UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2026

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.)**
60 First Street, 2nd Floor, Suite 250 **Cambridge, MA**	**02141**
(Address of principal executive offices)	**(Zip Code)**

(617) 468-1999

(Registrant's telephone number, including area code)

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

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 is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 3, 2026, the registrant had 13,725,646 shares of common stock, $0.001 par value per share, outstanding.

Table of Contents

		Page
PART I.	FINANCIAL INFORMATION	1
Item 1.	Financial Statements (Unaudited)	1
	Condensed Consolidated Balance Sheets	1
	Condensed Consolidated Statements of Operations and Comprehensive Loss	2
	Condensed Consolidated Statements of Stockholders' Equity	3
	Condensed Consolidated Statements of Cash Flows	4
	Notes to Unaudited Condensed Consolidated Financial Statements	5
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	18
Item 3.	Quantitative and Qualitative Disclosures About Market Risk	28
Item 4.	Controls and Procedures	28
PART II.	OTHER INFORMATION	29
Item 1.	Legal Proceedings	29
Item 1A.	Risk Factors	29
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	78
Item 3.	Defaults Upon Senior Securities	78
Item 4.	Mine Safety Disclosures	78
Item 5.	Other Information	78
Item 6.	Exhibits	79
Signatures		80

Unless the context otherwise indicates, references in this Quarterly Report on Form 10-Q to the "Company," "we," "our" and "us" refer, collectively, to Korro Bio, Inc., a Delaware corporation, and its consolidated subsidiaries (including Legacy Korro) after completion of our November 2023 business combination. The term "Legacy Korro" refers to privately held Korro Bio Ops, Inc. (formerly known as Korro Bio, Inc.), which we acquired in the November 2023 business combination.

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 Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, the trademarks and trade names in this Quarterly Report on Form 10-Q 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.

In this Quarterly Report on Form 10-Q, when we refer to our leads or lead candidates, we are referring to our RNA-editing oligonucleotides, or REOs, that we are researching for potential nomination as a development candidate. When we refer to our development candidates, we generally mean an REO that we have nominated for clinical development.

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or this Quarterly Report, 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, express or implied 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," "intend," "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 Quarterly Report may include, for example, express or implied statements about:

- the timing, progress and results of our Phase 1/2 clinical trial program of KRRO-121, or ANCHOR, including the completion of Part 1 in healthy volunteers, start of dosing of patients with urea cycle disorders, or UCD, and timing of interim clinical readout;
- the timing and progress of preclinical development of KRRO-111, including ongoing studies, and the timing of regulatory submission;
- the timing of and our ability to make regulatory submissions and obtain regulatory approvals and clearances, including initiating Part 2 of ANCHOR and any investigational new drug application, or IND, or comparable foreign application for KRRO-121 or KRRO-111 and any other developmental candidates;
- the acceptance by the U.S. Food and Drug Administration, or the FDA, and other regulatory authorities of data from clinical trials conducted outside the United States;
- the potential of KRRO-121 to treat hyperammonemia in patients with UCD and hepatic encephalopathy, or HE, and the development and regulatory pathway for KRRO-121 in HE;
- the potential benefits of orphan drug designation granted by the European Commission and rare pediatric disease designation granted by the FDA for KRRO-121 for the treatment of UCD;
- the initiation, timing, progress, results, cost of and other statements regarding 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, and the period during which the results of the trials will become available, including the availability of interim, topline or preliminary data;
- our strategy;
- our cash runway and operating expenses (including research and development) and capital expenditure requirements, and our ability to obtain additional funding;
- the anticipated benefits of our organizational streamlining and workforce reductions;
- the therapeutic and commercial potential of our development candidates, including the potential of KRRO-121 as a first-in-class treatment and of KRRO-111 as a best-in-class treatment;
- the extent to which results of preclinical studies may be predictive of results in clinical trials;
- the termination of our collaboration and license agreement with Novo Nordisk A/S, or Novo Nordisk, including the expected effective date of the termination, the reimbursement of wind-down costs and the expected recognition of the remaining deferred revenue under the agreement in the fourth quarter of 2026, and any future collaboration or licensing arrangements;
- 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 development candidates;
- competitive developments, including the impact on our competitive position of rival products and development candidates, such as therapies in development for UCD and alpha-1 antitrypsin deficiency, or AATD, and our ability to meet such competition;

- our reliance on third parties, including contract research organizations, clinical trial sites in Australia and third-party manufacturers and suppliers;
- the effects of macroeconomic and geopolitical conditions, including inflation, interest rates, tariffs and military hostilities, on our business and operations; 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 II, Item 1A of this Quarterly Report and the "*Summary of Risk Factors*" below. 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 Quarterly Report, 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 Quarterly 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 II, Item 1A of this Quarterly Report. 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.
- 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.
- If preclinical studies or clinical trials of any lead candidates or development candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results (such as we experienced in the Phase 1/2a REWRITE clinical trial of KRRO-110 for AATD), we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such development candidates.
- Our business is substantially dependent on the success of KRRO-121, our lead development candidate, which only recently entered clinical development and has not been administered to any patient with UCD or HE. If KRRO-121 fails to advance successfully, our business would be materially and adversely affected.
- ANCHOR is the first clinical trial of KRRO-121 and of any development candidate designed to generate a de novo protein using our OPERA platform, and its design, including its initial evaluation may not generate data sufficient to support further development of, or regulatory approval.
- We have not initiated any clinical trial of KRRO-121 in patients with HE, and the development and regulatory pathway for KRRO-121 in HE is uncertain and is likely to require larger, longer and more costly clinical trials than in UCD.
- If we experience delays or difficulties in the enrollment of patients in clinical trials, including patients with UCD, which is a rare disease, or other delays in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.
- We may not realize the anticipated benefits of our organizational streamlining and workforce reductions.
- 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 development candidates. It will be many years before we or our collaborators commercialize a development candidate or generate any revenues, if ever. Additionally, other genetic editing technologies may be discovered that provide significant advantages over RNA editing, which could materially harm our business.
- RNA editing is a novel technology with limited clinical validation 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 development efforts, and our preclinical studies and clinical trials may not be successful. KRRO-121 only recently entered clinical development, and we have not completed any clinical trial of any of our development candidates. Any favorable results we have, including those of KRRO-121 and KRRO-111, may not be predictive of results that may be observed in later preclinical studies or clinical trials, such as our experience in the Phase 1/2a REWRITE clinical trial of KRRO-110 for AATD, and we have experienced, and may continue to experience, delays in developing a potential treatment for AATD. If we are unable to commercialize our development candidates or experience significant delays in doing so, our business will be materially harmed.
- Any development candidates we develop may fail in preclinical or clinical development or be delayed to a point where they do not become commercially viable.
- Interim, topline and preliminary data from our clinical trials, including ANCHOR, may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.

- Because we are developing REOs, 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.
- If we are not able to obtain or protect intellectual property rights related to any of our lead candidates or development candidates, development and commercialization of our REO leads or development candidates may be adversely affected.
- We rely, and expect to continue to rely, on third parties, including contract research organizations and clinical trial sites in Australia, to conduct our clinical trials, including ANCHOR, and those third parties may not perform satisfactorily.
- Novo Nordisk has elected to terminate our collaboration agreement, and we may not be successful in finding other strategic collaborators for continuing development of certain of our development candidates or successfully commercializing or competing in the market for certain indications.
- The price of our common stock is volatile and fluctuates substantially, which could result in substantial losses for our stockholders.
- 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.
- Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.
- Unfavorable global economic conditions could adversely affect our business, financial condition, results of operations or cash flows.
- Our business may be impacted by macroeconomic conditions, including inflation, rising interest rates and volatile market conditions, and other uncertainties beyond our control, including geopolitical events, such as ongoing U.S. and Israeli military action in Iran, and effects thereof.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Korro Bio, Inc.
Condensed Consolidated Balance Sheets
(unaudited)
(amounts in thousands, except share and par value amounts)

	September 30, 2026	December 31, 2025
Assets:		
Current assets:		
Cash and cash equivalents	$ 40,215	$ 21,824
Short-term marketable securities	64,779	53,332
Accounts receivable	—	969
Prepaid expenses and other current assets	3,941	6,247
Total current assets	108,935	82,372
Property and equipment, net	7,244	8,164
Operating lease right-of-use assets	7,333	8,058
Restricted cash, net of current portion	2,409	2,409
Long-term marketable securities	19,829	10,031
Other non-current assets	2,370	2,472
Total assets	$ 148,120	$ 113,506
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,296	$ 1,651
Accrued expenses and other current liabilities	5,544	7,614
Operating lease liabilities, current portion	3,073	2,672
Deferred revenue, current portion	3,010	—
Total current liabilities	14,923	11,937
Operating lease liabilities, net of current portion	38,450	40,814
Deferred revenue, net of current portion	4,825	7,835
Other non-current liabilities	1,548	1,481
Total liabilities	59,746	62,067
Commitments and contingencies (Note 12)		
Stockholders' equity		
Preferred stock, $0.001 par value; 10,000,000 shares authorized at September 30, 2026 and December 31, 2025; no shares issued and outstanding at September 30, 2026 and December 31, 2025	—	—
Common stock, $0.001 par value; 200,000,000 shares authorized at September 30, 2026 and December 31, 2025; 14,725,646 shares and 9,417,295 shares issued at September 30, 2026 and December 31, 2025, respectively; 13,725,646 shares and 9,417,295 shares outstanding at September 30, 2026 and December 31, 2025, respectively	14	9
Additional paid-in capital	540,720	435,064
Treasury stock, at cost, 1,000,000 shares held at September 30, 2026 and no shares held at December 31, 2025	(12,120)	—
Accumulated other comprehensive (loss) income	(305)	212
Accumulated deficit	(439,935)	(383,846)
Total stockholders' equity	88,374	51,439
Total liabilities and stockholders' equity	$ 148,120	$ 113,506

The accompanying notes are an integral part of these condensed consolidated financial statements.

Korro Bio, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)
(amounts in thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Collaboration revenue	$ —	$ 1,090	$ —	$ 5,100
Operating expenses:				
Research and development	12,104	13,820	38,643	53,694
General and administrative	6,352	6,507	20,359	21,632
Restructuring charge	—	—	—	1,233
Total operating expenses	18,456	20,327	59,002	76,559
Loss from operations	(18,456)	(19,237)	(59,002)	(71,459)
Other income:				
Other income, net	1,168	1,176	3,376	4,242
Total other income, net	1,168	1,176	3,376	4,242
Loss before provision for income taxes	(17,288)	(18,061)	(55,626)	(67,217)
Provision for income taxes	(260)	—	(463)	(1)
Net loss	$ (17,548)	$ (18,061)	$ (56,089)	$ (67,218)
Other comprehensive income:				
Unrealized (loss) gain on available-for-sale marketable securities	(131)	48	(558)	(29)
Foreign currency translation adjustments, net	15	(11)	41	(25)
Comprehensive loss	$ (17,664)	$ (18,024)	$ (56,606)	$ (67,272)
Net loss per share, basic and diluted	$ (0.98)	$ (1.92)	$ (3.57)	$ (7.16)
Weighted-average shares used in computing net loss per share, basic and diluted	17,867,091	9,391,559	15,717,711	9,388,816

The accompanying notes are an integral part of these condensed consolidated financial statements.

Korro Bio, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(unaudited)
(amounts in thousands, except share amounts)

	Common Stock		Treasury Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
Balance at December 31, 2025	9,417,295	$ 9	—	$ —	$ 435,064	$ 212	$ (383,846)	$ 51,439
Exercises of stock options	1,487	—	—	—	17	—	—	17
Stock-based compensation expense	—	—	—	—	2,731	—	—	2,731
Issuance of common stock in connection with the at-the-market program, net of offering costs	501,861	—	—	—	5,020	—	—	5,020
Issuance of common stock and pre-funded warrants in connection with the private placement, net of offering costs	4,501,928	5	—	—	80,253	—	—	80,258
Other comprehensive loss	—	—	—	—	—	(207)	—	(207)
Net loss	—	—	—	—	—	—	(19,625)	(19,625)
Balance at March 31, 2026	14,422,571	$ 14	—	$ —	$ 523,085	$ 5	$ (403,471)	$ 119,633
Stock-based compensation expense	—	—	—	—	2,703	—	—	2,703
Vesting of restricted stock units	233,075	—	—	—	—	—	—	—
Vesting of performance stock units	50,000	—	—	—	—	—	—	—
Other comprehensive loss	—	—	—	—	—	(194)	—	(194)
Net loss	—	—	—	—	—	—	(18,916)	(18,916)
Balance at June 30, 2026	14,705,646	$ 14	—	$ —	$ 525,788	$ (189)	$ (422,387)	$ 103,226
Stock-based compensation expense	—	—	—	—	2,812	—	—	2,812
Vesting of restricted stock units	20,000	—	—	—	—	—	—	—
Issuance of pre-funded warrants in exchange for common stock	—	—	(1,000,000)	(12,120)	12,120	—	—	—
Other comprehensive loss	—	—	—	—	—	(116)	—	(116)
Net loss	—	—	—	—	—	—	(17,548)	(17,548)
Balance at September 30, 2026	14,725,646	$ 14	(1,000,000)	$ (12,120)	$ 540,720	$ (305)	$ (439,935)	$ 88,374
Balance at December 31, 2024	9,377,259	$ 9	—	$ —	$ 426,724	$ 268	$ (266,586)	$ 160,415
Exercises of stock options	11,643	—	—	—	172	—	—	172
Stock-based compensation expense	—	—	—	—	1,761	—	—	1,761
Other comprehensive loss	—	—	—	—	—	(4)	—	(4)
Net loss	—	—	—	—	—	—	(23,387)	(23,387)
Balance at March 31, 2025	9,388,902	$ 9	—	$ —	$ 428,657	$ 264	$ (289,973)	$ 138,957
Exercises of stock options	1,940	—	—	—	5	—	—	5
Stock-based compensation expense	—	—	—	—	1,998	—	—	1,998
Other comprehensive loss	—	—	—	—	—	(87)	—	(87)
Net loss	—	—	—	—	—	—	(25,770)	(25,770)
Balance at June 30, 2025	9,390,842	$ 9	—	$ —	$ 430,660	$ 177	$ (315,743)	$ 115,103
Exercises of stock options	7,142	—	—	—	128	—	—	128
Stock-based compensation expense	—	—	—	—	1,825	—	—	1,825
Other comprehensive income	—	—	—	—	—	37	—	37
Net loss	—	—	—	—	—	—	(18,061)	(18,061)
Balance at September 30, 2025	9,397,984	$ 9	—	$ —	$ 432,613	$ 214	$ (333,804)	$ 99,032

The accompanying notes are an integral part of these condensed consolidated financial statements.

Korro Bio, Inc.
Condensed Consolidated Statements of Cash Flows
(unaudited)
(amounts in thousands)

	Nine Months Ended September 30,	
	2026	2025
Operating Activities:		
Net loss	$ (56,089)	$ (67,218)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash lease expense	725	504
Stock-based compensation expense	8,246	5,584
Depreciation expense	1,103	3,631
Net amortization of premiums and discounts on marketable securities	(7)	(467)
Loss on disposal of property and equipment	7	81
Changes in operating assets and liabilities:		
Accounts receivable	969	876
Prepaid expenses and other current assets	1,309	(189)
Accounts payable	1,542	(1,674)
Accrued expenses and other current liabilities	(2,031)	(319)
Deferred revenue	—	(1,268)
Operating lease liabilities	(1,963)	(97)
Other non-current assets	186	(303)
Net cash used in operating activities	(46,003)	(60,859)
Investing Activities:		
Purchases of marketable securities	(86,996)	(26,189)
Proceeds from maturities of marketable securities	65,200	55,700
Purchases of property and equipment	(85)	(434)
Net cash (used in) provided by investing activities	(21,881)	29,077
Financing Activities:		
Proceeds from the issuance of common stock in connection with the at-the-market program, net of offering costs	5,020	—
Proceeds from the issuance of common stock and pre-funded warrants in connection with the private placement, net of offering costs	80,258	—
Proceeds from exercises of stock options	17	305
Net cash provided by financing activities	85,295	305
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(17)	(11)
Net increase (decrease) in cash, cash equivalents and restricted cash	17,394	(31,488)
Cash, cash equivalents and restricted cash, beginning of period	25,230	59,049
Cash, cash equivalents and restricted cash, end of period	$ 42,624	$ 27,561
Non-cash investing and financing activities:		
Purchases of property and equipment in accounts payable and accrued expenses	$ 105	$ 14
Supplemental cash flow information:		
Cash paid for income taxes	$ 109	$ 1
Cash paid for operating lease liabilities	$ 5,437	$ 3,944

The accompanying notes are an integral part of these condensed consolidated financial statements.

