# Korro Bio Highlights Data for its Lead Program in Alpha-1 Antitrypsin Deficiency (AATD) and Progress Across its RNA Editing Portfolio

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- KRRO-110 has a potentially best-in-class profile with secretion of ~50μM functional AAT protein after a single dose in a preclinical mouse model
- Evidence of greater than 40% editing of SERPINA1 RNA in non-human primates (NHPs) further supports the potential translation of KRRO-110 in humans
- Demonstrated greater than 50% editing using GalNAc conjugates in the liver in a preclinical mouse model
- Generated a *de novo* protein variant in NHPs, further highlighting the potential power of our RNA editing approach

CAMBRIDGE, Mass., Jan. 18, 2024 (GLOBE NEWSWIRE) -- Korro Bio, Inc. (Korro) (Nasdaq: KRRO), a biopharmaceutical company focused on developing a new class of genetic medicines for both rare and highly prevalent diseases, presented new data for KRRO-110 and reported progress across its RNA editing portfolio at the J.P. Morgan Healthcare Conference on January 9, 2024.

"KRRO-110 data showed the highest levels of corrected protein in a human transgenic PiZ mouse model, supporting a potentially best-in-class therapeutic for AATD patients," said Chief Executive Officer and President, Ram Aiyar, Ph.D. "Beyond AATD, our ability to generate *de novo* mutations to activate a biological pathway is uncharted in the field of RNA editing. The progress we have made across our platform and pipeline underscores our commitment to bringing differentiated therapeutic options to patients with significant unmet medical needs."

**Pipeline updates**

*KRRO-110*

As announced on December 7, 2023, Korro selected KRRO-110 as its first development candidate for the potential treatment of AATD, an inherited genetic disorder caused by single nucleotide variants (SNVs) in the SERPINA1 gene. AATD can lead to severe progressive lung disease, including emphysema and chronic obstructive pulmonary disease (COPD), and severe liver disease leading to inflammation, cirrhosis, and fibrosis. Preclinical data to date for KRRO-110 demonstrated:

- High specificity with no bystander effects in MZ human primary hepatocytes
- Intravenous administration at 2 mg/kg resulted in secretion of ~50μM functional AAT as early as 7 days post-single dose in a human transgenic PiZ mouse model
- Increase in AAT protein and the inhibition of elastase activity were sustained through week 9 when dosed every 2 weeks, demonstrating durability in mice
- Greater than 40% editing in NHPs utilizing an earlier generation oligonucleotide designed to edit a surrogate SERPINA1 RNA target site

Preclinical development of KRRO-110 is ongoing in preparation for a regulatory filing expected in the second half of 2024, with a potential interim clinical readout in the second half of 2025.

*Platform Update*

Korro's proprietary RNA editing platform, OPERA™, integrates a deep understanding of adenosine deaminase acting on RNA (ADAR) enzymology with expertise in oligonucleotide chemistry, machine learning optimization of oligonucleotides and fit-for-purpose delivery. CHORDs™, or **C**ustomized **H**igh-fidelity **O**ligonucleotides for **R**NA **D**eamination, are single-stranded, anti-sense oligonucleotides designed to have high target efficiency and specificity by leveraging the pillars of OPERA.

- Developed proprietary oligonucleotide chemistry using structural biology insights that enhances *in vivo* potency and durability of CHORDs
- Computational efficiency enables rapid iteration of CHORDs across targets
- Using CHORDs, Korro achieved greater than 50% editing *in vivo* utilizing both a ligand-based GalNAc conjugate and an LNP-based delivery approach

*Creating De Novo Mutation to Alter Protein Function*

In addition to repairing pathogenic SNVs, CHORDs can be used to engineer *de novo* SNVs and change amino acids on proteins to endow them with desired altered properties while still preserving their broader functional capabilities. In preclinical studies, Korro has demonstrated the ability to:

- Activate a transcription factor in an undisclosed target
  - Demonstrated activation of a transcription factor by creating a *de novo* protein variant resulting in sustained downstream activity in NHPs lasting longer than 21 days, demonstrating potential to preserve transcription factor function
- Selectively modulate TDP-43 to reduce protein aggregation
  - TDP-43 is a protein associated with ALS, FTD and other neurodegenerative diseases. A single RNA edit to TDP-43 is predicted to lead to the synthesis of a protein variant that does not aggregate, thereby preserving normal function and protecting downstream activity essential for neuronal health
- Modulate ion channels to within physiological levels in $Na_V1.7$
  - $Na_V1.7$ is a voltage-gated sodium channel that is essential for pain sensation and electrical signaling in the central nervous system. Korro has demonstrated that site-specific changes are sufficient to decrease the activity of $Na_V1.7$. This approach has the potential to deliver potent analgesic activity without the dose-limiting toxicities that have been observed by sodium channel blockers

**J.P. Morgan Healthcare Conference Presentation**

A replay of Korro's presentation at the J.P. Morgan Healthcare Conference can be accessed from the Investor section of Korro's website at www.korrobio.com or directly with this link. The replay is available for 30 days following the January 9, 2024 conclusion of the presentation.

**About Korro**

Korro is a biopharmaceutical company focused on developing a new class of genetic medicines for both rare and highly prevalent diseases using its proprietary RNA editing platform. Korro is generating a portfolio of differentiated programs that are designed to harness the body's natural RNA editing process to effect 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, Mass. For more information, visit korrobio.com.

**Forward-Looking Statements**

Certain statements in this press release may constitute "forward-looking statements". 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: Korro's RNA editing technology and the benefits of OPERA; the market opportunity for KRRO-110 and potential benefits over other AATD modalities; the potential of KRRO-110 to be a best-in-class drug candidate for AATD; the potential safety and efficacy of KRRO-110; Korro's expected cash runway and plans for discovery and preclinical studies, as well as clinical trials, including timing of regulatory filings and data readouts and other developments or results in connection therewith. 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 inherent in biopharmaceutical development; risks associated with pre-clinical studies and clinical trials; and other risks associated with obtaining regulatory approvals and protecting intellectual property; as well as risks associated with general economic conditions; the inability to recognize the anticipated benefits of the recently completed merger, which may be affected by, among other things, competition, Korro's ability to grow and manage growth profitably, maintain relationships with customers and suppliers and retain key employees; costs related to merger; the possibility that Korro may be adversely affected by other economic, business, and/or competitive factors; other risks and uncertainties indicated from time to time in Korro's filings with the SEC, including in Exhibit 99.2 to its Current Report on Form 8-K filed with the SEC on November 6, 2023, 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. 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 their 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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