Korro Receives European Medicines Agency Orphan Drug Designation for KRRO-110

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CAMBRIDGE, Mass., July 21, 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, announced today that it has been granted orphan drug designation by the European Medicines Agency (EMA) Committee, who adopted a positive opinion on KRRO-110, Korro's investigational medicine for the treatment of Alpha-1 Antitrypsin Deficiency (AATD).

"The EMA orphan drug designation for KRRO-110 is a significant milestone for Korro and highlights an urgent need to bring innovative solutions to people with AATD seeking new, disease-modifying therapies," said Kemi Olugemo, MD, Chief Medical Officer at Korro. "Backed by encouraging preclinical data, KRRO-110 has best-in-class potential for the treatment of AATD. This designation represents an important step toward bringing KRRO-110 to European patients."

In March 2025, KRRO-110 received orphan drug designation from the U.S. Food and Drug Administration for the treatment of AATD. KRRO-110 is currently being evaluated in the Phase 1/2a REWRITE clinical study for AATD, and an interim readout is expected in the second half of 2025.

The EMA grants orphan designation status to medicines intended for the treatment, prevention or diagnosis of life-threatening or chronically debilitating diseases affecting fewer than 5 in 10,000 people in the European Union, and the medicine must be of significant benefit to patients. Orphan drug designation provides companies with various development incentives, including protocol assistance, reduced regulatory fees, and market exclusivity once the medicine is approved.

About REWRITE

REWRITE is a Phase 1/2a two-part single and multiple dose-escalating clinical 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).

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, a potential best-in-class compound based on preclinical data, 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 based on editing RNA for both rare and highly prevalent diseases. 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.

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 timing of data readouts and completion of the Phase 1/2a REWRITE clinical trial; KRRO-110's potential to treat both liver and lung manifestations of AATD; KRRO-110's best-in-class potential; and realizing the incentives offered from the orphan drug designation of KRRO-110; 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 clinical study; risks associated with regulatory oversight of clinical studies, enrollment risks and risks of expanding to other jurisdictions along with other risks inherent in biopharmaceutical development; risks associated with pre-clinical studies and clinical studies; and other risks associated with obtaining regulatory approvals and protecting intellectual property; as well as risks associated with general economic conditions (including recent geopolitical uncertainty and potential supply chain disruptions due to changes in economic policy); and other risks and uncertainties indicated from time to time in Korro's filings with the SEC, including Part I Item 1A. "Risk Factors" in Korro's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission, or 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.

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