Korro Bio, Inc.
Notes to Unaudited Condensed Consolidated Financial Statements

1. The Company and Liquidity

Nature of Business

Korro Bio, Inc. (together with its subsidiaries, the "Company") is a biopharmaceutical company leveraging a novel oligonucleotide promoted editing of RNA, or OPERA, platform to develop a new class of genetic medicines for rare and highly prevalent diseases. The Company was incorporated in November 2014 as a Delaware corporation and in November 2023 completed a reverse merger with its now wholly-owned subsidiary and changed its name from Frequency Therapeutics, Inc. ("Frequency") to Korro Bio, Inc. 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. Lead candidates and development candidates currently under development will require significant additional research and development efforts, including extensive preclinical 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.

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, cash equivalents and marketable securities as of September 30, 2026 of $124.8 million will enable the Company to fund its planned operating expenses and capital expenditure requirements for at least 12 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 September 30, 2026, the Company had an accumulated deficit of $439.9 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 development 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, lead candidates and development 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.

Unaudited Interim Condensed Consolidated Financial Information

The accompanying condensed consolidated financial statements as of September 30, 2026 and for the three and nine months ended September 30, 2026 and 2025 are unaudited. The financial data and other information contained in the notes hereto as of September 30, 2026 and for the three and nine months ended September 30, 2026 and 2025 are also unaudited. The condensed consolidated balance sheet data as of December 31, 2025 was derived from the Company's audited consolidated financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025 (the "2025 Form 10-K").

The unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited annual consolidated financial statements, and in the opinion of management, reflect all adjustments, which include any normal recurring

adjustments necessary for the fair presentation of the Company's interim period results. These unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2025, and the notes thereto, included in the 2025 Form 10-K.

The results for the three and nine months ended September 30, 2026 are not necessarily indicative of results to be expected for the year ended December 31, 2026, or any other interim periods, or any future year or period.

Summary of Significant Accounting Policies

The significant accounting policies used in preparation of the condensed consolidated financial statements are described in the Company's audited consolidated financial statements as of the year end December 31, 2025, and the notes thereto, which are included in the 2025 Form 10-K. Other than the warrants and treasury stock policies described below, there have been no material changes to the significant accounting policies previously disclosed in the 2025 Form 10-K.

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. Estimates and assumptions reflected in these consolidated financial statements include, but are not limited to accrued research and development expenses and stock-based compensation expense. 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.

Warrants

The Company accounts for common stock warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant’s specific terms and applicable authoritative guidance in Accounting Standards Codification (“ASC”) No. 480, Distinguishing Liabilities from Equity (“ASC 480”), and ASC No. 815, Derivatives and Hedging (“ASC 815”). The assessment considers whether the warrants are freestanding financial instruments pursuant to ASC 480, whether the warrants meet the definition of a liability pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to the Company’s own common stock and whether the warrant holders could potentially require “net cash settlement” in a circumstance outside of the Company’s control, among other conditions for equity classification. This assessment, which requires the use of judgment, is conducted at the time of warrant issuance and as of each subsequent quarterly period end date while the warrants are outstanding.

For issued or modified warrants that meet all of the criteria for equity classification, the warrants are required to be recorded as a component of additional paid-in capital at the time of issuance. For issued or modified warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance and remeasured each balance sheet date thereafter. Changes in the estimated fair value of the liability-classified warrants are recognized as a non-cash gain or loss in the accompanying consolidated statements of operations and comprehensive loss.

Treasury Stock

The Company records treasury stock activities under the cost method. Treasury stock is included in authorized and issued shares but excluded from outstanding shares. The re-issuance of treasury stock is accounted for on a first-in, first-out basis and any differences between the cost of treasury stock and the re-issuance proceeds are charged or credited to additional paid-in capital.

Recent Accounting Pronouncements—Yet to be Adopted

The Company considers the applicability and impact of all Accounting Standards Updates (“ASU” or “ASUs”). Accounting standards that have been issued or proposed by the Financial Accounting Standards Board (“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 2024, the FASB issued *ASU 2024-03, Income Statement-Reporting Comprehensive Income- Expense Disaggregation Disclosures*, which requires disclosure of additional information about specific expense categories in the notes to the financial statements on an interim and annual basis. The standard is effective for fiscal years beginning after December 15, 2026, and for interim periods beginning after December 15, 2027, with early adoption permitted. The Company is currently evaluating the disclosure requirements related to this new standard.

3. Fair Value Measurements

The Company measures the fair value of money market funds based on quoted prices in active markets for identical securities. Cash equivalents and marketable securities include U.S. treasury bills and U.S. government agency 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 and liabilities measured at fair value on a recurring basis as of September 30, 2026 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)
Financial assets				
Cash equivalents:				
Money market funds	$ 24,472	$ 24,472	$ —	$ —
U.S. treasury bills	14,898	—	14,898	—
Marketable securities				
U.S. treasury bills	4,985	—	4,985	—
U.S. government agency securities	79,623	—	79,623	—
MS APA asset	1,548	—	—	1,548
Total financial assets	$ 125,526	$ 24,472	$ 99,506	$ 1,548
Financial liabilities				
CVR liability	$ 1,548	$ —	$ —	$ 1,548
Total financial liabilities	$ 1,548	$ —	$ —	$ 1,548

Assets and liabilities measured at fair value on a recurring basis as of December 31, 2025 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)
Financial assets				
Cash equivalents:				
Money market funds	$ 21,377	$ 21,377	$ —	$ —
Marketable securities				
U.S. government agency securities	63,363	—	63,363	—
MS APA asset	1,481	—	—	1,481
Total financial assets	$ 86,221	$ 21,377	$ 63,363	$ 1,481
Financial liabilities				
CVR liability	$ 1,481	$ —	$ —	$ 1,481
Total financial liabilities	$ 1,481	$ —	$ —	$ 1,481

The fair value of the contingent value right ("CVR") liability and the asset purchase agreement relating to Frequency's multiple sclerosis program (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 judgment, estimates and assumptions, including the probability of technical and regulatory success, and discount rates. For the nine months ended September 30, 2026, the aggregate change in fair value of the CVR liability and MS APA asset was less than $0.1 million. For the year ended December 31, 2025, the aggregate change in fair value of the CVR liability and MS APA asset was $0.1 million.

There were no changes in valuation techniques, nor were there any transfers among the fair value hierarchy levels during the nine months ended September 30, 2026 or during the year ended December 31, 2025.

4. Marketable Securities

Marketable securities were comprised as follows as of September 30, 2026 (in thousands):

	Maturities	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
U.S. treasury bills	Within 1 year	$ 4,986	$ —	$ (1)	$ 4,985
U.S. government agency securities	Within 1 year	60,015	—	(221)	59,794
U.S. government agency securities	Between 1 to 2 years	20,000	2	(173)	19,829
Total		$ 85,001	$ 2	$ (395)	$ 84,608

Marketable securities were comprised as follows as of December 31, 2025 (in thousands):

	Maturities	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
U.S. government agency securities	Within 1 year	$ 53,220	$ 112	$ —	$ 53,332
U.S. government agency securities	Between 1 to 2 years	9,985	46	—	10,031
Total		$ 63,205	$ 158	$ —	$ 63,363

The amortized cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. There were no realized gains or losses on available-for-sale securities for the periods presented. None of the investments were in an unrealized loss position for greater than 12 months as of September 30, 2026. The unrealized losses on the Company's available-for-sale securities were caused by the impact of central bank and market interest rates on the investments held. The Company does not intend to sell the investments, and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost bases. After analyzing the securities in an unrealized loss position, the portion of these losses that relates to changes in credit quality is insignificant. The Company did not record an allowance for credit losses as of September 30, 2026. Furthermore, the Company does not believe that these securities expose the Company to undue market risk or counterparty credit risk.

5. Restricted Cash

As of September 30, 2026, the Company maintained current restricted cash of $0.0 million and non-current restricted cash of $2.4 million. As of December 31, 2025, the Company maintained current restricted cash of $1.0 million and non-current restricted cash of $2.4 million. The current restricted cash of $1.0 million as of December 31, 2025 is included under the caption “prepaid expenses and other current assets” in the Company's condensed consolidated balance sheet. 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 September 30, 2026 and December 31, 2025 that sums to the total of the same amounts shown in the consolidated statements of cash flows (in thousands):

	September 30, 2026	December 31, 2025
Cash and cash equivalents	$ 40,215	$ 21,824
Restricted cash	2,409	3,406
Cash, cash equivalents and restricted cash	$ 42,624	$ 25,230

6. Property and Equipment, Net

Property and equipment, net, as of September 30, 2026 and December 31, 2025 was comprised as follows (in thousands):

	Estimated Useful Life (in Years)	September 30, 2026	December 31, 2025
Laboratory equipment	5	$ 8,415	$ 8,312
Furniture and office equipment	4	651	651
Computer equipment	3	89	89
Leasehold improvements	Shorter of useful life or remaining lease term	9,681	9,681
Total property and equipment, gross		18,836	18,733
Less: accumulated depreciation		(11,592)	(10,569)
Total property and equipment, net		$ 7,244	$ 8,164

Property and equipment are recorded at historical cost, net of accumulated depreciation. Depreciation expense for the three months ended September 30, 2026 and 2025 was $0.4 million and $1.2 million, respectively. Depreciation expense for the nine months ended September 30, 2026 and 2025 was $1.1 million and $3.6 million, respectively.

7. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities as of September 30, 2026 and December 31, 2025 were comprised as follows (in thousands):

	September 30, 2026	December 31, 2025
Employee compensation and benefits	$ 2,756	$ 1,846
External research and development services	2,156	3,236
Severance	—	2,196
Professional fees	514	266
Other operating expenses	118	70
Total accrued expenses and other current liabilities	$ 5,544	$ 7,614

8. Common Stock

As of September 30, 2026, the Company was authorized to issue 200,000,000 shares of common stock, $0.001 par value per share. 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 September 30, 2026, no dividends had been declared.

As of September 30, 2026 and December 31, 2025, the Company had reserved for future issuance the following number of shares of common stock:

	September 30, 2026	December 31, 2025
Exercises of outstanding stock options	1,894,911	1,647,077
Exercise of outstanding warrant	8,049	8,049
Exercise of pre-funded warrants	4,148,836	—
Vesting of restricted stock unit awards	442,745	506,981
Vesting of performance stock unit awards	70,000	—
Future issuances under 2023 Stock Incentive Plan	102,290	142,985
Future issuances under 2023 ESPP Plan	345,671	262,440
Future issuances under 2026 Inducement Plan	53,400	—
Total reserved for future issuance	7,065,902	2,567,532

March 2026 Private Placement

On March 9, 2026, the Company entered into a subscription agreement with certain new and existing accredited investors to sell and issue in a private placement ("PIPE"): (i) an aggregate of 4,501,928 shares of its common stock at a purchase price of $11.11 per share and (ii) pre-funded warrants to acquire an aggregate 3,148,836 shares of its common stock at a purchase price of $11.109 per pre-funded warrant. The closing of the PIPE occurred on March 10, 2026 raising aggregate gross cash proceeds of approximately $85.0 million, before deducting placement agent fees and offering expenses of approximately $4.7 million. The pre-funded warrants have an exercise price of $0.001 per share. No pre-funded warrants issued in connection with the PIPE have been exercised as of September 30, 2026.

August 2026 Warrant Exchange

On August 5, 2026, the Company entered into an exchange agreement ("Exchange") with entities affiliated with Venrock Healthcare Capital Partners ("Venrock"), pursuant to which (i) Venrock agreed to surrender an aggregate of 1,000,000 shares of the Company's common stock for no consideration, to the Company, and (ii) the Company agreed to issue pre-funded warrants to purchase an aggregate of 1,000,000 shares of its common stock, with an exercise price of $0.001 per share and no expiration date. The pre-funded warrants are exercisable immediately and have terms substantially identical to the pre-funded warrants issued in the PIPE. No pre-funded warrants issued in connection with the Exchange have been exercised as of September 30, 2026. The surrendered shares were not retired and were recorded as treasury stock of $12.1 million, based on the fair value of the Company's common stock on the date of the exchange, with a corresponding increase to additional paid-in capital for the pre-funded warrants issued.

The Company has assessed the pre-funded warrants issued in connection with the PIPE and Exchange for appropriate equity or liability classification pursuant to the Company’s accounting policy described in Note 2, Basis of Presentation and Summary of Significant Accounting Policies. During this assessment, the Company determined the pre-funded warrants are a freestanding instrument that does not meet the definition of a liability pursuant to ASC 480 and does not meet the definition of a derivative pursuant to ASC 815. The pre-funded warrants are indexed to the Company’s common stock and meet all other conditions for equity classification under ASC 815. Based on the results of this assessment, the Company concluded that the pre-funded warrants are a freestanding equity-linked financial instrument that meets the criteria for equity classification under ASC 815. Accordingly, the pre-funded warrants are classified as equity and accounted for as a component of additional paid-in capital at the time of issuance. The Company also determined that the pre-funded warrants should be included in the determination of basic and diluted earnings per share in accordance with ASC 260, Earnings per Share.

At-The-Market Equity Program

In December 2024, the Company filed a Shelf Registration Statement on Form S-3 (the "Shelf Registration Statement") with the Securities and Exchange Commission ("SEC"), which covered the offering, issuance and sale by the Company of up to an aggregate of $400.0 million of the Company's common stock, preferred stock, debt securities, units and/or warrants. The Shelf Registration Statement included a prospectus covering the offering, issuance and sale of up to $100.0 million of the Company's common stock from time to time through an "at-the-market" (“ATM”) offering under the Securities Act of 1933, as amended.

Also in December 2024, the Company entered into an Open Market Sale Agreement (the “Sales Agreement”) with TD Cowen Securities (USA) LLC ("TD Cowen"), acting as the Company's Agent and/or principal (the "Sales Agent") with respect to an ATM offering program under which the Company may from time to time, at its sole discretion, issue and sell shares of its common stock having an aggregate offering price of up to $100.0 million through the Sales Agent. TD Cowen is entitled to compensation for its services equal to up to 3.0% of the aggregate gross proceeds of any shares of common stock sold through TD Cowen under the Sales Agreement. Pursuant to the Sales Agreement, any shares will be sold pursuant to the Shelf Registration Statement filed with the SEC on December 2, 2024, including the ATM prospectus contained therein, as declared effective by the SEC on December 9, 2024. In January 2026, the Company issued 501,861 shares of common stock under the Sales Agreement for gross proceeds of $5.1 million, before deducting offering expenses of approximately $0.1 million.

9. Preferred Stock

As of September 30, 2026, 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.

10. Stock-based Compensation

In April 2026, the Company's Board of Directors adopted the Korro Bio, Inc. 2026 Inducement Plan (the "Inducement Plan"). The Inducement Plan provides for the grant of non-qualified stock options, stock appreciation rights, restricted stock units, restricted stock awards, unrestricted stock awards, and dividend equivalent rights. Awards under the Inducement Plan may only be granted to new employees who were not previously an employee or director of the Company or are commencing employment with the Company

following a bona fide period of non-employment, in either case, as an inducement material to the individual's entering into employment with the Company and in accordance with the requirements of Nasdaq Listing Rule 5635(c)(4). A total of 100,000 shares of common stock were reserved for issuance under the Inducement Plan.

