Korro Reports Third Quarter Financial Results, Provides Updates on KRRO-110 in Alpha-1 Antitrypsin Deficiency and Additional Pipeline Programs

November 12, 2025

—KRRO-110 produced functional protein in Alpha-1 Antitrypsin Deficiency (AATD) patients

—KRRO-110 did not reach projected levels of functional protein following a single administration

—Pivoting to GalNAc delivery for patients with AATD; development candidate nomination expected in the first half of 2026

—Nominated KRRO-121, designed to create a de novo protein variant to activate a biological pathway for patients with hyperammonemia

—Reports third quarter 2025 financial results; ended third quarter 2025 with $102.5 million in cash, cash equivalents and marketable securities; extending cash runway into second half of 2027 by implementing a strategic restructuring

CAMBRIDGE, Mass., November 12, 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 provided a program update for its Phase 1/2a REWRITE clinical trial of KRRO-110 in AATD, reported financial results for the third quarter of 2025, and provided a business update.

"Today, we announced that KRRO-110 generated functional M-AAT protein in AATD patients. We're encouraged by the evidence of clinical activity, which we believe confirms our ability to edit RNA and produce therapeutic proteins in humans. While a single administration of KRRO-110 achieved functional protein production, it did not achieve the protein levels we projected based on preclinical data. Initial analysis indicates differences in the pharmacokinetics of the delivery components observed between healthy volunteers and AATD patients. The valuable insights gained from REWRITE, combined with the significant progress we've made in potency, have informed our strategic decision to advance a GalNAc-conjugated construct for AATD. We are on track for a potential development candidate nomination in the first half of 2026." said Ram Aiyar, Ph.D., CEO and President of Korro Bio.

"In addition, we have nominated our next development candidate, KRRO-121, a GalNAc-conjugated construct that activates a biological pathway by creating a de novo variant, for patients with hyperammonemia. This marks our first step in expanding our proprietary RNA editing platform beyond protein repair. We are working to advance KRRO-121 and a GalNAc version for AATD patients into the clinic in the second half of 2026 and in 2027, respectively."

"To focus our resources on generating clinical data and advancing additional GalNAc-conjugated programs targeting the liver, we are implementing a strategic restructuring that reduces our workforce by approximately a third while extending our cash runway into the second half of 2027. We are grateful for our employees and their commitment. A special thanks to the AATD community, the participants in the REWRITE study, and the investigators who are continuing to work with us as we evaluate next steps for the program. We remain committed to our mission of delivering transformative genetic medicines to patients."

REWRITE Clinical Trial Update:

The REWRITE Phase 1/2a clinical trial is a two-part single and multiple-dose escalating study evaluating the safety and tolerability of KRRO-110, including healthy adults and clinically stable AATD patients with the PiZZ genotype. Korro has completed all six planned single ascending dose (SAD) healthy volunteer (HV) cohorts. Each HV cohort consisted of six participants, with four receiving active drug and two receiving placebo. Doses of KRRO-110 tested in the HVs (n=24) include 0.04, 0.1, 0.2, 0.4, 0.8 and 1.2 mg/kg, with the primary objective to evaluate safety and tolerability. KRRO-110 is currently being evaluated in two AATD patient cohorts at 0.6 mg/kg (n=3) and 0.8 mg/kg (n=4), with seven patients dosed. The AATD patient cohorts are open label with up to four patients in each cohort. There are currently no plans to complete additional SAD patient cohorts for KRRO-110. Korro is evaluating the totality of the clinical data to evaluate the next steps, if any, for KRRO-110 in the multiple-ascending dose (MAD) portion of the REWRITE clinical trial.

Key Findings Include:

KRRO-110 Safety Observations (as of the November 6, 2025 Data Cutoff Date):

- No dose-limiting toxicities or treatment emergent serious adverse events observed.
- Mild-to-moderate infusion-related reactions (IRRs) observed in two healthy volunteers at the highest dose of 1.2 mg/kg, two AATD patients at 0.6 mg/kg, and two AATD patients at 0.8 mg/kg.
- All IRRs resolved within 24 hours post KRRO-110 dosing; intervention was limited to antipyretics and antihistamines.
- KRRO-110 safety profile is consistent with Lipid Nanoparticles (LNP) infusion-related class effects.

