Lucchino	Director	March 26, 2024
/s/ Rachel Meyers Rachel Meyers	Director	March 26, 2024
/s/ Timothy Pearson Timothy Pearson	Director	March 26, 2024

KORRO BIO, Inc.
One Kendall Square
Building 600-700
Suite 6-401
Cambridge, MA 02139

www.korrobio.com