The Company also grants stock-based awards under its 2023 Stock Option and Incentive Plan (the "2023 Plan"), which was approved by the Company's stockholders in November 2023. The Company also has outstanding stock option awards under the Korro Bio Ops, Inc. 2019 Plan (the "Legacy Korro 2019 Plan"), the Frequency 2014 Stock Incentive Plan (the "2014 Plan"), and the Frequency 2019 Incentive Award Plan (the "Frequency 2019 Plan"), but is no longer granting awards under these plans. The Company also issues shares under the 2023 Employee Stock Purchase Plan (the "2023 ESPP"), which was approved by the Company's stockholders in November 2023. The first offering period under the 2023 ESPP commenced on July 1, 2026.

Stock-based Compensation Expense

Total stock-based compensation expense recognized in the consolidated statements of operations and comprehensive loss for the three and nine months ended September 30, 2026 and 2025 was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 1,072	$ 685	$ 2,787	$ 2,106
General and administrative	1,740	1,140	5,459	3,478
Total stock-based compensation expense	$ 2,812	$ 1,825	$ 8,246	$ 5,584

Stock Option Activity

The fair value of stock options granted during the nine months ended September 30, 2026 and 2025 was calculated on the date of grant using the following weighted-average assumptions:

	Nine Months Ended September 30,	
	2026	2025
Risk-free interest rate	4.1%	4.3%
Expected dividend yield	—%	—%
Expected term (in years)	6.0	6.0
Expected volatility	78.3%	73.9%

Using the Black-Scholes option pricing model, the weighted-average grant date fair value of stock options granted during the nine months ended September 30, 2026 and 2025 was $8.71 and $18.96 per share, respectively.

The following table summarizes changes in stock option activity during the nine months ended September 30, 2026 (in thousands, except share and per share amounts):

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2025	1,647,077	$ 26.49	7.3	$ —
Granted	452,862	$ 12.42		
Exercised	(1,487)	$ 11.68		
Forfeited	(129,843)	$ 29.10		
Cancelled	(73,698)	$ 25.51		
Outstanding as of September 30, 2026	1,894,911	$ 22.50	7.2	$ 16
Exercisable at September 30, 2026	1,059,516	$ 24.66	6.0	$ —

Compensation cost of $4.2 million and $5.6 million related to the options was recognized during the nine months ended September 30, 2026 and 2025, respectively. Compensation cost of $1.5 million and $1.8 million related to the options was recognized during the three months ended September 30, 2026 and 2025, respectively.

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 nine months ended September 30, 2026 and 2025 was $0.0 million and $0.4 million, respectively.

As of September 30, 2026, there was unrecognized stock-based compensation expense related to unvested stock options of $9.9 million, which the Company expects to recognize over a weighted-average period of approximately 2.4 years.

Restricted Stock Units

During the nine months ended September 30, 2026, the Company granted Restricted Stock Units ("RSUs") to employees of the Company. RSUs are valued at the market price of a share of the Company's stock on the date of grant. Compensation expense for RSUs is recognized on a straight-line basis over the service period. Forfeitures are recorded in the period in which they occur.

The following table summarizes changes in RSU activity during the nine months ended September 30, 2026:

	Shares	Weighted-Average Grant Date Fair Value Per Share
Unvested restricted stock unit awards as of December 31, 2025	506,981	$ 7.96
Issued	253,315	$ 13.07
Vested	(253,075)	$ 8.36
Forfeited	(64,476)	$ 8.79
Unvested restricted stock unit awards as of September 30, 2026	442,745	$ 10.54

Compensation cost of $3.3 million and $0.0 million related to the RSUs was recognized during the nine months ended September 30, 2026 and 2025, respectively. Compensation cost of $1.3 million and $0.0 million related to the RSUs was recognized during the three months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, there was unrecognized stock-based compensation expense related to unvested RSUs of $3.3 million, which the Company expects to recognize over a weighted-average period of approximately 1.4 years.

Performance Stock Units

During the nine months ended September 30, 2026, the Company granted Performance Stock Units ("PSUs") to an employee of the Company.

PSUs are valued at the market price of a share of the Company's stock on the date of grant. Compensation expense for PSUs is recognized on a straight-line basis over the requisite service periods when the achievement of the performance condition is determined to be probable, using management's best estimate. If a performance condition is not determined to be probable or is not met, no stock-based compensation expense is recognized, and any previously recognized expense is reversed. Forfeitures are recorded in the period in which they occur.

The following table summarizes changes in PSU activity during the nine months ended September 30, 2026:

	Shares	Weighted-Average Grant Date Fair Value Per Share
Unvested performance stock unit awards as of December 31, 2025	—	$ —
Issued	120,000	$ 13.05
Vested	(50,000)	$ 13.05
Forfeited	—	$ —
Unvested performance stock unit awards as of September 30, 2026	70,000	$ 13.05

Compensation cost of $0.7 million and $0.0 million related to the PSUs was recognized during the nine months ended September 30, 2026 and 2025, respectively. No compensation cost related to the PSUs was recognized during the three months ended September 30, 2026 and 2025. As of September 30, 2026, there was unrecognized stock-based compensation expense related to unvested PSUs of $0.9 million, which will be recognized if and when achievement of the related performance conditions is deemed probable.

Employee Stock Purchase Plan

The 2023 ESPP allows employees to purchase shares of the Company's common stock. The purchase price is equal to 85% of the lower of the closing price of the Company's common stock on the first day of the offering period or the last day of the offering period. The Company commenced the first offering pursuant to the 2023 ESPP on July 1, 2026, and such offering will end on November 19, 2026.

During the nine months ended September 30, 2026, no shares of common stock were purchased pursuant to the 2023 ESPP. Compensation cost of less than $0.1 million related to the 2023 ESPP was recognized during the three and nine months ended September 30, 2026. No compensation cost related to the 2023 ESPP was recognized during the three and nine months ended September 30, 2025.

11. Collaboration Agreements

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 and comprehensive loss 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, 2025, development milestones of $2.5 million had been achieved. The Company recorded $0.1 million and $1.9 million within research and development expense in the condensed consolidated statement of operations and comprehensive loss during the nine months ended September 30, 2026 and 2025, respectively, related to the Genevant Agreement. The Company recorded $0.0 million and $0.1 million within research and development expense in the condensed consolidated statement of operations and comprehensive loss during the three months ended September 30, 2026 and 2025, respectively, related to the Genevant Agreement.

Novo Nordisk Agreement

On September 13, 2024, the Company entered into a research collaboration and license agreement with Novo Nordisk A/S ("Novo Nordisk"), pursuant to which the Company granted Novo Nordisk an exclusive worldwide license under certain intellectual property rights to research, develop, manufacture, commercialize or otherwise exploit certain licensed compounds and licensed products for an initial target in the cardiometabolic field and for a second target (to be nominated by Novo Nordisk within a specified time period as set forth in the agreement). Under the agreement, the Company is responsible for certain research and development activities with respect to licensed compounds and licensed products directed against the initial target and the second target (if nominated by Novo Nordisk), and the Company is eligible to receive cost reimbursement from Novo Nordisk for the Company's performance of such research and development activities under the agreement with respect to such target(s). Novo Nordisk may undertake subsequent worldwide development, manufacturing, marketing and commercialization of the licensed products directed against the initial target and the second target (if applicable). For additional information relating to the terms of such agreement, see Note 12 in the audited consolidated financial statements included in the 2025 Form 10-K.

In October 2024, the Company received an upfront nonrefundable payment of $10.0 million from Novo Nordisk related to the first product candidate, or program target. The Company is entitled to an additional upfront nonrefundable payment from Novo Nordisk upon the selection of the second program target as well as related cost reimbursements.

The Company assessed this arrangement in accordance with ASC *Topic 606, Revenue from Contracts with Customers,* and concluded that the contract counterparty, Novo Nordisk, is a customer. The Company determined that the research services and the exclusive licenses granted under the collaboration agreement for the initial target are not distinct and, therefore, the Company accounts for the research services and exclusive licenses as a single performance obligation. Additionally, the Company concluded that Novo Nordisk's option to select a second target does not give rise to a material right or performance obligation until the additional target is selected due to the additional services being priced at standalone selling price. The Company recognizes revenue related to the

combined license and research services performance obligation over time using the input method. Under the input method, the extent of progress towards completion is measured based on the ratio of costs incurred to date to the total estimated costs at completion of the performance obligation, which the Company believes best measures its progress towards satisfying the combined performance obligation. A cost-based input method of revenue recognition requires management to make estimates of costs to complete the performance obligation. In making such estimates, judgment is required to evaluate assumptions related to cost estimates.

Effective November 11, 2025, the Company and Novo Nordisk entered into an amendment to the research collaboration and license agreement under which the Company and Novo Nordisk agreed to pause the research collaboration and license agreement for 12 months (the "hold period"), effective as of the date of the amendment. During the hold period, the parties agreed that all research and development activities and corresponding obligations under the agreement will be suspended without any liability or payment obligation for either party. The Company also agreed to promptly wind-down its research and development activities in connection with the license agreement and Novo Nordisk agreed to reimburse the Company for certain wind-down costs associated with the hold period. All wind-down activities were completed and reimbursed as of March 31, 2026.

As of September 30, 2026, the total transaction price was determined to be $40.0 million based on the $10.0 million upfront nonrefundable payment and $30.0 million of estimated research services for the first program target. During the nine months ended September 30, 2026 and 2025, the Company recognized total collaboration revenue of $0.0 million and $5.1 million, respectively. During the three months ended September 30, 2026 and 2025, the Company recognized total collaboration revenue of $0.0 million and $1.1 million, respectively. In November 2025, the Company agreed to a 12-month pause on such collaboration with Novo Nordisk, and accordingly, prior to receipt of the termination notice described below, the Company expected to recognize revenue through 2028.

On November 3, 2026, Novo Nordisk delivered written notice of its election to terminate the collaboration agreement in its entirety for convenience, as the agreement permits on 90 days' prior written notice. The termination will take effect on February 1, 2027, at which time the licenses granted to Novo Nordisk under the agreement will terminate. Novo Nordisk will reimburse the Company for certain third-party wind-down costs and non-cancellable committed costs attributable to the termination. The upfront payment and other amounts previously received are non-refundable. The termination notice is a nonrecognized subsequent event, and no adjustments have been made to the condensed consolidated financial statements as of and for the three and nine months ended September 30, 2026.

The Company had $3.0 million of current deferred revenue and $4.8 million of non-current deferred revenue as of September 30, 2026, based on the period the services were expected to be performed and/or related costs to be incurred, which was the Company's best estimate as of September 30, 2026, prior to receipt of the termination notice. As the Company has no remaining obligation to perform research services under the agreement, the Company expects to recognize the remaining deferred revenue under the agreement of approximately $7.8 million in the fourth quarter of 2026. See Note 17, Subsequent Events.

No milestones were achieved under the agreement and, as a result of the termination, the Company will not receive any milestone payments or royalties.

12. Commitments and Contingencies

Leases

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 full amount of the $13.1 million tenant improvement allowance as of December 31, 2024. The lease has a remaining term of approximately eight 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.

The maturity of undiscounted payments due under lease liabilities and the present value of those liabilities as of September 30, 2026 were as follows (in thousands):

	As of September 30, 2026
Remainder of 2026	$ 1,848
2027	7,557
2028	7,778
2029	8,007
2030	8,242
Thereafter	28,471
Total Future Minimum Lease Payments	61,903
Less: Interest	(20,380)
Present Value of Operating Lease Liabilities	$ 41,523

During the fourth quarter of 2025, the Company recorded a non-cash, long-lived asset impairment charge of $15.0 million related to its operating right-of-use asset. For additional information relating to the charge, see Note 3 in the audited consolidated financial statements included in the 2025 Form 10-K.

As of September 30, 2026, the weighted average remaining lease term was 7.6 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 condensed consolidated statement of operations and comprehensive loss of its operating leases (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating lease costs	$ 1,386	$ 1,411	$ 4,200	$ 4,234
Variable lease costs	493	417	1,504	1,323
Short-term lease costs	171	203	570	713
Total lease costs	$ 2,050	$ 2,031	$ 6,274	$ 6,270

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.

The Company was not subject to any material legal proceedings or claims as of September 30, 2026.

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.

13. 401(k) Savings Plan

The Company has a defined-contribution savings plan (the "401(k) Plan") under Section 401(k) of the Internal Revenue Code of 1986, as amended (the "IRC"). 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. Effective in 2025, the Company makes an employer non-elective safe harbor contribution of 3% of the participant's salary, subject to employer match limitations under the IRC. As such, the Company made $0.3 million and $0.4 million of contributions to the 401(k) Plan during the nine months ended September 30, 2026 and 2025, respectively.

14. Net Loss per Share

The 4,148,836 shares underlying the pre-funded warrants are included in the calculation of basic and diluted net loss per share as of September 30, 2026, as the exercise price of $0.001 per share is not substantive and the shares are issuable for little or no consideration. Shares held in treasury are excluded from the weighted-average number of shares outstanding. No pre-funded warrants were outstanding as of September 30, 2025. The following common stock equivalents have been excluded from the calculation of diluted net loss per share because their effect would be anti-dilutive:

	Nine Months Ended September 30,	
	2026	2025
Outstanding stock options	1,894,911	1,795,493
Unvested restricted stock unit awards	442,745	—
Unvested performance stock unit awards	70,000	—
Outstanding warrant	8,049	8,049
Total	2,415,705	1,803,542

15. Segment Information

The Company operates and manages its business as one reportable segment and one operating segment, which is the business of discovering, developing and commercializing therapies derived from or incorporating its RNA-editing technology. The Company's chief operating decision maker ("CODM") is the chief executive officer.

The CODM assesses performance, monitors budget versus actual results, and decides how to allocate resources based on net loss that also is reported on the consolidated statements of operations and comprehensive loss. The CODM allocates resources based on the Company's available cash resources, forecasted cash flow, and expenditures on a consolidated basis, as well as an assessment of the probability of success of its research and development activities. Resource allocation decisions are informed by budgeted and forecasted expense information, along with actual expenses incurred to date. The accounting policies of the Company's single reportable segment are the same as those described in the summary of significant accounting policies. The level of disaggregation and amounts of significant segment expenses that are regularly provided to the CODM are the same as those presented in the consolidated statements of operations and comprehensive loss. Therefore, duplication of the Company's consolidated statements of operations and comprehensive loss in this segment disclosure is not required.

The measure of segment assets is reported on the consolidated balance sheets as total consolidated assets. All long-lived assets are located in the United States. Long-lived assets primarily consist of property and equipment, net, operating lease right-of-use assets and long-term marketable securities.

Factors used in determining the reportable segment include the nature of the Company's operating activities, the organizational and reporting structure and the type of information reviewed by the CODM regularly to allocate resources and evaluate financial performance.

The Company does not have intra-entity sales or transfers.

16. Restructuring

In May 2025, the Company implemented a strategic plan to streamline its operations, including a reduction in the Company's workforce by approximately 20%. In November 2025, the Company implemented a strategic restructuring to extend cash runway, including a reduction in the Company's workforce by approximately 34%.

During the three months ended June 30, 2025, the Company recorded a one-time restructuring charge of $1.2 million related to the May 2025 workforce reduction, including employee severance, benefits and related termination costs, which are included under the caption "Restructuring charge" in the Company's consolidated statement of operations and comprehensive loss. During the year ended December 31, 2025, all activities related to the May 2025 workforce reduction were completed. During the nine months ended September 30, 2026, all activities related to the November 2025 workforce reduction were completed. Restructuring cost accruals are included under the caption "Accrued expenses and other current liabilities" in the Company's condensed consolidated balance sheet.

