Keros Therapeutics Announces Initial Topline Results from the Phase 1 Clinical Trial of KER-065 in Healthy Volunteers

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- *Trial achieved key objectives for safety, tolerability, pharmacokinetics and pharmacodynamics*
- *Clinical data from this trial, together with preclinical data, support the therapeutic potential of KER-065 for broad impact in Duchenne muscular dystrophy ("DMD") and other neuromuscular indications*
- *Keros plans on advancing KER-065 into a Phase 2 clinical trial in DMD, subject to positive regulatory interaction*
- *Keros to host a conference call and webcast today, March 31, 2025, at 8:00 a.m. ET*

LEXINGTON, Mass., March 31, 2025 (GLOBE NEWSWIRE) -- Keros Therapeutics, Inc. ("Keros") (Nasdaq: KROS), a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapeutics to treat a wide range of patients with disorders that are linked to dysfunctional signaling of the transforming growth factor-beta ("TGF-ß") family of proteins, today announced initial topline results from the Phase 1 clinical trial of KER-065 in healthy volunteers. Topline results from this ongoing trial are through the multiple ascending dose ("MAD") treatment period (Day 85).

"We are pleased to report topline results that met the key objectives of the Phase 1 clinical trial and provided important insights to inform the development of KER-065 for patients with DMD," said Jasbir S. Seehra, Ph.D., Chair and Chief Executive Officer. "Considering the limitations of currently available therapies, the need for additional treatments in DMD remains high, and KER-065 has the potential to address multiple aspects of the disease, including across important tissues and underlying genetic deficiencies. Our strong financial foundation enables us to continue advancing our promising pipeline of novel therapeutics, and we look forward to engaging with regulators towards the aim of moving KER-065 to a Phase 2 clinical trial in the first quarter of 2026."

Key findings of this trial as of a February 6, 2025 data cut-off date include the following:

- KER-065 was generally well-tolerated, with no major safety signals observed to date
  - No serious adverse events or dose-limiting toxicities reported
- Evidence for activin inhibition across tissues of interest, as KER-065 elicited:
  - Increases in bone specific alkaline phosphate (BSAP), a biomarker of bone formation, and decreases in C-Terminal Telopeptide (CTX), a biomarker of bone resorption
  - Increases in adiponectin, a biomarker of fat mobilization, and decreases in leptin, a biomarker of fat mass
  - Changes in body composition, as demonstrated by increases in bone mineral density and muscle mass and decreases in fat mass, which in totality were consistent with activin inhibition

"We observed evidence of activin inhibition based on multiple biomarkers and body composition data. These data, coupled with preclinical and mechanistic insights on the pivotal role of the activin pathway in neuromuscular pathobiology, demonstrate the exciting therapeutic potential of KER-065 in DMD and other neuromuscular disorders," said Yung H. Chyung, M.D., Chief Medical Officer.

Keros plans on engaging with regulatory authorities, starting in the third quarter of 2025. Subject to the outcome of these regulatory interactions, Keros expects to initiate a Phase 2 clinical trial of KER-065 in patients with DMD in the first quarter of 2026.

Conference Call and Webcast Information

Keros will host a conference call and webcast today, March 31, 2025, at 8:00 a.m. Eastern time. The conference call will be webcast live at: https://event.webcasts.com/starthere.jsp?ei=1713593&tp_key=5723864d86. The live teleconference may be accessed by dialing (877) 407-0309 (domestic) or (201) 389-0853 (international). An archived version of the call will be available in the investors section of the Keros website at http://ir.kerostx.com for 90 days following the conclusion of the call.

About the KER-065 Phase 1 Clinical Trial

The KER-065 Phase 1 clinical trial is a randomized, double-blind, placebo-controlled, two-part dose escalation (single ascending dose and MAD) trial in healthy volunteers. The primary objectives of this trial were to assess safety, tolerability and pharmacokinetics of KER-065. Exploratory endpoints include assessments of the pharmacodynamic effect on bone, adipose, muscle, cardiac tissue and fibrosis.

