Korro Selects KRRO-111 as Development Candidate for the Potential Treatment of Alpha-1 Antitrypsin Deficiency (AATD)

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- Potential best-in-class therapeutic delivered subcutaneously (GalNAc-conjugated oligonucleotide) for a debilitating genetic condition impacting both the liver and lungs
- Demonstrated >90% editing of SERPINA1 transcript *in vivo* translating into ~90% repaired functional alpha-1 antitrypsin (AAT) protein in plasma in a mouse model of AATD
- Demonstrated a near elimination of active Z-protein production while showing progressive clearance of pre-existing aggregates in a mouse model of AATD
- Pre-clinical data highlighted the possibility of repeat dose therapy to achieve the functional equivalent of a DNA modification without altering the genome

CAMBRIDGE, Mass., May 19, 2026 (GLOBE NEWSWIRE) -- Korro Bio, Inc. (Korro) (Nasdaq: KRRO), a 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, today announced the addition of KRRO-111 for the potential treatment of AATD to its pipeline of therapeutic programs in development. KRRO-111 is a proprietary GalNAc-conjugated oligonucleotide that is delivered subcutaneously to the liver cells, where it is engineered to co-opt the hepatocytes' endogenous Adenosine Deaminase Acting on RNA (ADAR) enzyme and repair a pathogenic single nucleotide variant (SNV) on AAT mRNA to restore production of normal AAT protein. This therapeutic profile along with KRRO-111's rapid onset of action and titratable dosing to treat this disorder, position the compound as a potential best-in-class candidate.

"The addition of KRRO-111 for AATD to our pipeline of transformative therapeutic candidates is the culmination of extensive and robust pre-clinical optimization, achieving the highest level of SERPINA1 editing reported to date using RNA editing. It appears to be a level of highly specific protein correction that functionally approaches what would be expected from a permanent specific DNA modification, while preserving the inherent safety advantages of working at the RNA level," commented Ram Aiyar, Ph.D., Chief Executive Officer and President of Korro Bio. "For patients living with AATD, this represents a meaningful step toward a therapy designed to address the root cause of their disease. This is an important clinical and corporate development milestone for the Company as it highlights the power of the OPERA platform to generate potential best-in-class genetic medicines for patients suffering from debilitating diseases. As we advance KRRO-111 for the potential treatment of AATD into the clinic alongside KRRO-121 for the potential treatment of hyperammonemia in patients with urea cycle disorders, we are well-capitalized to report clinical data from both programs."

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 does not adequately address the manifestations of AATD.

In preclinical studies, KRRO-111 demonstrated best-in-class RNA editing where >90% of the AAT transcripts in the liver cells have been corrected, translating into ~90% M-AAT protein. In a repeat-dose study, KRRO-111 (3 mg/kg) was administered to PiZZ mice with a loading phase followed by dosing once every two weeks. KRRO-111 nearly eliminated active Z protein production, reducing non-inclusion Z-AAT by ~95% versus vehicle at both days 28 and 56, reflecting near-complete cessation of Z protein synthesis across the hepatocyte population. Pre-existing aggregates were progressively cleared, with inclusion-associated Z-AAT significantly reduced by approximately 69% at day 28, consistent with autophagic clearance of accumulated protein following successful editing; the residual burden reflects pre-existing disease rather than incomplete editing. By simultaneously increasing circulating M-AAT protein and decreasing pathogenic Z-AAT aggregates in the lung, KRRO-111 has the potential to improve both lung and liver manifestations of the disease.

The Company plans to present additional data from its KRRO-111 program at a future scientific meeting or Company-hosted event.

About Korro

Korro is a 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 oligonucleotides 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 oligonucleotides, 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 oligonucleotide-based approach, Korro expects to bring its medicines to patients by leveraging its proprietary OPERA platform with precedented 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 korrobio.com.

Korro intends to use its Investor Relations website, LinkedIn, and X (Twitter) as 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 and follow @KorroBio on 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 potential of, and market opportunity for, KRRO-111 as a treatment for AATD; KRRO-111's ability to achieve the functional equivalent of a DNA modification without altering the genome; Korro's cash runway and financial resources; the therapeutic potential of the OPERA platform; and reaching and reporting clinical milestones for KRRO-121 and 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 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 associated with pre-clinical studies and conducting clinical trials; risks associated with validating in clinical trials observations from pre-clinical studies; risks associated with collaborating with third parties; other risks associated with 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 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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