Korro Receives Orphan Drug Designation for KRRO-121 From U.S. FDA for the Treatment of Urea Cycle Disorders

November 24, 2026

CAMBRIDGE, Mass., Nov. 24, 2026 (GLOBE NEWSWIRE) -- Korro Bio, Inc. (Korro) (Nasdaq: KRRO), a clinical-stage biopharmaceutical company leveraging its oligonucleotide promoted editing of RNA (OPERA®) platform to develop a new class of genetic medicines for rare and highly prevalent diseases, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for KRRO-121, the Company's GalNAc-conjugated RNA-editing oligonucleotide (REO) in development for the treatment of hyperammonemia in patients with urea cycle disorders (UCDs) and patients with hepatic encephalopathy (HE).

"People living with UCDs are in urgent need for a treatment to manage the debilitating effects from these conditions, and receiving orphan drug designation for KRRO-121 from the FDA is an important development milestone for this potentially transformational therapy, which follows orphan drug designation from the European Medicines Agency and rare pediatric disease designation from the FDA earlier this year," commented Ram Aiyar, PhD, Chief Executive Officer and President of Korro. "KRRO-121 is currently being evaluated in a global Phase 1/2 clinical trial entitled ANCHOR and we look forward to initiating dosing of UCD patients in the first half of 2027, with an interim data readout expected in the second half of 2027."

Orphan Drug Designation is granted by the FDA to support the development of therapies intended to treat rare diseases or conditions affecting fewer than 200,000 people in the United States. Benefits of the designation may include tax credits for qualified clinical trials, exemption from certain FDA user fees, and up to seven years of market exclusivity upon regulatory approval. Orphan Drug Designation does not alter the standard regulatory requirements or approval process.

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 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.

About ANCHOR

ANCHOR (AssessmeNt of Control of Hyperammonemia through Oligonucleotide-based RNA editing) is Korro's global two-part Phase 1/2 clinical trial program evaluating the safety, tolerability, pharmacokinetics (PK) and clinical activity of KRRO-121. Part 1 will include Phase 1 single ascending dose (SAD) and Phase 1 multiple ascending dose (MAD) studies of KRRO-121 in healthy adult volunteers. Part 2 will be a Phase 2 trial in patients with UCDs.

Part 1 SAD, which started in September 2026, is enrolling up to 30 healthy volunteers across up to five cohorts, placebo-controlled at each dose level. Part 1 MAD will evaluate certain selected doses of KRRO-121 in up to 20 healthy volunteers across two cohorts. The primary goal of Part 1 is to evaluate safety, tolerability and PK of KRRO-121.

Part 2 of the ANCHOR program will evaluate KRRO-121 in patients with UCDs, evaluating safety, tolerability, PK and clinical activity. Dosing of UCD patients is expected to start in the first half of 2027, with an interim clinical readout expected in the second half of 2027. For additional information about the ANCHOR program, visit ClinicalTrials.gov (NCT07773246); the subsequent trials in the two-part ANCHOR program will be appended to ClinicalTrials.gov as those trials commence.

About Hyperammonemia

Hyperammonemia, the dangerous accumulation of ammonia in the blood, manifests across multiple indications, including urea cycle disorders (UCDs) and hepatic encephalopathy (HE). UCDs are rare inborn errors of metabolism involving deficiencies of enzymes required for ureagenesis, the process of converting ammonia to urea for excretion. The absence or deficiency of any of the urea cycle enzymes can result in hyperammonemia and an increased risk of hyperammonemic crises (HACs), which can result in severe and permanent neurological symptoms, coma and death. HE is a neuropsychiatric complication of liver disease characterized by cognitive dysfunction and altered consciousness. HE is primarily caused by the liver's inability to adequately detoxify ammonia, typically occurring in patients with liver cirrhosis. This leads to ammonia accumulating in the bloodstream and crossing the blood-brain barrier, causing brain dysfunction that ranges from subtle cognitive impairment to severe confusion and coma.

About KRRO-121

KRRO-121 is a GalNAc-conjugated RNA-editing oligonucleotide for the potential treatment of hyperammonemia in patients with UCDs of any mutational background as well as patients with HE. Utilizing Korro's proprietary OPERA ® platform, KRRO-121 is designed to generate a stabilized, *de novo* glutamine synthetase (GS) protein, a critical enzyme involved in ammonia clearance. This synthetic rescue approach is designed to augment ammonia clearance in hyperammonemia-driven diseases such as UCDs and HE. Korro's preclinical data support the potential for KRRO-121 to be a pan-UCD treatment that may control ammonia levels, along with the potential to enable diet liberalization, reduce dependence on nitrogen scavengers

and lower the risk of HACs. KRRO-121 also has the potential to enhance ammonia control in HE patients, which may reduce the risk of recurrent HE episodes. KRRO-121 may offer infrequent subcutaneous dosing, which, if confirmed in clinical studies, could offer significant improvement over current treatments that require multiple daily doses. KRRO-121 has the potential to be a differentiated, first-in-class, disease-modifying therapy for both UCD and HE.

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 design, conduct, enrollment and progression of the ANCHOR Phase 1/2 clinical trial program, including the timing of results from ANCHOR; the potential of, and market opportunity for, KRRO-121, including its potential to be a transformational therapy for people living with UCDs; the potential benefits of orphan drug designation and rare pediatric disease designation for KRRO-121; the extent to which preclinical findings for KRRO-121 translate into clinical results; the therapeutic potential of the OPERA platform; Korro's ability to provide additional treatment options for, and improve outcomes for, patients living with UCDs; reaching clinical milestones across multiple programs; 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 preclinical studies and conducting clinical trials; risks associated with validating in clinical trials observations from preclinical 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 Part II Item 1A. "Risk Factors" in Korro's Quarterly Report on Form 10-Q filed with the SEC on August 6, 2026, 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
Malini Chatterjee, PhD
Blueprint Life Science Group
mchatterjee@bplifescience.com or ir@korrobio.com
917.330.4269