The following is a summary of the activity for the November 2025 Workforce Reduction:

	Employee separation costs
Balance at December 31, 2025	$ 1,893
Adjustments	26
Cash payments	(1,919)
Balance at September 30, 2026	$ —

17. Subsequent Events

On November 3, 2026, Novo Nordisk delivered written notice of its election to terminate the collaboration agreement in its entirety for convenience, effective February 1, 2027 after a contractual 90 day notice period. The termination notice is a nonrecognized subsequent event; accordingly, no adjustments were made to the condensed consolidated financial statements as of September 30, 2026. As a result, the Company expects to recognize the remaining deferred revenue under the agreement of approximately $7.8 million in the fourth quarter of 2026. See Note 11, Collaboration Agreements.

Item 2. 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 unaudited condensed consolidated financial statements and related notes included elsewhere in this Quarterly Report and the audited consolidated financial statements and related notes included in our Annual Report on Form 10-K for the year ended December 31, 2025, or the 2025 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 II, Item 1A of this Quarterly Report 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 clinical-stage biopharmaceutical company leveraging a novel oligonucleotide promoted editing of RNA, or OPERA, platform to develop a new class of genetic medicines for rare and highly prevalent diseases. OPERA provides precise, tissue-directed delivery of RNA-editing oligonucleotides, or REOs, that modify the targeted native mRNA transcript to repair or form a *de novo* protein with enhanced functionality. The platform combines a suite of capabilities consisting of sophisticated knowledge of transcription biology through ADAR proteins (Adenosine Deaminases Acting on RNA), machine learning optimization of REOs, linker chemistry expertise, along with use of a highly targeted tissue-specific delivery methodology. As such, the OPERA platform has enabled us to generate and advance a portfolio of differentiated programs that are designed to harness the body's natural RNA editing process, providing precise yet transient single base edits to produce therapeutic proteins with augmented activity versus its endogenous counterpart. 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 REO-based approach, we expect to bring our medicines to patients by leveraging our proprietary OPERA platform with precedented delivery modalities, including N-acetylgalactosamine, or GalNAc, conjugated delivery for subcutaneous administration, manufacturing know-how, and established regulatory pathways of approved oligonucleotide medicines. However, the scientific evidence to support the feasibility of developing our development 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 limited clinical data has been generated to date.

The versatility of RNA editing combined with our OPERA platform broadens the therapeutic target space significantly. 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 RNA to mimic an SNV, we believe we will be able to address unmet patient need by transiently modifying gene expression and the resultant protein function.

We continue to make meaningful advancements across our programs, including KRRO-121 as a potential first-in-class treatment for hyperammonemia that has the potential to address substantial unmet need in patients with poor ammonia control, including those with urea cycle disorders, or UCD, and hepatic encephalopathy, or HE, as well as KRRO-111, our proprietary GalNAc-conjugated REO in development for the treatment of AATD.

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 development candidates. Since inception, we have funded our operations primarily through the private placement of our equity securities. To date, we have raised approximately $501.0 million of aggregate gross proceeds from sales of our equity securities.

We have incurred significant operating losses since inception. Our net losses were $56.1 million and $67.2 million for the nine months ended September 30, 2026 and 2025, respectively. We had an accumulated deficit of $439.9 million as of September 30, 2026. 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, advance development candidates through clinical development, and seek regulatory approvals for our development candidates. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our preclinical studies, initiation and conduct of any clinical trials, and our expenditures on other research and development activities, including the expansion of our pipeline.

We do not have any development 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 development candidates, if ever, and as appropriate, move pipeline candidates into the clinic and complete clinical development. If we obtain regulatory approval for our development 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 our business, results of operations and financial condition.

Program Updates and Other Recent Developments

KRRO-121

KRRO-121 is a GalNAc-conjugated REO in development for the potential treatment of hyperammonemia in patients with UCDs of any mutational background as well as patients with HE. Utilizing our proprietary OPERA platform, KRRO-121 is designed to generate a stabilized, *de novo* glutamine synthetase, or GS, protein, a critical enzyme in an alternate pathway involved in ammonia clearance. This synthetic rescue approach is designed to augment ammonia clearance in hyperammonemia-driven diseases such as UCDs and HE.

Our preclinical data support the potential for KRRO-121 to be a pan-UCD treatment that may control ammonia levels, along with the potential to enable diet liberalization, reduce dependence on nitrogen scavengers, and lower the risk of hyperammonemic crises. In preclinical studies, KRRO-121 stabilized GS protein in human induced pluripotent stem cell, or iPSC, -derived hepatocytes deficient in ornithine transcarbamylase, or OTC, and we observed similar results in iPSC-derived hepatocytes deficient in argininosuccinate synthetase 1, or ASS1. In mouse models of OTC deficiency and carbamoyl phosphate synthetase 1, or CPS-1, deficiency subjected to an ammonia challenge, a mouse-optimized surrogate oligonucleotide reduced plasma ammonia levels following the challenge relative to vehicle control. In a mouse model of argininosuccinate lyase, or ASL, deficiency, treatment with the mouse-optimized surrogate oligonucleotide increased median survival from 18 days to 23 days compared to vehicle. Results of preclinical studies may not be predictive of results in humans. Whether KRRO-121 will control ammonia levels, enable diet liberalization, reduce dependence on nitrogen scavengers or lower the risk of hyperammonemic crises in patients with UCDs, or enhance ammonia control or reduce the risk of recurrent HE episodes in patients with HE, has not been established and will depend on the results of clinical trials. KRRO-121 may permit infrequent subcutaneous dosing; however, its dosing regimen has not been established in clinical studies.

KRRO-121 is being evaluated in ANCHOR (AssessmeNt of Control of Hyperammonemia through Oligonucleotide-based RNA editing), our global, two-part Phase 1/2 clinical trial program evaluating the safety, tolerability, pharmacokinetics, or PK, and clinical activity of KRRO-121. Part 1 of ANCHOR consists of a Phase 1 single ascending dose, or SAD, study, to be followed by a Phase 1 multiple ascending dose, or MAD, study, of KRRO-121 and placebo in healthy volunteers to evaluate safety, tolerability and PK in up to 50 participants. The SAD portion is designed to enroll up to 30 healthy volunteers across up to five cohorts, and the MAD portion is designed to evaluate selected doses of KRRO-121 in up to 20 healthy volunteers across two cohorts. Both the SAD and MAD portions are placebo-controlled. Part 2 of ANCHOR is designed as an open-label Phase 2 multiple dose study in participants with UCDs who are on stable background standard of care to assess safety, tolerability, PK and clinical activity. The initiation, design and endpoints of Part 2 are subject to regulatory review and clearance to proceed.

In September 2026, we initiated Part 1 of ANCHOR and dosed the first cohort of healthy volunteers in the SAD portion with a single dose of KRRO-121 or placebo. We announced the dosing of the first cohort in October 2026. ANCHOR does not include patients with HE, and we have not initiated a clinical trial of KRRO-121 in patients with HE. Dosing of UCD patients in Part 2 of ANCHOR is expected to start in the first half of 2027, subject to regulatory clearance to proceed, with an interim clinical readout expected in the second half of 2027.

In August 2026, we announced that the European Commission granted orphan drug designation to KRRO-121 for the treatment of UCDs, and in August 2026, the U.S. Food and Drug Administration, or FDA, granted rare pediatric disease designation to KRRO-121 for the treatment of UCDs.

KRRO-111

KRRO-111 is a proprietary GalNAc-conjugated REO in development for the potential treatment of AATD. KRRO-111 is delivered subcutaneously to the liver cells, where it is engineered to co-opt the hepatocytes' endogenous ADAR enzyme and repair a pathogenic SNV on alpha-1 antitrypsin, or AAT, mRNA to restore production of the normal M variant of AAT, or M-AAT, protein while reducing synthesis of the mutant Z variant of AAT, or Z-AAT. This therapeutic profile and titratable dosing position KRRO-111 as a potential best-in-class candidate. In preclinical studies, KRRO-111, to our knowledge, demonstrated best-in-class RNA editing where over 90% of the AAT transcripts in the liver cells have been corrected, translating into approximately 90% M-AAT protein. In a repeat-dose study, KRRO-111 was administered to PiZZ mice with a loading phase followed by dosing once every two weeks. KRRO-111 demonstrated a reduction of non-inclusion Z-AAT by approximately 95% and a reduction in pre-existing aggregates by approximately 62% at day 28 with RNA editing in a mouse model of AATD. Pre-existing aggregates were progressively cleared, with

inclusion-associated Z-AAT significantly reduced by approximately 62% at day 28, consistent with autophagic clearance of accumulated protein following successful editing.

In October 2026, we announced additional preclinical data for KRRO-111. In a repeat-dose study in NSG-PiZ mice in which KRRO-111 was administered subcutaneously once weekly, SERPINA1 mRNA editing reached 96%, with M-AAT comprising 93% of total circulating AAT and total AAT concentrations reaching 172 μM (approximately six-fold relative to vehicle) at day 57. In a repeat-dose study in immunocompetent C57BL/6-PiZ mice in which KRRO-111 was administered subcutaneously once weekly, KRRO-111 resulted in robust, dose-dependent correction of SERPINA1 mRNA and M-AAT. KRRO-111 also reduced whole liver Z-AAT levels by up to 95% relative to vehicle control at day 57, as measured by liquid chromatography tandem mass spectrometry. By simultaneously increasing circulating M-AAT protein and decreasing pathogenic Z-AAT aggregates in the lung, KRRO-111 has the potential to be the best-in-class agent, improving both lung and liver manifestations of AATD. Results of preclinical studies may not be predictive of results in humans, and these potential effects of KRRO-111 have not been evaluated in humans. Regulatory submission for KRRO-111 is expected in the first half of 2027.

Collaboration with Novo Nordisk

In September 2024, we entered into a research collaboration and license agreement, or the collaboration and license agreement, with Novo Nordisk A/S, or Novo Nordisk, which we amended in November 2025 to pause the agreement for 12 months. We completed the wind-down of our research and development activities under the agreement in the first quarter of 2026. Novo Nordisk did not exercise its right to substitute the collaboration target during the hold period. We did not recognize collaboration revenue under the collaboration and license agreement during the three months ended September 30, 2026. On November 3, 2026, after the end of the quarter, we received written notice from Novo Nordisk of its election to terminate the collaboration and license agreement for convenience. The termination will be effective on February 1, 2027. For additional information, see Note 11, Collaboration Agreement and Note 17 Subsequent Events, in the condensed consolidated financial statements included in this Quarterly Report.

Financial Operations Overview

Revenue

We have not generated any revenue from product sales, and do not expect to generate any revenue from the sale of products in the near future. During the nine months ended September 30, 2026 and 2025, we recognized $0.0 million and $5.1 million of collaboration revenue, respectively, which is related to our research collaboration and license agreement with Novo Nordisk. On November 3, 2026, Novo Nordisk delivered written notice of its election to terminate the collaboration agreement in its entirety for convenience. Accordingly, we expect to recognize the remaining deferred revenue under the agreement of approximately $7.8 million in the fourth quarter of 2026.

For additional information about our revenue recognition policy and our collaboration and license agreement with Novo Nordisk, which is being terminated effective February 1, 2027, see Note 11, Collaboration Agreements, in the condensed consolidated financial statements included in this Quarterly Report.

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 development 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;
- expenses incurred under agreements with contract research organizations, or CROs, that conduct our preclinical studies and clinical trials;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical study materials, as well as supplies and materials used to manufacture clinical trial materials;
- costs of outside consultants and contractors engaged in research and development activities, including their fees and travel expenses;
- 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, clinical trials or other services performed. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. Significant judgment and estimates are made in determining the accrued expense balances and prepaid expense balances at the end of any reporting period.

A large portion of our research and development expenses have been external expenses, which we track on a program-by-program basis following nomination as a development candidate. Our internal research and development expenses are primarily personnel-related expenses, including stock-based compensation expense and facility expenses, mainly including office rent expenses and depreciation expenses. We do not allocate our internal research and development expenses to specific drug candidate programs as they are deployed across multiple projects under research and development.

The successful development of our development 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 development candidates, conduct discovery and research activities for our preclinical programs, and expand 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 development 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 development candidates to pursue and how much funding to direct to each development 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 development 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 development 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 development 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 development candidates;
- commercializing development 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 development candidates in preclinical and clinical development could mean a significant change in the costs and timing associated with the development of these development candidates. We may never succeed in achieving regulatory approval for any of our development candidates. We may obtain unexpected results from our clinical trials. We may elect to discontinue, delay or modify clinical trials of some development candidates or focus on other development candidates. For example, if the Food and Drug Administration, or FDA, European Medicines Agency, or EMA, the Human Research Ethics Committee, or HREC, Therapeutic Goods Administration, or TGA, the Health and Disability Ethics Committees, or HDEC, 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 development 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, finance, accounting, human resources and other consulting fees. General and administrative expenses also include facility costs not otherwise included in research and development expenses.

We expect to continue to incur costs associated with being a public company and maintaining controls over financial reporting, including costs of accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses.

Restructuring Charges

In May 2025, we announced a strategic plan to streamline our operations, including a workforce reduction of approximately 20%, and in November 2025 announced a further workforce reduction of approximately 34%. Restructuring charges consist primarily of severance and employee benefits costs incurred in relation to our reductions in force.

Other Income, Net

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

Results of Operations

Comparison of the Three and Nine Months Ended September 30, 2026 and 2025

The following table summarizes our results of operations for the three and nine months ended September 30, 2026 and 2025:

	Three Months Ended September 30,			Nine Months Ended September 30,		
(in thousands)	2026	2025	Change	2026	2025	Change
Collaboration revenue	$ —	$ 1,090	$ (1,090)	$ —	$ 5,100	$ (5,100)
Operating expenses:						
Research and development	12,104	13,820	(1,716)	38,643	53,694	(15,051)
General and administrative	6,352	6,507	(155)	20,359	21,632	(1,273)
Restructuring charge	—	—	—	—	1,233	(1,233)
Total operating expenses	18,456	20,327	(1,871)	59,002	76,559	(17,557)
Loss from operations	(18,456)	(19,237)	781	(59,002)	(71,459)	12,457
Other income, net						
Other income, net	1,168	1,176	(8)	3,376	4,242	(866)
Total other income, net	1,168	1,176	(8)	3,376	4,242	(866)
Loss before provision for income taxes	(17,288)	(18,061)	773	(55,626)	(67,217)	11,591
Provision for income taxes	(260)	—	(260)	(463)	(1)	(462)
Net loss	$ (17,548)	$ (18,061)	$ 513	$ (56,089)	$ (67,218)	$ 11,129

Collaboration Revenue

During the three months ended September 30, 2026 and 2025, we recognized $0.0 million and $1.1 million, respectively, of collaboration revenue from our research collaboration and license agreement with Novo Nordisk. During the nine months ended September 30, 2026 and 2025, we recognized $0.0 million and $5.1 million, respectively, of collaboration revenue from our research collaboration and license agreement with Novo Nordisk. On November 3, 2026, Novo Nordisk delivered written notice of its election to terminate the collaboration agreement in its entirety for convenience. Accordingly, we expect to recognize the remaining deferred revenue under the agreement of approximately $7.8 million in the fourth quarter of 2026.

