Keros Therapeutics Presents Clinical Data from its Elritercept (KER-050) Program at the 29th Annual Hybrid Congress of the European Hematology Association

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- *Elritercept continued to demonstrate a durable transfusion independence in lower-risk myelodysplastic syndromes, including in patients with high transfusion burden*
- *Durable clinical responses were associated with improvements in patient-reported measures of fatigue*
- *Data from ongoing Phase 2 clinical trial in myelofibrosis continue to demonstrate that elritercept can not only ameliorate ineffective hematopoiesis and address cytopenias, but also provide broader clinical benefit, as supported by observed reductions in spleen volume and improved total symptom scores*
- *Keros will host a corporate update call and webcast today, June 17, 2024, at 8:00 a.m. ET*

LEXINGTON, Mass., June 17, 2024 (GLOBE NEWSWIRE) -- Keros Therapeutics, Inc. (“Keros” or the “Company”) (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 that it presented additional data from its two ongoing Phase 2 clinical trials of elritercept (KER-050), one in patients with very low-, low-, or intermediate-risk myelodysplastic syndromes (“MDS”) and one in patients with myelofibrosis (“MF”), at the 29th Annual Hybrid Congress of the European Hematology Association (“EHA”), held in person in Madrid, Spain and virtually from June 13 through 16, 2024. In addition, Keros presented preclinical data showing that, in an animal model of MF, a research form of elritercept promoted erythropoiesis, mitigated anemia associated with MF, improved anemia associated with ruxolitinib therapy and improved muscle mass and function.

“The data we presented at EHA continues to show an encouraging broad profile of elritercept and supports its potential to treat not just the disease-associated cytopenias, but also impact the pathogenesis of MDS and MF,” said Jasbir S. Seehra, Ph.D., President and Chief Executive Officer. “We are excited by the results we presented, including the durability of transfusion independence observed with elritercept, and are excited to progress towards initiating a registrational Phase 3 clinical trial in MDS following positive feedback from the U.S. Food and Drug Administration.”

Select Clinical Presentations

- *Durable Clinical Benefit with Elritercept (KER-050) Treatment: Findings From an Ongoing Phase 2 Trial in Participants with Lower-Risk MDS*

This ongoing, open-label, two-part, Phase 2 clinical trial is evaluating elritercept in patients with very low-, low-, or intermediate-risk MDS. As of April 3, 2024 (the “data cut-off date”), 87 patients had received at least one dose of elritercept at the recommended Part 2 dose (“RP2D”) (collectively, the “safety population”). Of these patients in the safety population, 81 had completed at least 24 weeks of treatment or discontinued as of the data cut-off date (collectively, the modified intent-to-treat 24-week population, or the “mITT $_{24}$ patients”). Data for hematological response and markers of hematopoiesis were presented from exploratory analyses of these mITT$_{24}$ patients. All data presented from this trial is as of the data cut-off date.

Of the 87 patients in the safety population, 57.5% (n=50) were high transfusion burden (“HTB”) while 25.3% (n=22) were low transfusion burden and 17.2% (n=15) were non-transfused (“NT”).
Elritercept was observed to be generally well-tolerated in the safety population. There were three cases of fatal treatment-emergent adverse events (“TEAEs”) in the trial that were all deemed unrelated to treatment. The most commonly reported TEAEs (in ≥15% of patients) were diarrhea, fatigue, dyspnea, dizziness, COVID-19, nausea and anemia. No patients had progressed to acute myeloid leukemia.

55.6% (n=45/81) of the mITT$_{24}$ patients achieved an overall erythroid response over the first 24 weeks of treatment, which is defined as meeting either modified International Working Group 2006 Hematological improvement-erythroid (“HI-E”) or transfusion independence (“TI”) for at least eight weeks in transfusion-dependent patients who required ≥ 2 red blood cell (“RBC”) units transfused at baseline.

Additional data from the mITT$_{24}$ patients include:

- 41.3% (n=26/63) of the TI-evaluable patients achieved TI for at least eight weeks over the first 24 weeks of treatment. 16 of those 26 patients (61.5%) achieved TI for at least 24 weeks over the first 48 weeks of treatment.
- Of the patients with HTB, 34.8% (n=16/46) achieved TI for at least eight weeks during the first 24 weeks of treatment. Eight of those 16 patients (50.0%) achieved TI for at least 24 weeks over the first 48 weeks of treatment.