KRRO-110 Pharmacodynamic and Pharmacokinetic Observations (as of the November 6, 2025 Data Cutoff Date):

- Across five AATD patients in the two SAD cohorts evaluable with turbidimetry, the greatest peak total AAT protein was approximately 10 μM and the greatest increase of total AAT protein from baseline was approximately 3 μM. The total AAT protein levels following single-dose administration did not reach the protective threshold of 11 μM.
- In three of the AATD patients dosed with 0.8 mg/kg evaluable with LC/MS, functional M-AAT protein was observed in each of the patients following administration of KRRO-110. The greatest increase of M-AAT protein from baseline observed at any time point was approximately 2 μM. Functional M-AAT protein lasted up to four weeks for the first patient evaluable with LC/MS, consistent with durability of editing and endogenous M-AAT protein half-life.

- Pharmacokinetic differences in the components of KRRO-110 in plasma were observed between HV and AATD patients, with apparent faster disassociation of KRRO-110, suggesting variability of this formulation in AATD patients compared to HV following a single dose.
- No evidence of bystander editing observed to date, suggesting high specificity, based on LC/MS data on M-AAT and Z-AAT.

KRRO-110 Regulatory Milestones Validate OPERA Platform Potential:

- First-ever RNA editing technology to receive Investigational New Drug clearance by the U.S. Food and Drug Administration (FDA) to Korro's knowledge.
- Fast Track designation granted by the FDA.
- Orphan Drug Designation granted by both the FDA and European Medicines Agency.

Pipeline Prioritization and Business Updates:

Prioritizing High-Potential GalNAc-Conjugated Programs Targeting the Liver:

- Korro has nominated KRRO-121 as its next development candidate, for the potential treatment of patients with hyperammonemia, including patients with urea cycle disorders (UCD) and hepatic encephalopathy (HE).
    - KRRO-121 is intended to treat all UCD patients regardless of their mutational background, representing a pan-UCD patient population.
    - KRRO-121 is also intended to prevent or reduce the number of hyperammonemic crises in HE patients.
    - KRRO-121 will be administered subcutaneously and is designed to create a de novo protein variant to activate a biological pathway.
    - Regulatory filing to enable commencement of first-in-human trial for KRRO-121 is anticipated in the second half of 2026.
- Pivoting to a GalNAc-conjugated construct for AATD, with nomination of a development candidate in the first half of 2026.
- Advancing additional GalNAc-conjugated programs for subcutaneous delivery targeting the liver in cardiometabolic indications.

Novo Collaboration Update

- Korro amended its research collaboration and license agreement with Novo Nordisk A/S (Novo Nordisk). This amendment establishes a 12-month pause to reassess the rationale for the current target under the first research program.

Workforce Reduction

- Approximately 34% reduction in workforce impacting all levels of the organization.
- Extending cash runway into second half of 2027 to provide sufficient capital to deliver clinical data from KRRO-121, advance at least one additional program, and execute partnership discussions to broaden pipeline development.
- Korro estimates that it will incur one-time restructuring charges of approximately $2.4 million including employee severance, benefits, and related termination costs, the majority of which Korro expects to recognize during the fourth quarter of 2025.

Departure of CMO

- Resignation of Dr. Kemi Olugemo, Chief Medical Officer, effective November 12, 2025. The board of directors of Korro and the company are thankful to Dr. Olugemo for her service at Korro.

Third Quarter 2025 Financial Results:

Cash Position: Cash, cash equivalents and marketable securities were $102.5 million as of September 30, 2025, compared to $163.1 million as of December 31, 2024. Korro expects its cash, cash equivalents and marketable securities as of September 30, 2025 will fund operating expenses and capital expenditure requirements into the second half of 2027.

Collaboration Revenue: Collaboration revenue was $1.1 million for the three months ended September 30, 2025, as compared to no collaboration revenue for the three months ended September 30, 2024. The increase was due to collaboration revenue earned in the third quarter of 2025 from Korro's collaboration with Novo Nordisk.