About KER-065

KER-065 is a novel ligand trap comprised of a modified ligand-binding domain derived from activin receptor type IIA and activin receptor type IIB that is fused to the portion of the human antibody known as the Fc domain. KER-065 is designed to act as a ligand trap and inhibit the biological effects of myostatin and activin A, two ligands that signal through activin receptors, to increase skeletal muscle regeneration, increase muscle size and strength, reduce body fat, reduce fibrosis of the skeletal muscle and increase bone strength. We are developing KER-065 for the treatment of neuromuscular diseases, with an initial focus on DMD.

About Duchenne Muscular Dystrophy (DMD)

DMD is the most common form of muscular dystrophy and results in muscle degeneration and premature death. DMD results from the lack of functional dystrophin protein that helps promote myofiber stability, caused by a gene mutation. The lack of dystrophin, an important structural component of muscle cells, causes muscle cells to have increased susceptibility to damage and to progressively die. Additionally, the absence of dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and the replacement of muscle with fibrotic and fatty tissue. The replacement of muscle fibers with fatty and fibrotic tissue leads to progressive loss of muscle strength and function leading to immobility and respiratory and cardiac complications. In DMD patients, heart muscle cells progressively die and are replaced with scar tissue. This cardiomyopathy eventually leads to heart failure, which is currently the leading cause of death among those with DMD. The National Organization for Rare Disorders estimates that approximately one in every 3,500 male births is affected by DMD worldwide.

About Keros Therapeutics, Inc.

Keros is a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapeutics to treat a wide range of patients with disorders that are linked to dysfunctional signaling of the TGF-ß family of proteins. Keros is a leader in understanding the role of the TGF-ß family of proteins, which are master regulators of the growth, repair and maintenance of a number of tissues, including blood, bone, skeletal muscle, adipose and heart tissue. By leveraging this understanding, Keros has discovered and is developing protein therapeutics that have the potential to provide meaningful and potentially disease-modifying benefit to patients. One of Keros' product candidates, cibotercept (KER-012), is being developed for the treatment of pulmonary arterial hypertension and for the treatment of cardiovascular disorders. Keros' second product candidate, KER-065, is being developed for the treatment of neuromuscular diseases. Keros' most advanced product candidate, elritercept (KER-050), is being developed for the treatment of low blood cell counts, or cytopenias, including anemia and thrombocytopenia, in patients with myelodysplastic syndrome and in patients with myelofibrosis.

Cautionary Note Regarding Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Words such as "anticipates," "believes," "continue," "expects," "enable," "potential" and "will" or similar expressions are intended to identify forward-looking statements. Examples of these forward-looking statements include statements concerning: Keros' expectations regarding its strategy and timing of its Phase 2 clinical trial for KER-065, including its plans to engage with regulatory authorities; Keros' ability to advance its pipeline of novel therapeutics; and the potential therapeutic benefits of KER-065. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These risks and uncertainties include, among others: Keros' limited operating history and historical losses; Keros' ability to raise additional funding to complete the development and any commercialization of its product candidates; Keros' dependence on the success of its product candidates, cibotercept, KER-065 and elritercept; that Keros may be delayed in initiating, enrolling or completing any clinical trials; competition from third parties that are developing products for similar uses; Keros' ability to obtain, maintain and protect its intellectual property; and Keros' dependence on third parties in connection with manufacturing, clinical trials and preclinical studies.

These and other risks are described more fully in Keros' filings with the Securities and Exchange Commission ("SEC"), including the "Risk Factors" section of the Company's Annual Report on Form 10-K, filed with the SEC on February 26, 2025, and its other documents subsequently filed with or furnished to the SEC. All forward-looking statements contained in this press release speak only as of the date on which they were made. Except to the extent required by law, Keros undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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