Research and Development Expenses

The following table summarizes our research and development expenses for the three and nine months ended September 30, 2026 and 2025:

(in thousands)	Three Months Ended September 30, 2026	2025	Change	Nine Months Ended September 30, 2026	2025	Change
Development candidate expenses						
KRRO-110 (AATD) external expenses	$ 130	$ 3,708	$ (3,578)	$ 1,192	$ 16,265	$ (15,073)
KRRO-111 (AATD) external expenses	1,220	138	1,082	3,462	138	3,324
KRRO-121 (UCD) external expenses	2,161	304	1,857	9,529	2,788	6,741
Unallocated research and development expenses						
Other research and pre-development candidate expenses	2,209	1,873	336	5,853	9,084	(3,231)
Personnel expenses	4,442	5,241	(799)	12,773	17,363	(4,590)
Facilities expenses	1,942	2,556	(614)	5,834	8,056	(2,222)
Total research and development expenses	$ 12,104	$ 13,820	$ (1,716)	$ 38,643	$ 53,694	$ (15,051)

Research and development expenses were $12.1 million for the three months ended September 30, 2026, compared to $13.8 million for the three months ended September 30, 2025. The decrease was primarily due to the following:

- a $3.6 million decrease in KRRO-110 external expenses primarily due to the termination of the Phase 1/2a REWRITE clinical trial during the first quarter of 2026; and
- a $0.8 million decrease in personnel-related expenses, primarily due to a decrease in headcount resulting from the May and November 2025 workforce reductions, partially offset by an increase in stock-based compensation costs; and
- a $0.6 million decrease in facilities expenses primarily due to a decrease in depreciation expense attributable to the impairment charge recorded during the fourth quarter of 2025;
- offset by a $1.9 million increase in KRRO-121 external expenses, primarily due to an increase in preclinical, manufacturing, and clinical costs for the Phase 1/2 ANCHOR clinical trial; and
- a $1.1 million increase in KRRO-111 external expenses, primarily due to an increase in preclinical and manufacturing costs; and
- a $0.3 million increase in other research and pre-development candidate expenses.

Research and development expenses were $38.6 million for the nine months ended September 30, 2026, compared to $53.7 million for the nine months ended September 30, 2025. The decrease was primarily due to the following:

- a $15.1 million decrease in KRRO-110 external expenses primarily due to the termination of the Phase 1/2a REWRITE clinical trial during the first quarter of 2026; and
- a $4.6 million decrease in personnel-related expenses, primarily due to a decrease in headcount resulting from the May and November 2025 workforce reductions, partially offset by an increase in stock-based compensation costs; and
- a $3.2 million decrease in other research and pre-development candidate expenses primarily due to a decrease in lab supplies and consumables; and
- a $2.2 million decrease in facilities expenses primarily due to a decrease in depreciation expense attributable to the impairment charge recorded during the fourth quarter of 2025;
- offset by a $6.7 million increase in KRRO-121 external expenses, primarily due to an increase in preclinical, manufacturing, and clinical costs for the Phase 1/2 ANCHOR clinical trial; and
- a $3.3 million increase in KRRO-111 external expenses, primarily due to an increase in preclinical and manufacturing costs.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the three and nine months ended September 30, 2026 and 2025:

(in thousands)	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Personnel-related expenses	$ 3,635	$ 3,348	$ 287	$ 11,618	$ 11,061	$ 557
Professional services	1,616	1,523	93	5,009	5,152	(143)
Facilities expenses	583	765	(182)	1,900	2,455	(555)
Other	518	871	(353)	1,832	2,964	(1,132)
Total general and administrative expenses	$ 6,352	$ 6,507	$ (155)	$ 20,359	$ 21,632	$ (1,273)

General and administrative expenses were $6.4 million for the three months ended September 30, 2026, compared to $6.5 million for the three months ended September 30, 2025. The decrease was primarily due to the following:

- a $0.4 million decrease in other expenses, primarily due to a decrease in information technology expenses; and
- a $0.2 million decrease in facilities expenses primarily due to a decrease in depreciation expense attributable to the impairment charge recorded during the fourth quarter of 2025;
- offset by a $0.3 million increase in personnel expenses mainly due to increased stock-based compensation costs, partially offset by a decrease in headcount resulting from the May and November 2025 workforce reductions; and
- a $0.1 million increase in professional services.

General and administrative expenses were $20.4 million for the nine months ended September 30, 2026, compared to $21.6 million for the nine months ended September 30, 2025. The decrease was primarily due to the following:

- a $1.1 million decrease in other expenses, primarily due to a decrease in information technology expenses; and
- a $0.6 million decrease in facilities expenses primarily due to a decrease in depreciation expense attributable to the impairment charge recorded during the fourth quarter of 2025; and
- a $0.1 million decrease in professional services;
- offset by a $0.6 million increase in personnel expenses mainly due to increased stock-based compensation costs, partially offset by a decrease in headcount resulting from the May and November 2025 workforce reductions.

Restructuring Charges

Restructuring charges for the nine months ended September 30, 2025 consisted of $1.2 million of employee termination benefits related to our workforce reduction in May 2025. We did not incur any restructuring charges for the three months ended September 30, 2025. During the year ended December 31, 2025, all activities related to our May 2025 workforce reduction were completed. We did not incur any restructuring charges for the three and nine months ended September 30, 2026.

Other Income, Net

Total other income, net was $1.2 million for the three months ended September 30, 2026 and 2025.

Total other income, net was $3.4 million for the nine months ended September 30, 2026, compared to $4.2 million for the nine months ended September 30, 2025. The decrease of $0.9 million was primarily due to lower interest income generated from a lower cash, cash equivalents and marketable securities balance during the year prior to our $85.0 million March 2026 private placement, or the March 2026 PIPE, and fluctuations in foreign currency exchange rates.

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 $501.0

million of aggregate gross proceeds from sales of our equity securities. As of September 30, 2026, we had cash, cash equivalents and marketable securities of $124.8 million.

Since inception, we have incurred significant operating losses and, as of September 30, 2026, had an accumulated deficit of $439.9 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.

In December 2024, we entered into a sales agreement with TD Cowen Securities (USA) LLC, or TD Cowen, under which we may, from time to time, issue and sell shares of our common stock having aggregate sales proceeds of up to $100.0 million, in a series of one or more at-the-market equity, or ATM, offerings. In January 2026, we issued 501,861 shares of common stock under the ATM offering program for gross proceeds of $5.1 million.

In March 2026, we closed the March 2026 PIPE selling (i) an aggregate of 4,501,928 shares of our common stock at a purchase price of $11.11 per share and (ii) pre-funded warrants to acquire an aggregate 3,148,836 shares of our common stock (at an exercise price of $0.001 per share) at a purchase price of $11.109 per pre-funded warrant, which resulted in gross cash proceeds of approximately $85.0 million, before deducting placement agent fees and offering expenses of approximately $4.7 million.

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $124.8 million. We expect that our cash, cash equivalents and marketable securities outstanding as of September 30, 2026 will be sufficient to fund our operating expenses and capital expenditure requirements into the second half of 2028. 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 development candidates and pursue commercialization of any approved development 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 development 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 scope, progress, results, and costs of discovery, preclinical development, laboratory testing, manufacturing and clinical trials for KRRO-121, KRRO-111, and other development candidates we may develop;
- the cost of continuing to build our OPERA platform and discover additional novel genetic medicines;
- the extent to which we partner our programs, acquire or in-license other lead candidates, development 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;
- any future headcount growth and associated costs, should 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 lead candidates or development candidates;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any development 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 development 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 development candidates even if we would otherwise prefer to develop and market such development candidates ourselves.

Cash Flows

Comparison of the Nine Months Ended September 30, 2026 and 2025

The following table summarizes our cash flows for the nine months ended September 30, 2026 and 2025:

	Nine Months Ended September 30,	
(in thousands)	**2026**	**2025**
Net cash used in operating activities	$ (46,003)	$ (60,859)
Net cash (used in) provided by investing activities	(21,881)	29,077
Net cash provided by financing activities	85,295	305
Effect of foreign exchange rate changes	(17)	(11)
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 17,394	$ (31,488)

Cash Used in Operating Activities

Net cash used in operating activities was $46.0 million for the nine months ended September 30, 2026, primarily resulting from our net loss of $56.1 million, which was primarily attributable to our research and development activities and our general and administrative expenses offset by $10.1 million of non-cash items.

Net cash used in operating activities was $60.9 million for the nine months ended September 30, 2025, primarily resulting from our net loss of $67.2 million, which was primarily attributable to our research and development activities and our general and administrative expenses, along with changes in our operating assets and liabilities of $3.0 million, offset by $9.3 million of non-cash items.

Cash (Used in) Provided by Investing Activities

Net cash used in investing activities was $21.9 million for the nine months ended September 30, 2026 and consisted of the purchase of marketable securities and the purchase of property and equipment, offset by proceeds from maturities of marketable securities.

Net cash provided by investing activities was $29.1 million for the nine months ended September 30, 2025 and consisted primarily of the proceeds from maturities of marketable securities, offset by purchase of marketable securities and the purchase of property and equipment.

Cash Provided by Financing Activities

Net cash provided by financing activities was $85.3 million for the nine months ended September 30, 2026 and consisted of net proceeds from the March 2026 PIPE, sales of shares under our ATM program, and exercises of stock options.

Net cash provided by financing activities was $0.3 million for the nine months ended September 30, 2025 and consisted of proceeds from exercises of stock options.

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. See the 2025 10-K for more information about our critical accounting policies as well as a description of our other significant accounting policies.

There have been no material changes to our critical accounting estimates from those described in Part II, Item 7. *"Management's Discussion and Analysis of Financial Condition and Results of Operations"* included in the 2025 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 condensed consolidated financial statements.

Smaller Reporting Company Status

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 3. Quantitative and Qualitative Disclosures About Market Risk.

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $124.8 million, which consist of bank deposits, money market funds and U.S. Treasury and other government-backed securities. 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 we do not have a material exposure to foreign currency risk.

Item 4. 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, who serves as our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2026. Based on such evaluation, our Chief Executive Officer has concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of September 30, 2026.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) 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.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this Quarterly Report, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors.

You should consider carefully the risks and uncertainties described below, together with all of the other information in this Quarterly Report 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 Quarterly Report 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 Quarterly Report. See "Cautionary Statement Regarding Forward Looking Statements."

Risks Related to Our Business

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 $56.1 million and $67.2 million for the nine months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, we had an accumulated deficit of $439.9 million. We have financed our operations primarily through private placements of our convertible preferred stock and common stock, and sales under our ATM offering program. 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, and recently, a $30.9 million impairment charge in the fourth quarter of 2025 related to our operating lease right-of-use asset and fixed assets. 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:

- progress KRRO-121 through our ongoing global two-part Phase 1/2 clinical trial program, or ANCHOR, and any subsequent clinical development, and advance KRRO-111 into and through clinical development;
- continue current research programs and preclinical and clinical development of any lead candidates or development candidates we may identify;
- seek to identify additional research programs and lead candidates, and nominate development candidates;
- further develop OPERA, our RNA editing platform;
- maintain, expand, enforce, defend and protect our intellectual property portfolio;
- seek marketing approvals for any development candidates that successfully complete clinical trials;
- develop, maintain and enhance a sustainable, scalable, reproducible and transferable manufacturing process for the candidates we may develop;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- hire additional personnel as we grow our business;
- acquire or in-license lead candidates, development candidates, intellectual property and technologies;
- establish and maintain collaborations;
- should we decide to do so, build and maintain a commercial-scale current good manufacturing practices, or cGMP, manufacturing facility; and
- operate as a public company.

We expect that it will be many years, if ever, before we have an RNA editing product ready for commercialization. To become and remain profitable, we must develop and, either directly or through collaborators, eventually commercialize a product or products with market potential. This will require us to be successful in a range of challenging activities, including identifying lead candidates, completing preclinical studies and clinical trials of development candidates, obtaining marketing approval for these development candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we are, may never generate revenues that are significant or large enough to achieve profitability.

In November 2025, we announced that we did not reach projected levels of protein in patients in our Phase 1/2a REWRITE clinical trial of KRRO-110 for AATD (which we subsequently terminated). KRRO-121 only recently entered clinical development, and our other programs, including KRRO-111, are in preclinical or earlier stages of development. In addition, we undertook workforce reductions in May 2025 and November 2025, and in the fourth quarter of 2025, as a result of identified indicators of impairment for our long-lived assets, primarily including our operating lease right-of-use, or ROU, asset, and property and equipment, we recognized a non-cash, long-lived asset impairment charge of $30.9 million. Because of the numerous risks and uncertainties associated with developing lead candidates and development candidates, and the risks associated with conducting preclinical studies and clinical trials, 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. Further, we may not even experience the expected benefits of our May 2025 and November 2025 workforce reductions or our decision to discontinue clinical development of KRRO-110. Further, the long-lived asset impairment charges recognized may affect how investors, analysts, and counterparties assess our financial position and prospects, potentially making it more difficult or expensive to raise capital or enter into collaborations on favorable terms.

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, 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 lead candidates and successfully complete research and development of such lead candidates;
- seek and obtain regulatory and marketing approvals for any development candidates for which we complete clinical trials;
- launch and commercialize any development 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 development 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 development candidates for which we obtain regulatory and marketing approval;
- develop, maintain and enhance a sustainable, scalable, reproducible and transferable manufacturing process for the candidates we may develop;
- address competing technological and market developments;
- negotiate favorable terms in any existing or future collaboration, licensing or other arrangements and perform 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 FDA, the EMA, a Human Research Ethics Committee in Australia, or HREC, the Australian Therapeutic Goods Administration, or TGA, 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 candidates we may develop are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved development 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 development 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, our lead candidates and development candidates. Because we have limited financial and managerial resources, we have prioritized our research programs and REO 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 lead candidates or development candidates or for other indications that later prove to have greater clinical or commercial potential and we have in the past reprioritized, and may need to further reprioritize, our focus. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. For example, KRRO-110, which used an LNP delivery system, did not reach projected levels of protein in patients in our now terminated Phase 1/2a REWRITE clinical trial for AATD and we have since transitioned to a GalNAc-conjugated delivery system for our AATD program and are now developing KRRO-111, a GalNAc-conjugated REO for AATD. Our spending on current and future research and development programs, lead candidates, and development candidates for specific indications may not yield any commercially viable products.

In addition, if we obtain marketing approval for any 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 continue 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 September 30, 2026, our cash, cash equivalents and marketable securities were $124.8 million, excluding restricted cash, or $127.2 million, including restricted cash. We believe our existing cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses and capital expenditure requirements through several value-creating milestones and into the second half of 2028. 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 development candidates. We cannot be certain that additional funding will be available on acceptable terms or at all. Although we have an effective shelf registration statement and an at-the-market offering program, we have no committed source of additional capital. 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 development candidates or other research and development initiatives. We could be required to seek collaborators for potential lead 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 lead candidates on unfavorable terms in certain markets where we otherwise would seek to pursue development or commercialization ourselves.

We have experienced, and may continue to experience, delays in developing a potential treatment for AATD. We have not completed any clinical trial of any of our development candidates and any favorable results we may have from our earlier preclinical studies, including those of KRRO-121 and KRRO-111, 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 development candidates using our proprietary RNA editing technology is both preliminary and limited. We dosed very few participants in our now terminated Phase 1/2a REWRITE clinical trial of KRRO-110 for AATD. Although we observed functional protein in AATD patients following a single administration of KRRO-110, we did not reach projected levels of protein in patients as of the data cut off date. We have not completed any clinical trials for any of our proposed delivery methods or RNA editing approaches, and we have transitioned from LNP to GalNAc-conjugated delivery for KRRO-111, our REO for our AATD program. Accordingly, we have experienced, and may continue to experience, delays in developing a potential treatment for AATD. KRRO-121 has been administered to only a limited number of healthy volunteers, and

we have not yet generated clinical data sufficient to evaluate its safety, tolerability, pharmacokinetics, or PK, or clinical activity. In the future, we may use other delivery modalities to deliver our other lead candidates. While LNPs have been validated clinically to deliver oligonucleotides, such as siRNA, they were not clinically proven to deliver oligonucleotides for RNA editing, such as KRRO-110 and our other lead candidates. Similarly, while GalNAc-conjugated delivery has been validated in multiple FDA-approved oligonucleotide therapeutics, there are no FDA-approved GalNAc-conjugated REO therapeutics. Both of our most advanced programs, KRRO-121 and KRRO-111, utilize GalNAc-conjugated delivery. If GalNAc-conjugated delivery fails to effectively deliver our REOs, multiple programs in our pipeline could be adversely affected.

