



## Korro Bio Announces Dosing of First Participants in REWRITE Phase 1/2a Study of KRRO-110 for Alpha-1 Antitrypsin Deficiency and Provides Pipeline Update

January 13, 2025

- *KRRO-110 is the first product candidate from Korro's proprietary RNA editing OPERA™ platform*
- *Interim readout from REWRITE expected in the second half of 2025*
- *Korro to advance additional pipeline programs towards clinical development*

CAMBRIDGE, Mass., Jan. 13, 2025 (GLOBE NEWSWIRE) -- Korro Bio, Inc. (Korro) (Nasdaq: KRRO), a clinical-stage biopharmaceutical company focused on developing a new class of genetic medicines based on editing RNA for both rare and highly prevalent diseases, today announced the initiation of dosing in its REWRITE study investigating KRRO-110 as a potential treatment for individuals with Alpha-1 Antitrypsin Deficiency (AATD).

"KRRO-110 is designed to restore therapeutic M-AAT protein levels in individuals with at least one Z allele by leveraging the body's endogenous ADAR enzyme. The swift initiation of the REWRITE study for AATD is a testament to our collective strength and focused execution at Korro," said Kemi Olugemo, MD, Chief Medical Officer at Korro. "We are encouraged by our preclinical data, which demonstrates rapid restoration of functional AAT protein levels and durable editing. Our goal is to create a clinically differentiated, disease-modifying treatment to address both lung and liver manifestations of AATD. We look forward to evaluating the therapeutic potential of KRRO-110 and sharing interim data in the second half of this year."

REWRITE is a two-part single and multiple dose-escalating study that will evaluate the safety and tolerability of KRRO-110 in up to 64 participants, including healthy adults and clinically stable AATD patients with the PiZZ genotype. Secondary and exploratory endpoints include pharmacokinetic and pharmacodynamic parameters that will guide optimal dose selection for later stage studies. Interim data from Part 1 (single ascending doses in healthy volunteers and individuals with AATD) is expected in the second half of 2025, and completion of the study is anticipated in 2026. For additional information about the REWRITE study, visit [ClinicalTrials.gov \(NCT06677307\)](https://ClinicalTrials.gov/NCT06677307).

In addition to advancing KRRO-110, Korro is progressing a pipeline of wholly owned programs and a Novo Nordisk partnered program in a cardiometabolic indication towards the clinic.

"We have evolved from a research organization to a clinical-stage drug development organization with our first participant in the REWRITE study recently dosed with KRRO-110. We have taken time to build our pipeline to demonstrate the versatility of RNA editing, building on data from other oligonucleotide programs," said Ram Aiyar, PhD, President and CEO. "Over the next three years, we expect to take three product candidates into the clinic, in two tissue types with a single modular platform. This will be the crux of our 3-2-1 strategy through 2027. I am excited about the prospect that RNA editing holds beyond the treatment of rare genetic disease and applying our platform to highly prevalent diseases."

### **About Alpha-1 Antitrypsin Deficiency (AATD) and KRRO-110**

AATD is a genetic disorder most commonly caused by a single missense mutation (G-to-A) in the SERPINA1 gene. Affected adults experience pulmonary emphysema and/or hepatic cirrhosis, as well as end organ manifestations. KRRO-110 is the first RNA editing oligonucleotide product candidate from Korro's proprietary RNA editing platform, Oligonucleotide Promoted Editing of RNA (OPERA™). KRRO-110 is designed to co-opt an endogenous enzyme, Adenosine Deaminase Acting on RNA (ADAR), to edit the "A" variant on SERPINA1 RNA, repair an amino acid codon, and restore secretion of normal AAT protein. This repair of the endogenous protein has the potential to clear protein aggregates from within liver cells to create a potentially clinically differentiated benefit for liver function and to preserve lung function by providing an adequate amount of normal AAT protein.

### **About Korro**

Korro is a clinical-stage 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, enabling 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, Massachusetts. For more information, visit [korrobio.com](https://korrobio.com).

Korro intends to use its Investor Relations website as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD. Accordingly, investors should monitor Korro's Investor Relations website, in addition to following Korro's press releases, SEC filings, public conference calls, presentations, and webcasts.

### **Forward-Looking Statements**

Certain statements in this press release may constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements include, but are not limited to, express or implied statements regarding expectations, hopes, beliefs, intentions or strategies of Korro regarding the future including, without limitation, express or implied statements regarding: the timing of the interim data readout and completion of the Phase 1/2a clinical study of KRRO-110 for AATD (REWRITE), including Korro's ability to complete the Phase 1/2a clinical study; KRRO-110's potential to treat individuals with AATD; KRRO-110's ability to restore therapeutic M-AAT protein levels; KRRO's potential as a clinically differentiated, disease-modifying treatment to address both lung and liver manifestations of AATD; Korro's ability to progress a pipeline of programs towards the clinic; the timing of, and Korro's ability to, deliver three product candidates in two tissue types with a single modular platform by the end of 2027; among others. 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," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "strive," "would," "aim," "target," "commit," and similar expressions may identify forward-looking statements, but the absence of these words does not mean that statement is not forward looking. Forward-looking statements are based on current expectations and assumptions that, while considered reasonable are inherently uncertain. New risks

and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. Factors that may cause actual results to differ materially from current expectations include, but are not limited to, various factors beyond management's control including risks of conducting a first-in-human clinical study; challenges with addressing any regulatory concerns necessary to proceed with enrollment and dosing; risks associated with enrolling sufficient participants and other risks inherent in biopharmaceutical development; risks associated with conducting pre-clinical studies and clinical studies and risks of replicating results from pre-clinical studies in clinical studies; and other risks associated with obtaining regulatory approvals and protecting intellectual property; as well as risks associated with general economic conditions; and other risks and uncertainties indicated from time to time in Korro's filings with the SEC, including Part II Item 1A. "Risk Factors" in Korro's most recent Quarterly Report on Form 10-Q filed with the SEC, as such may be amended or supplemented by its other filings with the SEC. Nothing in this press release should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that any of the contemplated results of such forward-looking statements will be achieved. You should not place undue reliance on forward-looking statements in this press release, which speak only as of the date they are made and are qualified in their entirety by reference to the cautionary statements herein. Except as required by law, Korro does not undertake or accept any duty to release publicly any updates or revisions to any forward-looking statements to reflect any change in its expectations or in the events, conditions or circumstances on which any such statement is based. This press release does not purport to summarize all of the conditions, risks and other attributes of an investment in Korro.

**Korro Bio Contact Information**

Investor & Media Contact

Tim Palmer

[IR@korro.bio](mailto:IR@korro.bio)