Research and Development (R&D) Expenses: R&D expenses were $13.8 million for the three months ended September 30, 2025, as compared to $16.0 million for the three months ended September 30, 2024. The decrease was driven primarily by decreases in KRRO-110 external expenses and other research and pre-development candidate expenses.

General and Administration (G&A) Expenses: G&A expenses were $6.5 million for the three months ended September 30, 2025, as compared to $7.3 million for the three months ended September 30, 2024. The decrease was primarily due to a $0.7 million decrease in professional services expenses.

Net Loss: Korro's net loss was $18.1 million for the three months ended September 30, 2025, as compared to $21.0 million for the three months ended September 30, 2024.

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.

About Hyperammonemia and KRRO-121

Hyperammonemia is due to insufficient clearance of ammonia from the blood stream. It manifests in multiple indications such as urea cycle disorders (UCD) and hepatic encephalopathy (HE). UCD are rare inborn errors of metabolism involving deficiencies of enzymes required for ureagenesis. The absence or deficiency of any of the urea cycle enzymes results in increased ammonia in the blood to dangerous levels. 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 detoxify ammonia. 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, significantly impacting patients' quality of life. KRRO-121 is an RNA-editing oligonucleotide conjugated with GalNAc for the potential treatment of hyperammonemia in patients with UCD of any mutational background in adults and adolescents as well as patients with HE. Utilizing Korro's proprietary OPERA™ platform, KRRO-121 is a GalNAc conjugated oligonucleotide designed to stabilize a critical enzyme involved in reducing ammonia levels.

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 reasons a single-dose administration of KRRO-110 did not reach protective protein levels in AATD patients; the pipeline in a product potential for KRRO-121; the timing of the regulatory filing for KRRO-121; the potential of Korro's GalNAC-conjugated programs targeting the liver, including KRRO-121 and GalNAc delivery for AATD patients; Korro's ability to activate a biological pathway with RNA editing; timing of nominating a development candidate for Korro's GalNAc-conjugated program for AATD; the costs of Korro's workforce reduction, and the benefits thereof; Korro's collaboration agreement with Novo Nordisk; and Korro's cash runway and uses thereof; 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 realizing the benefits of its workforce reduction; estimating the costs of such workforce reduction; the impact of the workforce reduction on operations; 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 SEC, including Part I Item 1A. "Risk Factors" in Korro's Quarterly Report on Form 10-Q filed with the SEC on the date hereof, 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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Korro Bio, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue:				
Collaboration revenue	$ 1,090	$ —	$ 5,100	$ —

Operating expenses:				
Research and development	13,820	15,964	54,590	46,674
General and administrative	6,507	7,328	21,969	22,196
Total operating expenses	20,327	23,292	76,559	68,870
Loss from operations	(19,237)	(23,292)	(71,459)	(68,870)
Other income:				
Other income, net	1,176	2,284	4,242	6,526
Total other income, net	1,176	2,284	4,242	6,526
Loss before benefit (provision) for income taxes	(18,061)	(21,008)	(67,217)	(62,344)
Benefit (provision) for income taxes	—	9	(1)	(38)
Net loss	$ (18,061)	$ (20,999)	$ (67,218)	$ (62,382)
Other comprehensive income:				
Unrealized gain (loss) on available-for-sale marketable securities	48	593	(29)	614
Foreign currency translation adjustments, net	(11)	(40)	(25)	(40)
Comprehensive loss	$ (18,024)	$ (20,446)	$ (67,272)	$ (61,808)
Net loss per share, basic and diluted	$ (1.92)	$ (2.26)	$ (7.16)	$ (7.11)
Weighted-average shares used in computing net loss per share, basic and diluted	9,391,559	9,303,218	9,388,816	8,771,743

Korro Bio, Inc.
Selected Condensed Consolidated Balance Sheet Data
(in thousands)
(unaudited)

	September 30, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 102,493	$ 163,054
Working capital[(1)]	83,588	116,572
Total assets	161,550	226,240
Total liabilities	62,518	65,825
Total stockholders' equity	99,032	160,415

(1) Working capital is defined as current assets less current liabilities.

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