Although KRRO-121 differs from KRRO-110 in its delivery approach (GalNAc conjugation rather than LNP), its intended mechanism (generating a stabilized, de novo GS protein rather than repairing a pathogenic variant) and its target indications, KRRO-110 is the only development candidate generated from our OPERA platform for which we have reported clinical data. Those data showed that, despite our preclinical results, the levels of protein achieved in patients were lower than we had projected. There is a risk that editing efficiency and protein levels observed in cell-based assays and animal models may not translate to humans. We cannot assure you that KRRO-121, KRRO-111 or our other development candidates will not encounter similar or other translational challenges. Because the KRRO-110 data reflect a different delivery approach and mechanism, they provide limited information regarding the potential safety or activity of KRRO-121 in humans.

In addition, our proprietary 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. The design of a clinical trial can determine whether its results will support approval of a development 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 development candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their development candidates. Many lead 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. Accordingly, any favorable results we may have from our earlier preclinical studies may not be predictive of results that may be observed in further preclinical studies or clinical trials.

If preclinical studies or clinical trials of any lead candidate or development candidate we may identify 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 development candidate.

Before obtaining marketing approval from regulatory authorities for the sale of any development candidates we may identify and develop, we must complete preclinical development and then conduct extensive clinical trials to demonstrate safety and efficacy in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete, and the outcome is uncertain. A failure of one or more clinical trials can occur at any stage of development. 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 lead candidates or development candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed, preventing marketing approval of their development candidates. For example, despite our preclinical data, we did not reach projected levels of protein in AATD patients in our REWRITE Phase 1/2a clinical trial.

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 development candidates we may identify and develop, including:

- delays in reaching a consensus with regulators on trial design;
- regulators, institutional review boards, or 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 development 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 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 such as UCD, 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 development 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 development candidates may be greater than we anticipate;
- the supply or quality of any development candidates or other materials necessary to conduct clinical trials of any development candidates may be insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing, and delivery of any development candidates 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 development candidates 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 development candidates beyond those that we currently contemplate, if we or our collaborators are unable to successfully complete clinical trials or other testing of any development candidates, 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 development candidates or not obtain marketing approval at all;
- choose to, or be required to, revise or transition strategy for our development candidates or protocols;
- 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 ANCHOR or any other any clinical trials will begin or proceed 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 development 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 development candidates we may develop, any of which may harm our business, financial condition, results of operations, and prospects.

Our business is substantially dependent on the success of KRRO-121, our lead development candidate, and if KRRO-121 fails to advance successfully, our business would be materially and adversely affected.

We have concentrated a significant portion of our current resources and efforts on the advancement of KRRO-121, our lead development candidate for the treatment of hyperammonemia, including UCD and HE. KRRO-121 utilizes GalNAc-conjugated delivery and is designed to generate a stabilized, de novo variant of GS with enhanced ammonia clearance capacity. KRRO-121 has been administered to only a limited number of healthy volunteers and has not been administered to any patient with UCD or HE. There can be no assurance that KRRO-121 will demonstrate safety or efficacy in humans, that we will obtain the regulatory and ethics approvals necessary to initiate Part 2 of ANCHOR or any other clinical trial of KRRO-121, including in HE, or that we will be able to enroll, conduct and complete ANCHOR or report data on our anticipated timelines. If KRRO-121 fails to demonstrate safety or efficacy, or if we experience significant delays in its clinical development, our business, financial condition, results of operations and prospects would be materially and adversely affected. Following the termination of our REWRITE clinical program investigating KRRO-110 for AATD, we only recently nominated KRRO-111, our GalNAc-conjugated REO for AATD, which is in preclinical development, including ongoing GLP toxicology studies. Our other programs, including our longevity and liver health program targeting AMPKγ1, and our ALS program targeting TDP-43, are in earlier stages of preclinical development and may not advance to clinical development on a timely basis, or at all.

ANCHOR is the first clinical trial of KRRO-121 and of any development candidate designed to generate a de novo protein using our OPERA platform. The design of ANCHOR, including its initial evaluation of KRRO-121 in healthy volunteers and its planned evaluation in a small number of patients with UCD, may not generate data sufficient to support further development of, or regulatory approval for, KRRO-121.

Part 1 of ANCHOR trial of KRRO-121 is a first-in-human trial in healthy volunteers. Because healthy volunteers do not have hyperammonemia, Part 1 is not expected to provide meaningful information about whether KRRO-121 can lower ammonia levels in patients. In addition, the safety, tolerability and PK profile of KRRO-121 observed in healthy volunteers may not be representative of its profile in patients with UCD, who may be receiving nitrogen scavengers and other therapies and adhering to protein-restricted diets, or in patients with HE, who generally have cirrhosis and impaired liver function. If the data from Part 1 do not support the selection of an appropriate dose or dosing regimen, or if dose escalation must be slowed, modified or stopped because of safety findings or PK results, the initiation of Part 2 could be delayed or we may need to modify our development plans for KRRO-121.

Part 2 of ANCHOR is designed to enroll patients with UCD across UCD genotypes who will remain on their background standard of care. The small number of patients, the absence of a control arm and the genetic and clinical heterogeneity of UCD may make the results of Part 2 difficult to interpret, and favorable results in some UCD subtypes may not be observed in others. Measures of clinical activity in UCD, such as plasma ammonia levels, can fluctuate substantially as a result of diet, intercurrent illness, adherence to background therapy and other factors unrelated to treatment. Although we believe our preclinical data support the potential for KRRO-121 to be a treatment for UCD of any mutational background and to enable diet liberalization, reduce dependence on nitrogen scavengers and lower the risk of hyperammonemic crises, or HACs, none of these potential benefits has been evaluated in humans, and ANCHOR is not designed to establish them. Even if the results of ANCHOR are favorable, the FDA or comparable foreign regulatory authorities may not agree that they are sufficient to support the initiation of a pivotal trial, may require additional or larger clinical trials, including randomized, controlled trials, or may require clinical outcome endpoints, any of which would make the development of KRRO-121 longer and more costly than we anticipate.

We have not initiated any clinical trial of KRRO-121 in patients with HE, and the development and regulatory pathway for KRRO-121 in HE is uncertain and is likely to require larger, longer and more costly clinical trials than in UCD.

Although we believe KRRO-121 has the potential to enhance ammonia control in patients with HE, ANCHOR does not include patients with HE, and we have not yet sought regulatory approval to initiate a clinical trial of KRRO-121 in HE. HE is a complication of advanced liver disease, most commonly cirrhosis, and patients with HE typically have significant comorbidities and impaired hepatic function, which may affect the safety, tolerability, PK and activity of KRRO-121 in this population. In particular, KRRO-121 relies on GalNAc-mediated uptake into hepatocytes through the asialoglycoprotein receptor, and the expression or function of this receptor, and the number of functional hepatocytes, may be reduced in patients with advanced liver disease, which could reduce the delivery or activity of KRRO-121 in patients with HE. Regulatory authorities may require us to conduct additional studies, such as studies in subjects with hepatic impairment, before or in connection with any clinical trial in HE. In addition, unlike UCD, HE affects a substantially larger patient population, may not qualify for orphan drug designation, and is treated with established therapies. As a result, we expect that any registrational program for KRRO-121 in HE would require larger, longer and more costly clinical trials than a program in UCD, potentially including trials designed to demonstrate a reduction in HE episodes or hospitalizations. We may not have sufficient resources to conduct such trials without a collaborator, and we may be unable to enter into a collaboration on acceptable terms or at all. Any of these factors could delay or prevent the development of KRRO-121 for HE, which would limit its commercial potential.

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

Clinical trials of a new development candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease the development candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, including 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, 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, among others. Clinical trials can also be impacted by other events unrelated to our business, such as geopolitical events and unrest, such as recent hostilities involving Iran, global pandemics, or economic uncertainty, including imposition of tariffs that impact the supply chain, or disruptions due to government shutdowns, which can impact regulatory reviews. In April 2025, the United States imposed tariffs on imports on its trading partners and tariff policy (and trading partners subject to such tariffs) has continued to evolve. Historically, tariffs have led to increased costs, political and trade tensions, which negatively affect global supply chains. These types of events can disrupt clinical trial sites and delay patient enrollment, all of which would have a negative impact on our business and ability to obtain regulatory approval. 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.

These risks are heightened for Part 2 of ANCHOR and any future clinical trials of KRRO-121 in UCD. UCDs are rare diseases, and the number of patients who meet the eligibility criteria for our trials is limited. Patients with UCD are managed at specialized metabolic centers, which may limit the number of clinical trial sites with access to eligible patients. Patients with UCD may also be geographically dispersed and may be eligible to participate in clinical trials of other investigational therapies for UCD. Patients whose disease is managed with existing therapies and dietary restrictions, and their physicians, may be reluctant to enroll in a trial of a novel investigational therapy, and the requirement that patients remain on stable background therapy, or other eligibility criteria, may further limit the pool of eligible patients. We will also need to activate clinical trial sites with access to patients with UCD, and obtain the required regulatory and ethics approvals for those sites. If we are unable to enroll a sufficient number of patients with UCD in a timely manner, the initiation or completion of Part 2 of ANCHOR, and our anticipated interim clinical readout in the second half of 2027, could be delayed.

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 any candidates. It will be many years before we or our collaborators commercialize a development candidate or generate any revenues, if ever. Additionally, other genetic medicine 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 development 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. We currently only have two nominated development candidates in our pipeline, KRRO-121, which only recently entered clinical development, and KRRO-111, which is in preclinical development, as we terminated further development of KRRO-110 after it failed to reach projected levels of protein in our first in-human clinical trial. 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 any candidates we successfully develop. 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.

Other than KRRO-121, which is in Part 1 of ANCHOR, all of our product development programs are still in the research or preclinical stage of development. Our research methodology may be unsuccessful in identifying lead candidates, our development 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 development candidates impractical to manufacture, unmarketable, or unlikely to receive marketing approval.

The pharmacological properties ascribed to the lead 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 (for example, KRRO-110 did not achieve projected levels of protein in patients in our REWRITE Phase 1/2a clinical trial). If our development 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 development 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 development 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 development candidates beyond the initial candidates we may develop, we have not yet successfully developed any candidate and our ability to expand our portfolio may never materialize.

RNA editing is a novel technology with limited clinical validation for human therapeutic use. The approaches we take to discover and develop novel therapeutics, including the oligonucleotide chemistries we use in our REOs, are unproven , may result in unexpected clinical outcomes or manufacturing challenges and may never lead to marketable products.

We are focused on developing products 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 completed clinical development. The scientific evidence to support the feasibility of developing development candidates based on these technologies is both preliminary and limited. Successful development of development candidates by us will require solving a number of issues, including optimizing the efficiency and specificity of such development candidates, and ensuring the therapeutic selectivity of such development candidates. There can be no assurance we will be successful in solving any or all of these issues.

The REOs we design using OPERA incorporate chemical modifications, including chemistries that have been used in other classes of oligonucleotide therapeutics, such as antisense oligonucleotides, or ASOs, and siRNA, as well as novel chemistries that we have developed through OPERA. The chemistries used in ASOs and siRNAs were developed and characterized for mechanisms of action that differ from that of our REOs, which are designed to bind to target RNA and recruit endogenous ADAR enzymes to edit that RNA, and there is limited experience with the use of these chemistries in RNA editing oligonucleotides. As a result, the safety, tolerability, PK, activity and other characteristics observed with these chemistries in ASOs, siRNAs or other oligonucleotide therapeutics may not be predictive of how they will perform in our REOs, and their use in our REOs may result in unexpected clinical outcomes. The novel chemistries we have developed through OPERA have limited or no history of use in humans and may likewise result in unexpected clinical outcomes, some of which may not become apparent until our REOs are administered to larger numbers of patients or for longer periods of time. Unexpected clinical outcomes associated with either type of chemistry could include lower than anticipated editing efficiency or durability, unintended editing, immunogenicity or other adverse effects, and could require us to modify or redesign our REOs, conduct additional preclinical studies or clinical trials, or discontinue development of one or more development candidates. In addition, because the same or similar chemistries may be used across multiple REOs, an adverse finding associated with a chemistry used in one development candidate could adversely affect our other development candidates and OPERA more broadly.

The chemistries used in our REOs may also complicate their manufacture. Synthesis, purification and analytical methods that have been established for ASOs and siRNAs may not be directly transferable to REOs that incorporate those chemistries, and established methods may not exist for the novel chemistries we have developed through OPERA. As a result, we or our CMOs may need to develop new or modified processes and analytical methods to manufacture and characterize our REOs and to identify and control impurities, and certain raw materials may be available only from a limited number of suppliers or may need to be custom synthesized. These factors could increase our manufacturing costs, make it more difficult to scale up manufacturing, establish specifications acceptable to regulatory authorities or demonstrate comparability following changes to our manufacturing processes, and could delay our preclinical studies and clinical trials. For additional information, see the risk factor entitled *"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."*

Until recently, we concentrated our research efforts primarily 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 development 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 development 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 RNA 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 development candidates or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development of development candidates and have focused our efforts to date primarily on platform development, discovery, research, and preclinical development and had only dosed a small number of participants in our now terminated Phase 1/2a REWRITE clinical trial of KRRO-110 for AATD. Our lead development candidate, KRRO-121, only recently entered clinical development in Part 1 of ANCHOR, and we only recently nominated KRRO-111 as our GalNAc-conjugated REO for AATD, which remains in preclinical development. All of our other 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 development 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.

We initiated Part 1 of ANCHOR in Australia under the Clinical Trial Notification, or CTN, scheme, under which the trial is approved by an HREC and notified to the TGA, and the TGA does not evaluate the trial protocol or supporting data. We have not submitted an IND to the FDA for KRRO-121, and the FDA has not reviewed ANCHOR under an IND. Commencing clinical trials in the United States, including any portion of ANCHOR or any other clinical trial of KRRO-121 or KRRO-111 that we may conduct in the United States, is subject to an IND being in effect and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In addition, the initiation of Part 2 of ANCHOR is subject to regulatory approval, and the initiation of clinical development of KRRO-111 will be subject to an IND or comparable foreign application. In the event that the FDA or a comparable foreign regulatory authority requires us to complete additional preclinical studies or we are required to satisfy other requests prior to commencing a clinical trial, the start of such trial 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 clinical trial applications, or CTAs, and comparable submissions in other countries, including countries in the European Union, the United Kingdom and Australia.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize any development 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. Part 1 of ANCHOR is being conducted in Australia and not under a U.S. IND, we may conduct Part 2 of ANCHOR at sites outside the United States, and we may in the future decide to conduct other 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, including that the trial was well-designed and conducted in accordance with good clinical practice, or GCP, requirements that the data are applicable to the U.S. population and U.S. medical practice, and that the FDA is able to validate the data through an onsite inspection if it deems necessary, and there can be no assurance that the FDA will accept data from ANCHOR or any other clinical trials conducted outside of the United States. Further, recent policy proposals in the U.S., if enacted in the future, may make acceptance by the FDA or inclusion in a marketing application of foreign data more difficult or costly. The FDA has also recently signaled increased scrutiny of foreign clinical trial data submitted in support of marketing applications, indicating plans for increased foreign site inspections and increased transparency of inspection results. If the FDA does not accept the data from ANCHOR or any other clinical trial that we conduct outside the United States, including as a result of access to clinical trial sites or documentation being delayed or restricted, including for geopolitical reasons, 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 development 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 development candidate testing and validation and additional administrative review periods.