- Of the TI-evaluable patients with baseline erythropoietin level less than 500 U/L, 50.0% (n=25/50) achieved TI for at least eight weeks over the first 24 weeks of treatment. Of the TI-evaluable patients with baseline erythropoietin level less than 500 U/L and HTB, 42.9% (n=15/35) achieved TI for at least eight weeks over the first 24 weeks of treatment.
- The median duration of transfusion independence was not met as of the data-cutoff date. 61.5% (n=16/26) of patients with a TI response had ongoing TI as of the data-cutoff. Of the patients that achieved TI, 42.3% (n=11/26) had responses of greater than one year, with all ongoing as of the data cut-off date.

The FACIT-Fatigue scale, a measure of self-reported fatigue and its impact upon daily activities and function, was utilized to assess health-related quality of life in 62 of the $mITT_{24}$ patients who were TI-evaluable and with baseline FACIT-Fatigue assessment. A difference of three in the FACIT-Fatigue scale is considered a minimally clinically important difference. In this group, patients who achieved TI had durable and clinically meaningful improvements in self-reported fatigue. Patients achieving TI of 24 weeks or longer had a mean change from baseline of 6.6 (n=12) versus patients who did not achieve TI of at least 24 weeks, who reported a mean change from baseline of -2.7 (n=25), for a mean difference of 9.4.

The majority of patients enrolled in this ongoing trial had HTB and/or multi-lineage dysplasia, indicating a difficult-to-treat trial population. Durable TI responses continue to be observed in a broad range of patients with lower-risk MDS, including in those with HTB, which support the potential for elritercept to ameliorate ineffective hematopoiesis across multiple lineages in patients with MDS. Patients who achieved TI showed clinically meaningful improvements in FACIT-Fatigue scores, indicating that elritercept may improve quality of life in patients with lower-risk MDS.

- *Elritercept (KER-050) Demonstrated Potential to Treat Myelofibrosis and Mitigate Ruxolitinib-Associated Cytopenias in the Phase 2 RESTORE Trial*

This ongoing, open-label, two-part Phase 2 clinical trial is evaluating elritercept administered with or without ruxolitinib in patients with MF who have anemia and were either currently on, failed, or ineligible for ruxolitinib at baseline. Safety data are presented for all patients that received at least one dose of elritercept (n=54) as of the data cut-off date. Evaluations of markers of hematopoiesis and anemia over 12 weeks, along with measurements of spleen volume and symptom scores (by the MF-symptom assessment form-Total Symptom Score, or “MF-SAF-TSS”) over 24 weeks, were presented for dose levels 1 through 4 in Part 1 and the RP2D, ranging from 0.75 mg/kg to 5.0 mg/kg (collectively, the “efficacy evaluable patients”). Enrollment of Part 1 of the trial, the dose escalation portion, is complete. Part 2, the dose expansion portion, is open and enrolling with a RP2D of 3.75 mg/kg with the option to up-titrate to 5.0 mg/kg. All data presented from this trial is as of the data cut-off date.

Elritercept was generally well-tolerated by the safety population. There were four cases of fatal TEAEs in the trial that were each deemed unrelated to treatment. The most commonly reported TEAEs (in ≥15% of patients) were thrombocytopenia and diarrhea. The majority of treatment-related TEAEs were mild to moderate, with three patients experiencing Grade 3 or higher treatment-related TEAEs.

Additional data from the efficacy evaluable patients include:

- Increases in hemoglobin were observed in the majority of evaluable non-transfusion dependent patients in both arms over a 12-week period within the first 24 weeks, suggesting that elritercept has the potential to address anemia due to MF and ruxolitinib-associated anemia.
- 60.6% (n=20/33) of patients that received at least three RBC units per 12 weeks at baseline in both arms and all dose levels tested showed reductions in transfusion burden over 12 weeks within the first 24 weeks. 60% (n=12/20) of the patients who showed reductions in transfusion burdens had a reduction of 50% or greater in the number of transfusions.
    - Of the patients receiving 3.0 mg/kg of elritercept or higher in combination with ruxolitinib, 72.7% (n=8/11) had reduction of 50% or greater and 45.5% (n=5/11) of patients achieved TI.
- At Week 24, some reduction in spleen volume was observed in 52.9% (n=9/17) of patients with baseline spleen size ≥ 450 cm^3 and a Week 24 spleen assessment, including three patients who had reductions of 35% or greater. Reductions in spleen volume in the combination arm generally occurred without an increase in ruxolitinib dose.
- At Week 24, some reduction in disease symptoms was observed in a majority of patients with at least two symptoms with an average score ≥ 3 or an average total score of ≥ 10 on the MF-SAF-TSS questionnaire at baseline and a week 24 MF-SAF-TSS assessment. Three patients had reductions of at least 50%, including two in the monotherapy arm and one in the combination arm.

