



Korro Reports Best-in-Class Preclinical Data for KRRO-111 Demonstrating Potential to Benefit Lung and Liver Manifestations of Alpha-1 Antitrypsin Deficiency

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- *Repeat dose mouse studies achieved >95% SERPINA1 mRNA editing, sustained restoration of circulating functional M-AAT and increased total AAT by ~6-fold*
- *KRRO-111 reduced pathogenic hepatic Z-AAT by up to 95%, highlighting its potential to reduce toxic protein accumulation in the liver*
- *Best-in-class preclinical profile supports KRRO-111's potential to address both liver and lung manifestations of AATD; regulatory submission expected in the first half of 2027*

CAMBRIDGE, Mass., Oct. 22, 2026 (GLOBE NEWSWIRE) -- Korro Bio, Inc. (Korro) (Nasdaq: KRRO), a clinical-stage biopharmaceutical company leveraging its novel oligonucleotide promoted editing of RNA (OPERA[®]) platform to develop a new class of genetic medicines for rare and highly prevalent diseases, today announced preclinical results for its pipeline program KRRO-111 for the potential treatment of alpha-1 antitrypsin deficiency (AATD) delivered subcutaneously. The results highlight KRRO-111's potential best-in-class profile and its ability to address both the lung and liver manifestations of AATD through a differentiated RNA editing approach.

"In these preclinical studies, KRRO-111 restored functional M-AAT while reducing the toxic Z-AAT that drives liver injury," said Loïc Vincent, PhD, Chief Scientific Officer of Korro. "That dual profile, with a redosable subcutaneous drug presentation, is what we believe a next-generation AATD therapy needs to support all AATD patients."

KRRO-111 is a proprietary GalNAc-conjugated RNA-editing oligonucleotide administered subcutaneously and selectively taken up by liver cells. Once inside the liver cells KRRO-111 harnesses endogenous Adenosine Deaminase Acting on RNA (ADAR) enzymes to correct the pathogenic SERPINA1 transcript, enabling production of M-AAT, while reducing synthesis of mutant Z-AAT.

KRRO-111 exhibited potent RNA editing activity (EC50 ~1 nM) achieving up to 98% editing in mouse and human hepatocytes. In single-dose studies in the C57BL/6-PiZ mouse model, KRRO-111 produced up to 91% SERPINA1 editing, with M-AAT accounting for up to 88% of total circulating AAT, and both responses remaining detectable through at least Day 42 post-dose.

In repeat-dose studies, KRRO-111 produced sustained RNA editing and restoration of circulating M-AAT across multiple subcutaneous dosing regimens. In NSG-PiZ mice, SERPINA1 mRNA editing reached 96%, with M-AAT comprising 93% of total circulating AAT and total AAT concentrations reaching 172 µM (~6-fold vs. vehicle) at Day 57. In immunocompetent C57BL/6-PiZ mice, KRRO-111 reduced whole liver Z-AAT levels by 95% relative to vehicle control, as measured by liquid chromatography tandem mass spectrometry (LC-MS/MS) at Day 57.

"People living with AATD face both lung and liver disease. Augmentation therapy addresses only part of that burden while there is no approved therapy for the liver disease," said Ram Aiyar, PhD, MBA, Chief Executive Officer and President of Korro. "Based on the strength and consistency of these data, we believe KRRO-111 has the potential to become a transformative therapy for both the lung and liver manifestations of AATD, and we look forward to advancing it into the clinic in the first half of 2027."

Regulatory submission for KRRO-111 is expected in the first half of 2027.

About Alpha-1 Antitrypsin Deficiency (AATD)

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. Greater than 95% of severe clinical cases of AATD are homozygous for the PiZ mutation (known as the PiZZ genotype). 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 AAT protein, which remains inadequate to address the lung and liver manifestations of AATD.

About Korro

Korro is a clinical-stage biopharmaceutical company leveraging a novel oligonucleotide promoted editing of RNA (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 (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 Korro 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, 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 REO-based approach, Korro expects to bring its medicines to patients by leveraging its proprietary OPERA platform with precedent delivery modalities, including N-acetylgalactosamine (GalNAc) conjugated for delivery for subcutaneous administration, manufacturing know-how and established regulatory pathways of approved oligonucleotide medicines. Korro is based in Cambridge, Massachusetts. For more information, visit korro.bio.

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LinkedIn, and X (Twitter), 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 best-in-class and best-in-disease potential of, and market opportunity for, KRRO-111 as a treatment for AATD, including its potential to address both the lung and liver manifestations of AATD and to become a transformative therapy, and its potential as a redosable subcutaneous therapy; the extent to which preclinical results for KRRO-111, including M-AAT restoration and reduction and clearance of Z-AAT observed in mouse models, will be predictive of results in humans; KRRO-111's ability to achieve the functional equivalent of a DNA modification without altering the genome; the ongoing GLP toxicology evaluation of KRRO-111; the timing of a regulatory submission for KRRO-111, including the expected submission in the first half of 2027; and the advancement of KRRO-111 into clinical development and reaching and reporting clinical milestones for KRRO-111; 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 a statement is not forward-looking. Forward-looking statements are based on current expectations and assumptions that, while considered reasonable by Korro and its management, 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 associated with pre-clinical studies and conducting clinical trials; risks associated with validating in clinical trials observations from pre-clinical studies, including the risk that results of pre-clinical studies, including in animal models, may not be predictive of results in humans; competition from other companies and therapies, and the absence of head-to-head comparisons supporting any best-in-class potential; risks associated with collaborating with third parties; changes in policies, staffing or priorities at the FDA or other regulatory authorities; 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 Securities and Exchange Commission (SEC), including under the caption "Risk Factors" in Korro's most recent Quarterly Report on Form 10-Q or Annual Report on Form 10-K, 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.

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