Commercialization of any development candidates we may develop will require preclinical and clinical development; regulatory and marketing approval in multiple jurisdictions, including by the FDA, the EMA, HREC and TGA; manufacturing supply, capacity

and expertise; a commercial organization; and significant marketing efforts. The success of our development 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 or continuation of our ongoing and planned clinical trials, including Part 2 of ANCHOR, or future clinical trials for any development candidates we may develop;
- successful enrollment and completion of clinical trials, including under the FDA's GCPs, good laboratory practice, or GLP, requirements, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our ongoing and future clinical trials, including ANCHOR, 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 development candidates we may develop;
- commercial launch of any development candidates we may develop, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of the development 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 products;
- maintenance of a continued acceptable safety, tolerability and efficacy profile of any development 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 development candidates we may develop, which would materially harm our business.

Any development 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 development candidates, we must conduct, at our own expense, extensive preclinical studies and clinical trials to demonstrate the safety and efficacy in humans of our development 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, or NHPs, to conduct certain preclinical studies. Over the past several years there has been an increasing global shortage of NHPs available for drug development that has matured into an acute global supply chain issue. The supply of these NHPs 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 NHPs 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 development 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 development 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, the EMA, HREC, TGA 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 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, the EMA, HREC, TGA 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 any pandemic or endemic or other geopolitical events that can disrupt supply chains, such as announced tariffs, hostilities in Iran, or other evolving economic policies;
- 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 development 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 development 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 development candidate being tested that are experienced by participants in our clinical trials or by individuals using drugs similar to our development candidates;
- poor or disappointing effectiveness of our development candidates during clinical trials;
- unfavorable outcome of FDA, EMA, HREC, TGA 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 development 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 development 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 development candidates fails or is delayed to a point where such development candidate is no longer commercially viable, our business may be materially harmed.

Interim, topline and preliminary data from our clinical trials, including ANCHOR, that we announce or publish from time to time may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary, interim or topline data from our clinical trials, such as the interim clinical readout from Part 2 of ANCHOR that we currently expect in the second half of 2027. These data are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. Interim data are also subject to the risk that one or more clinical outcomes may materially change as participant enrollment continues and more participant data become available, a risk that may be heightened in open-label trials with small numbers of participants, such as Part 2 of ANCHOR. Adverse differences between preliminary, interim or topline data and final data could significantly harm our business prospects. Further, others, including regulatory authorities, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses, or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular development candidate and our company in general. In addition, the information we choose to publicly disclose regarding a particular trial is typically selected from a more extensive amount of available information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the preliminary, interim or topline data that we report differ from final results, or if others, including

regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our development candidates may be harmed, which could harm our business, financial condition, results of operations and prospects.

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;
- 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 development candidates for our research and development and preclinical activities, or manufacture sufficient amounts of our development candidates to meet our clinical requirements and timelines, our business may be materially harmed.

In order to develop our development candidates, apply for regulatory approvals and commercialize our development 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; supply chain disruptions (including as a result of announced tariffs, other geopolitical events or global health pandemics) could impact our business. Additionally, our cost of goods development is at an early stage. The actual cost to manufacture and process our development candidates could be greater than expected and could materially and adversely affect the commercial viability of our development candidates.

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 development candidates expose us to various manufacturing challenges and risks, which may include delays in manufacturing adequate supply of our development 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 development 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 development 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 development candidate.

Although we continue to build on our experience in manufacturing oligonucleotides, we have limited experience as a company manufacturing development candidates for commercial supply. We may never be successful in manufacturing development 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 development 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 development 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 development 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 development candidate or indication and fail to capitalize on development 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 development 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 development 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 development 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 development candidate, we may relinquish valuable rights to that development 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 development candidate.

Our development 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 dosed very few participants with our development candidates in human clinical trials, and KRRO-121 has been administered to only a limited number of healthy volunteers in Part 1 of ANCHOR. Moreover, there have been only a limited number of clinical trials involving the use of genetic medicine technologies and RNA editing technology similar to our technology. It is impossible to predict when, or if, any development 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.

KRRO-121 is the first development candidate we have advanced into the clinic that is designed to generate a *de novo* protein, and its novel mechanism of action increases the risk of unanticipated safety findings. KRRO-121 is designed to edit GS mRNA in hepatocytes to produce a stabilized variant of GS that does not occur naturally, which could elicit an immune response against the edited protein or otherwise have effects that we have not anticipated. In addition, because GS converts ammonia and glutamate into glutamine, increasing GS activity could alter glutamine or glutamate levels, and elevated glutamine has been associated with neurological toxicity in patients with hyperammonemia, including HE. Although we have not observed significant increases in plasma glutamine in our preclinical studies, these findings may not be replicated in humans, particularly over longer periods of dosing. Our REOs also recruit endogenous ADAR enzymes and could result in unintended editing at other sites in the transcriptome or interfere with the normal function of ADAR, and GalNAc-conjugated oligonucleotides as a class have been associated with injection site reactions and, for certain compounds, liver enzyme elevations and other adverse effects. Any such findings in ANCHOR could cause us, an HREC, the TGA or other regulatory authorities to delay, modify, suspend or terminate the trial.

If any such adverse events occur, ANCHOR or our future clinical trials could be suspended, placed on clinical hold or otherwise delayed or terminated. If we are unable to demonstrate that any adverse events were caused by the administration process or related procedures, the FDA, the EMA, HREC, TGA or other regulatory authorities could order us to cease further development of, or deny approval of, any development 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 ANCHOR or any future trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate ANCHOR or any future clinical trial of any of our development candidates, the commercial prospects of such development candidates may be harmed and our ability to generate product revenues from any of these development candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other development candidates, and may adversely affect our business, financial condition, results of operations and prospects significantly.

Additionally, if any of our development 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 development candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such development candidate;
- we may be required to change the way a development 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 development candidates we develop, or experience significant delays in doing so, we may not realize the full commercial potential of any products we may develop.

Our success may depend, in part, on our ability to identify patients who are likely to benefit from therapy with any products 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 development 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 development 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 products.

As a result of these factors, we may be unable to successfully develop and realize the commercial potential of any development 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 establish sales and marketing capabilities or enter into agreements with third parties to market and sell any development candidates, we may be unable to generate any revenues.

We currently do not have an organization for the sales, marketing and distribution of any development 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 development candidates, we may enter into strategic alliances with third parties to develop and commercialize any development 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 development 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 development 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 development 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 development candidates, the market may not be receptive to our development candidates upon their commercial introduction, which will prevent us from becoming profitable.

Our development 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 REOs. 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 development 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 development 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 development 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 development 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, including UCD, HE, and AATD. 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;
- development 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 products. 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 development 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. As a result of the discontinuation of KRRO-110 and our transition to GalNAc-conjugated delivery for our AATD program, we are behind certain of our competitors that have clinical-stage programs for AATD, which could negatively impact our ability to develop a commercially viable product for such indication. In addition, we have described certain of our development candidates as having first-in-class or best-in-class potential. These characterizations are based on preclinical data, and we have not conducted any head-to-head clinical comparisons with existing or investigational therapies. Accordingly, our development candidates may not prove to be first-in-class or best-in-class or to have advantages over competing therapies.

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 products, 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 development 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.

Healthcare legislative or regulatory reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of development candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any development candidates for which we obtain marketing approval. Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs. Recent and proposed healthcare reform measures in the United States have focused in particular on prescription drug pricing, reimbursement, and manufacturer financial obligations under government healthcare programs. These measures include statutory changes, executive actions, and proposed regulations that may affect pricing, coverage, reimbursement methodologies, and market access for pharmaceutical and biological products. For example, in September 2026, CMS finalized the Global Benchmark for Efficient Drug Pricing, or GLOBE, Model, a mandatory Medicare Part B payment model that ties manufacturer rebates to international reference prices and is scheduled to begin January 1, 2027, although the final model excludes certain categories of drugs, including drugs designated and approved only for rare diseases or conditions. CMS has not finalized the companion Medicare Part D GUARD Model, and the GLOBE Model is the subject of litigation. We cannot predict whether or how these or similar models would apply to any of our development candidates, if approved, including if we obtain approval for an indication that is not a rare disease or condition.

For a more complete discussion of healthcare reform and regulatory developments that may affect our business, see Item 1, *"Business—Government Regulation—Healthcare Reform Measures"* in the 2025 10-K.

There has been increasing legislative, regulatory, and enforcement interest in the United States with respect to prescription drug pricing practices. Specifically, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs.

The continuing efforts of the government, insurance companies, managed care organizations and other payers of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any of our development candidates, if approved;
- the ability to set a price that we believe is fair for any of our development candidates, if approved;
- our ability to generate revenues and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

We expect that additional healthcare reform measures will be adopted in the future, any of which could result in reduced demand for our development candidates or additional pricing pressures. These cost containment measures (be it federal or state regulations, or regional bidding processes, or most-favored-nation pricing practices, or otherwise) could reduce the ultimate demand for any approved development candidates and or put pressure on our pricing, which could negatively affect our business, financial condition, results of operations and prospects.

In addition, a 2024 U.S. Supreme Court decision may increase regulatory uncertainty and litigation risk and could delay or complicate FDA regulatory review, policymaking, or implementation of regulatory initiatives. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action. Any such challenges, if successful, could have an impact on our business, and any such impact could be material. In addition to potential changes to regulations and agency guidance as a result of legal challenges, these decisions may result in increased regulatory uncertainty and delays in and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations.

If the market opportunities for any development 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 development 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 development 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 development 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 development 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 development candidates that are designed to encourage the research and development of development 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 development candidates. See Item 1 "*Business—Government Regulation—Expedited Development and Review Programs for Drugs*" in the 2025 10-K 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 development candidates, there can be no assurance that we will realize their intended benefits.

In the future, we may also seek approval of development candidates under the FDA's accelerated approval pathway or request priority review. There can be no assurance that FDA would allow any of the development 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 European Union, we may seek to participate in The PRIority MEdicines, or PRIME, scheme for our development 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 European Union. There is no guarantee, however, that our development 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*" in the 2025 10-K.

Although we have obtained rare pediatric disease designation for KRRO-121 for the treatment of UCDs, we may not be eligible to receive a priority review voucher in the event the FDA determines we no longer meet the criteria for designation, revokes the designation or FDA approval does not occur prior to September 30, 2029.

The sponsor of an application for a rare pediatric disease drug product may be eligible for a voucher that can be used or sold to obtain a priority review for a subsequent application submitted under section 505(b)(1) of the Federal Food, Drug, and Cosmetic Act or section 351 of the Public Health Service Act. Designation of a drug as a product for a rare pediatric disease does not guarantee that a marketing application for such drug will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Even though we have received rare pediatric disease designation for KRRO-121 for the treatment of UCDs, we would need to request a rare pediatric disease priority review voucher in the marketing application for KRRO-121. Vouchers for rare pediatric disease drugs are awarded for qualifying applications when the drug receives approval. After September 30, 2029, the FDA may not award any rare pediatric disease priority review vouchers, although the FDA's authority to do so could be extended by Congress in the future.

Risks Related to Regulatory, Legal, and Clinical Trials

Because we are developing REOs, 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 development 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 2024 final 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 REO therapies. The general lack of policies, practices or guidelines specific to oligonucleotides may hinder or slow review by the FDA or other foreign regulatory agencies of any regulatory filings that we may submit. Moreover, the FDA or other foreign regulatory agencies 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 development 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 development 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 development 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.

Because we are developing development candidates in the field of genetic medicines in which there is little clinical experience, there is increased risk that the FDA, the EMA, HREC, TGA 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 KRRO-111 or any other development candidates we identify, and to initiate Part 2 of ANCHOR or other clinical trials of KRRO-121, we will need to submit INDs or comparable foreign applications to regulatory authorities and obtain regulatory clearance to commence clinical development. Because the development 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 development candidates we may develop. As we are initially seeking to identify and develop development 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, HREC, TGA 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). For example, we intend to evaluate the clinical activity of KRRO-121 in UCD using measures that may include plasma ammonia levels. Regulatory authorities may not accept changes in ammonia levels or other biomarkers as a basis for approval and may instead require clinical outcome endpoints, such as a reduction in HACs, which could require larger and longer clinical trials. 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 European Union and other countries may make similar comments with respect to these endpoints and data. Any development 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 RNA 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 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.

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 development candidate and the approval may be for a narrower indication than we seek.

Prior to commercialization, any of our development 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 development candidates involved. Failure to obtain marketing approval for a development candidate will prevent us from commercializing the development candidate. We have not received approval to market any of our development 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 development 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 development 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 development candidate.

Approval of any of our development 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 development 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 development 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 development 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 development 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 development 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 development 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 development candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our development 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 development candidates and our ability to generate revenue will be materially impaired.

Our development 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 development 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 development 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 development 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 development 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 development candidates, when and if any of them are approved.

Our development 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 development 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 development candidates, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such development 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 development candidates;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our development 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 development 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, applicable product tracking and tracing requirements 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 development 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 development 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 development 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.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our development candidates could limit our product revenues.

Because our development 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 development 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 – Pharmaceutical Coverage and Reimbursement"* in the 2025 10-K.

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 development 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.

While the EMA has granted orphan drug designation to KRRO-121 for the treatment of UCD, we may not be able to obtain or maintain orphan drug designation or exclusivity for KRRO-121 or our other development candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

In August 2026, we announced that the EMA granted orphan drug designation to KRRO-121 for the treatment of UCD. KRRO-121 has not received orphan drug designation from the FDA. Under the Orphan Drug Act, the FDA may designate a development 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 development candidates by the EMA in the European Union. 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 development 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 European Union. The exclusivity period in the European Union 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. In addition, orphan designation in the European Union is re-assessed at the time of marketing authorization, and because there are authorized treatments for UCD, we would need to demonstrate that KRRO-121 provides a significant benefit to patients affected by UCD in order to maintain its orphan designation and obtain market exclusivity in the European Union. We may not be able to make that showing.

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 development candidates, the agency must find that the development 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 development 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 development candidate, that exclusivity may not effectively protect the development candidate from competition because different development candidates can be approved for the same approved use or condition. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same development candidates for the same approved use or condition if the FDA concludes that the later development 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.

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 violate 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, and may in the future be, subject to extensive federal, state, local, and comparable foreign healthcare laws and regulations, including those relating to fraud and abuse, anti-kickback restrictions, false claims, transparency and reporting, patient privacy and data protection, and anti-bribery and related requirements, which may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, develop, market, sell, and distribute our products for which we obtain marketing approval. The scope and enforcement of these laws is uncertain, subject to rapid change, and dependent on evolving government and industry interpretations, and authorities have increased scrutiny of interactions between healthcare companies and healthcare providers and third-party payors. If our operations are found to be in violation of applicable requirements, we may be subject to penalties and other sanctions, which may include civil or criminal penalties, criminal prosecution, monetary damages, disgorgement, contractual damages, reputational harm, curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs (including Medicare and Medicaid), the imposition of a corporate integrity agreement, suspension or withdrawal of product approvals, restrictions or prohibitions on marketing, and other enforcement actions. Any investigation or enforcement action, even if ultimately resolved favorably, could cause us to incur significant legal and compliance expenses, divert management attention, and adversely affect our business, financial condition, results of operations, and prospects. We intend to develop and implement a comprehensive corporate compliance program prior to commercialization; however, compliance programs can mitigate but cannot eliminate the risk of noncompliance and related enforcement. For more information, see Item 1, *"Business—Governmental Regulation—Other Healthcare Laws"* in the 2025 10-K.

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. 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.

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.

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 development candidates we may develop and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our development 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 products 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 currently maintain clinical trial insurance, and we anticipate that we will need to increase our insurance coverage as we expand ANCHOR to include patients and additional jurisdictions, initiate additional clinical trials and if we successfully commercialize any product. 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. If such an event were to occur and cause interruptions in our operations, it could result in a 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 development candidates could be significantly delayed.

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

We and the third parties upon which we rely face a variety of evolving threats, which could cause cybersecurity incidents or data breaches. Cybersecurity incidents and data breaches are increasing in their frequency, sophistication and intensity, and have become

increasingly difficult to detect. Cybersecurity incidents and data breaches could include wrongful conduct by hostile foreign governments, industrial espionage, wrongful conduct by employees or vendors, human error, wire fraud and other forms of cyber fraud, the deployment of harmful malware or ransomware, denial-of-service attacks, social engineering fraud (including successful phishing attacks) or other means to threaten data confidentiality, integrity and availability.

