Travere Therapeutics to Present Abstracts at the Society for the Study of Inborn Errors of Metabolism Annual Symposium 2024

August 22, 2024

Posters to be presented highlight clinical study designs investigating pegtibatinase as the first potential disease-modifying treatment for classical homocystinuria

SAN DIEGO, Aug. 22, 2024 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc., (Nasdaq: TVTX) today announced that the Company will present two posters in classical homocystinuria (HCU) at the Society for the Study of Inborn Errors of Metabolism (SSIEM) annual symposium in Porto, Portugal, September 3-6, 2024.

At SSIEM, the Company will present the trial designs of the pivotal Phase 3 HARMONY Study and ENSEMBLE long-term extension study of pegtibatinase, a novel investigational enzyme replacement therapy for the treatment of classical HCU. Additionally, the Company will share the trial design of cohort 7 in the Phase 1/2 COMPOSE Study of pegtibatinase in pediatric participants with classical HCU.

“We believe that pegtibatinase has the potential to effectively replace the deficient CBS enzyme activity, and that it could ultimately become the first disease-modifying therapy for people living with classical HCU,” said Jula Inrig, M.D., chief medical officer of Travere Therapeutics. “We look forward to sharing the study designs of our innovative clinical programs in this rare metabolic disorder at the SSIEM symposium this year.”

SSIEM Poster Presentations
Rosa Mota Pavilion, Porto, Portugal

Pegtibatinase, an Investigational Enzyme Replacement Therapy for Classical Homocystinuria (HCU): design of the HARMONY and ENSEMBLE Phase 3 studies
Poster: EP-013
Abstract Category: Clinical studies, patient-reported outcome measures
Wednesday, September 4, 2024, 6:15 – 8:15 p.m. GMT+1

COMPOSE Phase 1/2, Dose-Escalation Cohort 7 Design: safety of pegtibatinase in children aged ≥5–<12 years with classical homocystinuria (HCU)
e-Poster: PO-072
Abstract Category: Clinical studies, patient reported outcome measures
Wednesday, September 4, 2024, 6:15 – 8:15 p.m. GMT+1

About Classical Homocystinuria

Classical homocystinuria (HCU) is a rare genetic metabolic disorder caused by a deficiency in the enzyme cystathionine beta synthase (CBS). CBS is a pivotal enzyme that is essential for the management of methionine and cysteine in the body. Classical HCU leads to toxic levels of homocysteine that can result in life-threatening thrombotic events such as stroke, pulmonary embolism and deep vein thrombosis, ophthalmologic and skeletal complications, as well as developmental delay. Current treatment options are limited to protein-restricted diet and use of vitamin B6 and betaine.

About Pegtibatinase

Pegtibatinase is an investigational PEGylated, recombinant enzyme replacement therapy designed to address the underlying cause of classical HCU. In preclinical studies, pegtibatinase has demonstrated an ability to reduce total homocysteine levels and improve clinical parameters. In December 2023 the Company initiated the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical HCU. The HARMONY Study is a global, randomized, multi-center, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate the efficacy and safety of pegtibatinase as a novel treatment to reduce total homocysteine (tHcy) levels. In May 2023 the Company announced that data from four patients treated with the highest dose of pegtibatinase in the Phase 1/2 COMPOSE Study showed a clinically meaningful 67.1% mean relative reduction in total homocysteine from baseline and was generally well-tolerated after 12 weeks of treatment. To date, the pegtibatinase program has been granted Breakthrough Therapy designation, Rare Pediatric Disease and Fast Track designations by the FDA, as well as Orphan Drug designation in the U.S. and Europe.

About Travere Therapeutics

At Travere Therapeutics, we are in rare for life. We are a biopharmaceutical company that comes together every day to help patients, families and caregivers of all backgrounds as they navigate life with a rare disease. On this path, we know the need for treatment options is urgent – that is why our global team works with the rare disease community to identify, develop and deliver life-changing therapies. In pursuit of this mission, we continuously seek to understand the diverse perspectives of rare patients and to courageously forge new paths to make a difference in their lives and provide hope – today and tomorrow. For more information, visit travere.com

Forward Looking Statements

This press release contains “forward-looking statements” as that term is defined in the Private Securities Litigation Reform Act of 1995. Without limiting the foregoing, these statements are often identified by the words “on-track,” “positioned,” “look forward to,” “will,” “would,” “may,” “might,” “believes,” “anticipates,” “plans,” “expects,” “intends,” “potential,” or similar expressions. In addition, expressions of strategies, intentions or plans are also forward-looking statements. Such forward-looking statements include, but are not limited to, references to: the potential for pegtibatinase to effectively replace the deficient CBS enzyme activity, the potential for pegtibatinase to ultimately become the first disease-modifying therapy for people living with

classical HCU, and statements relating to clinical studies, including but not limited to trial design, anticipated results and timing related thereto. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks related to the timing and outcome of the studies described herein and uncertainties associated with the regulatory review and approval process, as well as risks and uncertainties associated with enrollment of clinical trials for rare diseases, and risks that ongoing or planned clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. The Company also faces risks related to its business and finances in general, the success of its commercial products and risks and uncertainties associated with its preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with the ongoing commercial launch of FILSPARI, market acceptance of its commercial products including efficacy, safety, price, reimbursement, and benefit over competing therapies, as well as risks associated with the successful development and execution of commercial strategies for such products, including FILSPARI. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that current or anticipated future clinical trials will not proceed as planned. Specifically, the Company faces risks related to the timing and potential outcome of the studies described herein, and the timing and potential outcome of the FDA's review of the Company's sNDA submission for full approval of FILSPARI in IgAN. There is no guarantee that regulators will grant full approval of sparsentan for IgAN or FSGS. The Company also faces the risk that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates, including as a result of macroeconomic conditions; risks relating to the Company's dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and exclusivity periods and intellectual property rights of third parties; risks associated with regulatory interactions; and risks and uncertainties relating to competitive products, including current and potential future generic competition with certain of the Company's products, and technological changes that may limit demand for the Company's products. The Company also faces additional risks associated with global and macroeconomic conditions, including health epidemics and pandemics, including risks related to potential disruptions to clinical trials, commercialization activity, supply chain, and manufacturing operations. You are cautioned not to place undue reliance on these forward-looking statements as there are important factors that could cause actual results to differ materially from those in forward-looking statements, many of which are beyond our control. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise. Investors are referred to the full discussion of risks and uncertainties, including under the heading "Risk Factors", as included in the Company's most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

Contact Info

Media:
888-969-7879
mediarelations@travere.com

Investors:
888-969-7879
IR@travere.com

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