



## Korro Announces Selection of its First Development Candidate for the Potential Treatment of Alpha-1 Antitrypsin Deficiency (AATD)

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- *KRRO-110 is the first RNA editing oligonucleotide candidate from Korro's proprietary OPERA™ platform*
- *Korro has demonstrated the ability to repair the misfolding of the Alpha-1 Antitrypsin (A1AT) protein containing the Z mutation, as well as reduce liver aggregates, in a preclinical mouse model*
- *Korro has also demonstrated high editing efficiency (>50% editing) in a preclinical mouse model, leading to increased secretion of normal A1AT protein to levels that Korro believes has the potential to be clinically meaningful for AATD patients*
- *Korro expects regulatory filing in the second half of 2024*

CAMBRIDGE, Mass., Dec. 07, 2023 (GLOBE NEWSWIRE) -- Korro Bio, Inc. (Korro) (Nasdaq: KRRO), a biopharmaceutical company focused on developing a new class of genetic medicines for both rare and highly prevalent diseases, has nominated its first development candidate, KRRO-110, for the potential treatment of AATD. KRRO-110 is a proprietary RNA editing oligonucleotide delivered to liver cells using clinically validated LNP technology licensed from Genevant. KRRO-110 is designed to co-opt an endogenous enzyme Adenosine Deaminase Acting on RNA (ADAR) to repair a pathogenic single nucleotide variant (SNV) on RNA and restore production of normal A1AT. Preclinical development of KRRO-110 is ongoing in preparation for a potential regulatory filing in the second half of 2024.

"The advancement of KRRO-110 highlights the power of our RNA editing platform, OPERA, and we are excited to see what we expect to be the first of many candidates move into the clinic over the coming years," said Ram Aiyar, PhD, Chief Executive Officer of Korro. "Selecting a development candidate for our AATD program is an important milestone for Korro, but more importantly, for patients. We are confident that the preclinical profile demonstrated by KRRO-110 will make it a potentially game-changing therapeutic candidate that reduces the disease burden and addresses the continued unmet need faced by patients with both liver and lung manifestations of AATD."

AATD is an inherited, autosomal recessive genetic disorder that is most frequently caused by a SNV mutation in the SERPINA1 gene, the most common of which is the "PiZ" mutation. Greater than 95% of severe clinical cases are homozygous for the PiZ mutation (known as the PiZZ genotype). Korro has generated compelling preclinical data demonstrating high editing efficiency (>50% editing), which it believes is a key threshold to achieving clinically meaningful secretion of normal A1AT protein in an *in vivo* mouse model, and targeted durability. In addition, Korro has also shown using surrogates that its product candidates have high translation of RNA editing efficiency from mice to non-human primates (NHPs), demonstrating the potential applicability of its approach in humans.

"AATD patients face a critical unmet need given the limited effectiveness of current standard-of-care options for either the liver or the lung manifestations of the disease," said Dr. Jeffrey Teckman, an expert in AATD from St. Louis University School of Medicine and a long-time advisor to Korro. "Korro's RNA editing approach has the potential to be transformative for AATD patients and Korro is well-positioned to deliver a best-in-class therapeutic. I look forward to having access to this therapy as quickly as possible."

### **About AATD**

AATD is an inherited genetic disorder caused by SNVs in the SERPINA1 gene that can lead to severe progressive lung disease, including emphysema and chronic obstructive pulmonary disease (COPD), and liver disease. There are an estimated 3.4 million individuals with deficiency allele combinations worldwide. The only current FDA-approved treatment for AATD is augmentation therapy, a once-weekly infusion of pooled human plasma-derived A1AT protein, which does not adequately address the manifestations of AATD.

### **About Korro**

Korro is a biopharmaceutical company focused on developing a new class of genetic medicines for both rare and highly prevalent diseases using its proprietary RNA editing platform. Korro is generating a portfolio of differentiated programs that are designed to harness the body's natural RNA editing process to effect a precise yet transient single base edit. By editing RNA instead of DNA, Korro is expanding the reach of genetic medicines by delivering additional precision and tunability, which has the potential for increased specificity and improved long-term tolerability. Using an oligonucleotide-based approach, Korro expects to bring its medicines to patients by leveraging its proprietary platform with precedented delivery modalities, manufacturing know-how, and established regulatory pathways of approved oligonucleotide drugs. Korro is based in Cambridge, Mass. For more information, visit [korrobio.com](http://korrobio.com).

### **Forward-Looking Statements**

Certain statements contained in this press release may be considered forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, including express or implied statements regarding the timing of Korro's regulatory filings, Korro's ability to replicate results from preclinical studies in clinical trials, KRRO-110's ability to restore production of normal A1AT, and its potential as a game-changing therapeutic, as well as Korro's ability to move additional candidates into the clinic, among others. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "anticipate," "plan," "likely," "believe," "estimate," "project," "intend," and other similar expressions among others. Statements that are

not historical facts are forward-looking statements. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: risks related to (i) biopharmaceutical development generally; (ii) conducting pre-clinical studies and pre-clinical trials, including obtaining necessary regulatory approvals; (iii) protecting and enforcing intellectual property; (iv) integrating operations post-merger and operating the combined company as a public company as well as other risks related to the recent business combination; (v) legislative, regulatory and economic developments; (vi) unpredictability and severity of catastrophic events, including, but not limited to, acts of terrorism or outbreak of war or hostilities, such as the recent Hamas-Israeli conflict, as well as management's response to any of the aforementioned factors; and (vii) such other factors as are set forth in Exhibit 99.2 to the Current Report on Form 8-K filed with the SEC on November 6, 2023 as may be supplemented or amended by other SEC filings. Except as required by applicable law, Korro undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

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