Like other companies in our industry, we and our third party vendors, have experienced, or may experience, threats and cybersecurity incidents relating to our and our third-party vendors' information systems and infrastructure. While we have not experienced any material cybersecurity incident or data breach to date, if such an event were to occur, it 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.

Although we devote resources to protect our information systems, there can be no assurance that our efforts will prevent cybersecurity incidents or data breaches that could 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. Attempts to disrupt or gain unauthorized access to our and our third-party vendors' information systems from malicious third parties or insider threats may incorporate widely varying and frequently changing tactics, which may be enhanced or facilitated by artificial intelligence. Further, our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our privacy and data security obligations. Although we maintain cyber liability insurance, this insurance may not provide adequate coverage against potential liabilities related to any experienced cybersecurity incident or breach. For additional information on our cybersecurity obligations as well as our cybersecurity program, see Item 1 "*Business—Government Regulation—Privacy and Cybersecurity*" and Item 1C "*Cybersecurity*" in the 2025 10-K.

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

To market and sell our future development 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 development 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 development 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 development candidates by regulatory authorities in another country may significantly diminish the commercial prospects of that development candidate and our business prospects could decline.

Disruptions at the FDA, SEC and other government agencies caused by staffing cuts, 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 staffing, 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. Currently, most federal agencies, including the FDA, are operating under a continuing resolution that funds the federal government through December 11, 2026. Without appropriation of adequate funding to federal agencies, our business operations related to our product development activities for the U.S. market could be impacted. A prolonged government shutdown could significantly impact the ability of the FDA to timely review and process our regulatory submissions, the National Institutes of Health, or NIH, to conduct research or provide grants, or other agencies to slow their work, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Disruptions at the FDA and other federal agencies, including substantial leadership departures, personnel cuts, and policy changes, may also slow the time necessary for new drugs to be reviewed and/or approved, which would harm our business. Changes and cuts in FDA staffing have been reported by some within the pharmaceutical industry as creating instances of delays in the FDA's

responsiveness or in its ability to review regulatory submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion.

There is substantial uncertainty as to the extent and manner in which the current administration will continue to seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our development candidates and any products for which we obtain approval. This uncertainty could present new challenges and/or opportunities as we navigate development and approval of our development candidates. Additionally, the current administration could issue or promulgate executive orders, regulations, policies or guidance that adversely affect us or create a more challenging or costly environment to pursue the development of new therapeutic candidates. Also, state governments may seek to address or react to changes at the federal level with changes to their regulatory frameworks in a manner that could impact our operations.

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 personal and health information of participants in our clinical trials, including ANCHOR, 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 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. In Australia, where we are conducting Part 1 of ANCHOR, we and our service providers may be subject to the Privacy Act 1988 (Cth), including the Australian Privacy Principles, and to state and territory health records laws. For additional information on these regimes, see Item 1 "*Business—Government Regulation—Privacy and Cybersecurity*" in the 2025 10-K. 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.

We may use and integrate AI into our business processes both in our own development and implementation of AI and through the adoption of commercially available tools. Use of this technology could pose cybersecurity, data privacy, IT, intellectual property, regulatory, legal, operational, competitive, reputational and other risks and challenges that could affect our business. Specifically, risks related to accuracy, bias, artificial intelligence hallucinations, discrimination, harmful content, misinformation, fraud, scams, targeted attacks (including model poisoning or data poisoning), surveillance, data leakage, inequality, environmental harms, and other harms may flow from our development, use, or deployment of AI technologies.

The use of certain AI technology can give rise to intellectual property risks, including by disclosing or otherwise compromising our confidential or proprietary intellectual property and intellectual property infringement, or by undermining our ability to assert or defend ownership rights in intellectual property created with the assistance of AI tools.

Additionally, we expect to see increasing government and supranational regulation related to AI use and ethics, which may also significantly increase the burden and cost of research, development and compliance in this area. For example, the European Union began implementing the Artificial Intelligence Act, or AI Act, with a significant part of the law scheduled to come into effect in August 2026. As currently enacted, the AI Act, which may be amended, imposes significant obligations on providers and deployers of high risk AI systems, and encourages providers and deployers of AI systems to account for EU ethical principles in their development and use of these systems.

In the United States, the AI regulatory environment is complex and uncertain. Over the past year, states have advanced, and in some cases passed, dozens of laws focusing on AI governance and regulation, including on deployment of AI in healthcare settings. At the federal level, the current U.S. administration has endorsed a federal moratorium on the enforcement of state AI laws, including through a December 2025 executive order. So far, these efforts have not been successful at curtailing state action on AI regulation, contributing to a complicated legislative patchwork, which may be litigated in state and federal courts. If we develop or deploy AI systems that are governed by these laws or regulations, we will need 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. We may also be subject to significant enforcement or litigation in the event of any perceived non-compliance.

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. If we enable or offer solutions that draw controversy due to perceived or actual negative societal impact, we may experience brand or reputational harm, competitive harm or legal liability. 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. In addition, the use of generative AI models in our internal or third-party systems may create new attack surfaces or methods for adversaries, which could impact us and our vendors. 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 development 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 rely, and expect to continue to rely, on third parties to conduct our clinical trials, including ANCHOR, 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 and clinical trials. We rely on CROs and on clinical investigators at clinical trial sites in Australia to conduct Part 1 of ANCHOR, and we expect to rely on additional CROs, investigators and sites, which may be located in other jurisdictions, for Part 2 of ANCHOR. For example, we may rely on third parties to conduct some of our preclinical animal experiments and supply key materials for our programs. 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 development candidates or successfully commercializing or competing in the market for certain indications.

We may decide to collaborate with non-profit organizations, universities, pharmaceutical and other biotechnology companies for the development and potential commercialization of existing and new development 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 development candidate, the costs and complexities of manufacturing and delivering such development 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 development 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 development 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. Even when we enter into collaborations such agreements may terminate earlier than expected and we may never see the full benefits of such agreement. For example, we agreed with Novo Nordisk to pause our September 2024 collaboration for 12 months and Novo Nordisk recently exercised its rights to terminate early for convenience.

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 development 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 development 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 development 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;
- program divestitures due to potential exclusivity obligations;
- 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 development 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 lead candidates and development candidates for our research, preclinical and clinical activities, and may do the same for commercial supplies of our lead candidates or development candidates.

We have not yet manufactured our development candidates on a commercial scale, and may not be able to do so for any of our development 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 development 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 development 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 development 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 REOs, which could severely impact the manufacturing of our development 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 development 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 lead candidates and

development candidates or materials. In some cases, the technical skills or technology required to manufacture our lead candidates and development 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 lead candidates and development 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 lead candidates and development 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 development candidates. If we are unable to obtain or maintain third-party manufacturing for development candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our development 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.

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. Although we implemented workforce reductions in May 2025 and in November 2025, 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. Our recent workforce reductions could impede future recruiting and hiring efforts. Failure to successfully recruit and retain personnel could impact our anticipated development plans and timelines. For example, 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 lead candidates, development 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 development candidates and implement our business plan will be limited.

We expect to need 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 future growth, which could disrupt our operations.

As of September 30, 2026, we had 58 full-time employees, including 42 who hold Ph.D. degrees or other advanced degrees; 44 employees are engaged in research and development and 14 employees are engaged in management or general and administrative activities. In May 2025, we announced a workforce reduction and organizational streamlining and in November 2025, we announced a further workforce reduction to extend our cash runway. In connection with the growth and advancement of our pipeline, we expect that we will need to increase the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, and as any development candidates near later stage clinical trials and potential commercialization by us, 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. Our workforce reduction may make these efforts more challenging. 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

future expansion of our operations or recruit and train additional qualified personnel. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a pre-commercial biotechnology company, we are actively pursuing new platforms and lead candidates in many therapeutic areas and across a wide range of diseases. Successfully developing development 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 and recent workforce reductions, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or, when appropriate, 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, further 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 development 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 development 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 lead candidates and development candidates, development and commercialization of our development 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 development candidates and platform technologies, as well as for methods used to manufacture our development candidates, and methods for treating patients for approved indications using our development 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., an NDA in the United States). Therefore, the risk that a third party might allege patent infringement may increase as our development 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 development candidates. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a development 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 development 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 development 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 development 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 development 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 development 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 lead candidates and development 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 lead candidates and development 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 development 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 lead candidates, development candidates and RNA editing technology. While we will endeavor to try to protect our lead candidates, development 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, our lead candidates and our development candidates.

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 lead candidates and development 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 issue as patents with claims that cover our lead candidates and development 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 our patents based on inventorship, validity, enforceability or scope, including through opposition, revocation, reexamination, post-grant and *inter partes* review proceedings. An adverse determination in any such 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 development 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 lead candidates and development candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize our development candidates. Further, if we encounter delays in development, testing, and regulatory review of new development candidates, the period of time during which we could market our development 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 development candidates.

Other parties have developed technologies that may be related or competitive to our technologies. 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. 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, it is also possible that prior art may exist that we are aware of but do 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 initiate legal proceedings to enforce our patent rights, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our development candidates, the defendant could counterclaim that the patent covering our product or development 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 of patent litigation is often 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 third party 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 lead candidates and development 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 lead candidates and development 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.

Even if we establish infringement of any of our patents by a third party, 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-license 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 development 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 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 development 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 development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our development 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 development 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 development 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 development 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 development 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 development candidates;
- the requirement that we redesign our development 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 our lead candidates or development 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 lead candidates or development 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 lead candidates or development 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 development 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 development 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 development 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 development 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 development 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 development 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 development candidates, which could significantly harm our business.

Likewise, our patents and patent applications, if issued as patents, directed to our proprietary technologies, lead candidates and development candidates are expected to expire from 2040 through 2046, 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 development 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 development 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 development 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 field of gene editing technology and filing patent applications potentially relevant to our business. In order to market our development 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 development 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 development 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 development 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, and if we are successful in obtaining an in-license, such collaboration may make us subject to governmental step-in rights under the Bayh-Dole Act. 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 development 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 lead candidates and development 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 development 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, lead candidates and development 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 lead candidates and development candidates or to 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. 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, 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, lead candidates and development 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 regard 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 development candidates for an adequate amount of time.

Patents have a limited lifespan. The terms of individual patents depend 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 Patent Term Extension, or PTE, and Patent Term Adjustment, or 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*" in the 2025 10-K. Even if patents covering our development 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 development candidates, patents protecting our development 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 development candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any development 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 development 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 development 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 development 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 development 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 been engaged in scientific collaborations 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 development candidates we develop 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 development candidates.

Our use of open source software could impose limitations on our ability to commercialize our development 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 development candidates. We could be required to seek licenses from third parties in order to continue offering our development candidates, to re-engineer our development candidates or to discontinue the sale of our development 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.

General Risk Factors

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 development 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.

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.

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 lead candidates and development candidates, including interim and other data from ANCHOR, 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;
- 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 development 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 development 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 development 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 are incurring and expect to continue to incur additional costs and increased demands upon management as a result of complying with the laws and regulations affecting public companies.

We are incurring and expect to continue to incur significant legal, accounting and other expenses operating as a public company, including costs associated with public company reporting obligations under the Exchange Act. Our 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 our current 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 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 a smaller reporting company with less than $100.0 million of revenues, 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. Although we no longer qualify as an emerging growth company, we still qualify as a "smaller reporting company," as such term is defined in Rule 12b-2 under the Exchange Act, which allows us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Once we are no longer 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 control 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 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, the trading price 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 54% 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 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 may invest or spend our cash 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. You may not agree with our decisions, and our use of our cash and cash equivalents 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. You will not have the opportunity to influence our decisions on how to use our cash resources.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or development 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 development candidates we may develop, or we may have to grant licenses on terms that may not be favorable.

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. The most recent U.S. federal tax legislation was signed into law on July 4, 2025 and modified how we account for research and development expenses. 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 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, 2025, we had federal and state NOLs of $462.0 million and $436.9 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 $439.6 million of federal NOLs that do not expire. Our state NOLs expire at various dates from 2035 through 2045. As of December 31, 2025, we had federal research and development tax credit carryforwards of $20.2 million that expire at various dates from 2037 through 2045. In addition, as of December 31, 2025, we had state research and development tax credit carryforwards of $9.9 million that expire at various dates from 2032 through 2040.

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or 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 November 2023 business combination or the private financing that closed immediately prior thereto 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.

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 development 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.

Our business may be impacted by macroeconomic conditions, including inflation, rising interest rates and volatile market conditions, and other uncertainties beyond our control, including geopolitical events, such as ongoing U.S. and Israeli military action in Iran, and effects thereof.

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 (such as what occurred in 2023), changes in inflation, interest rates and overall economic conditions and uncertainties (including as a result of announced tariffs or other policy changes by the current U.S. administration). 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. 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. 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. Moreover, significant political, trade, regulatory developments, and other circumstances beyond our control, such as geopolitical events, like the ongoing U.S. and Israeli military action in Iran and effects thereof, could have a material adverse effect on our financial condition or results of operations.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Not applicable.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

During the three months ended September 30, 2026, the following officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, amended or terminated contracts, instructions or written plans for the purchase or sale of our securities that were intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act (each, a "Rule 10b5-1 plan"): On July 24, 2026, Ram Aiyar, our Chief Executive Officer, President and Interim Chief Financial Officer, adopted a Rule 10b5-1 plan providing for the potential sale of up to 25,000 shares of our common stock issued upon the vesting and settlement of restricted stock units; this plan is scheduled to expire on January 21, 2028. On September 1, 2026, Todd Chappell, our Chief Operating Officer, adopted a Rule 10b5-1 plan providing for the potential sale of up to 13,944 shares of our common stock issued or issuable upon the vesting and settlement of restricted stock units, as well as up to 5,000 shares of our common stock issuable upon the exercise of stock options; this plan is scheduled to expire on September 3, 2027. On September 1, 2026, Jeffrey Cerio, our Senior Vice President, General Counsel and Corporate Secretary, adopted a Rule 10b5-1 plan providing for the potential sale of up to 18,842 shares of our common stock issued or issuable upon the vesting and settlement of restricted stock units; this plan is scheduled to expire on December 31, 2027.

During the three months ended September 30, 2026, none of our other officers or directors informed us that they adopted, modified or terminated a Rule 10b5-1 plan or a trading plan not intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act.

Item 6. Exhibits.

Exhibit Number	Description
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	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 June 12, 2024)
3.5	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)
10.1	Exchange Agreement, dated as of August 5, 2026, by and among the Korro Bio, Inc. and certain investors party thereto (incorporated by reference to Exhibit 10.1 to the registrant's Current Report on Form 8-K filed on August 6, 2026).
10.2	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the registrant's Current Report on Form 8-K filed on August 6, 2026).
31.1*	Certification of Principal Executive Officer and 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
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 With Embedded Linkbase Documents
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.

† 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.

SIGNATURES

Pursuant to the requirements 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: November 5, 2026

By: /s/ Ram Aiyar

Ram Aiyar
President and Chief Executive Officer and Interim Principal Financial Officer

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO RULE 13a-14(a) / RULE 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, Ram Aiyar, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2026 of Korro Bio, Inc. (the "registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ Ram Aiyar

Ram Aiyar
President and Chief Executive Officer
(Principal Executive Officer and Interim Principal Financial Officer)

Dated: November 5, 2026

Exhibit 32.1

CERTIFICATIONS 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

In connection with the Quarterly Report on Form 10-Q of Korro Bio, Inc. (the "Company") for the period ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, that, to the best of his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Ram Aiyar
Ram Aiyar
President and Chief Executive Officer
(Principal Executive Officer and Interim Principal Financial Officer)

Dated: November 5, 2026

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Korro Bio, Inc. under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.