The data support the potential of elritercept to ameliorate ineffective hematopoiesis and address cytopenias due to MF and associated with ruxolitinib, and provide broader clinical benefit in patients, as supported by the observed reduction in spleen volume and improvement in total symptom scores.

Conference Call and Webcast Information

Keros will host a corporate update conference call and webcast today, June 17, 2024, at 8:00 a.m. Eastern time, to discuss the additional data from its two ongoing Phase 2 clinical trials of elritercept, one in patients with MDS and one in patients with MF, as well as additional updates to the Company's pipeline.

The conference call will be webcast live at: https://event.webcasts.com/starthere.jsp?ei=1673414&tp_key=3e89bee7b4. 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 https://ir.kerostx.com/ for 90 days following the conclusion of the call.

About the Ongoing Phase 2 Clinical Trial of Elritercept in Patients with MDS (NCT04419649)

Keros is conducting an open label, two-part, multiple ascending dose Phase 2 clinical trial to evaluate elritercept in patients with very low-, low-, or intermediate-risk MDS who either have or have not previously received treatment with an erythroid stimulating agent.

The primary objective of this trial is to assess the safety and tolerability of elritercept in patients with MDS that are RS positive or non-RS. The primary objective of Part 2 of this trial is confirmation of the safety and tolerability of the RP2D (3.75 mg/kg and 5.0 mg/kg). The secondary objectives of this trial are to evaluate the pharmacokinetics, pharmacodynamics and efficacy of elritercept.

About the Ongoing Phase 2 Clinical Trial of Elritercept in Patients with MF-Associated Cytopenias (RESTORE trial; NCT05037760)

Keros is conducting an open label, two-part, multiple ascending dose Phase 2 clinical trial to evaluate elritercept as a monotherapy and in combination with ruxolitinib in patients with MF-associated cytopenias.

The primary objective of this trial is to assess the safety and tolerability of elritercept in patients with MF-associated cytopenias. The primary objective of Part 2 of this trial is confirmation of the safety and tolerability of the RP2D (3.75 mg/kg and 5.0 mg/kg). The secondary objectives of this trial are to evaluate the pharmacokinetics, pharmacodynamics and efficacy of elritercept administered with or without ruxolitinib.

About Elritercept

Keros' lead product candidate, elritercept, is an engineered ligand trap comprised of a modified ligand-binding domain of the TGF-ß receptor known as activin receptor type IIA that is fused to the portion of the human antibody known as the Fc domain. Elritercept is being developed for the treatment of low blood cell counts, or cytopenias, including anemia and thrombocytopenia, in patients with MDS and in patients with MF.

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. We are 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, we have discovered and are developing protein therapeutics that have the potential to provide meaningful and potentially disease-modifying benefit to patients. Keros' lead product candidate, elritercept, is being developed for the treatment of low blood cell counts, or cytopenias, including anemia and thrombocytopenia, in patients with MDS and in patients with MF. Keros' second product candidate, cibotercept (KER-012), is being developed for the treatment of pulmonary arterial hypertension and for the treatment of cardiovascular disorders. Keros' third product candidate, KER-065, is being developed for the treatment of obesity and for the treatment of neuromuscular diseases.

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 "potential," "progress" 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 growth, strategy, progress and the design, objectives, endpoints and timing of its clinical trials for elritercept, including its regulatory plans; the potential of elritercept to treat beyond MF- and MDS-associated cytopenias to have a direct effect on the pathogenesis of MF and MDS, respectively; the potential of KER-050 to ameliorate ineffective hematopoiesis across multiple lineages in patients with MDS and to improve quality of life in patients with lower-risk MDS; and the potential of KER-050 to address anemia due to MF and ruxolitinib-associated anemia. 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, elritercept, cibotercept and KER-065; 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 Quarterly Report on Form 10-Q, filed with the SEC on May 8, 2024, 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.

Investor Contact:
Justin Frantz
jfrantz@kerostx.com
617-221-6042