Travere Therapeutics to Present Abstracts at the Society for the Study of Inborn Errors of Metabolism Annual Symposium 2023

August 24, 2023

SAN DIEGO, Aug. 24, 2023 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced that the Company will present clinical data from the Phase 1/2 COMPOSE Study of pegtibatinase, a novel investigational enzyme replacement therapy being evaluated for the treatment of classical homocystinuria (HCU), at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium in Jerusalem, Israel, August 29 – September 1, 2023.

The Company and its collaborators will also present analyses on its prospective HCU natural history study, the prevalence of HCU, and the burden of HCU from the patient perspective highlighting the devastating nature of this rare metabolic disease over patients' lifetimes and underscoring the urgent need for new treatments. In addition, the Company will present data on the clinical burden of HCU and the relationship between total homocysteine (tHcy) and clinical outcomes, which has been recognized as one of the highest ranked posters at SSIEM.

Oral Presentation

Pegtibatinase, an Investigational Enzyme Replacement Therapy for the Treatment of Classical Homocystinuria (HCU): Latest Findings from the COMPOSE Phase 1/2 Trial
Parallel Session: Clinical Studies and Outcomes I
August 30, 9:45 – 10:00 a.m. IDT, Oren 2

Poster Presentations
Poster presentations will take place in the Exhibitions Hall (ICC) on August 29, 3:30 – 7:30 p.m. IDT; August 30, 7:30 a.m. – 8:35 p.m. IDT; August 31, 7:45 a.m. – 3:30 p.m. IDT; and September 1, 8:15 a.m. – 12:30 p.m. IDT.

Clinical Characterization of Classical Homocystinuria Due to Cystathionine-beta Synthase Deficiency: Results from the ACAPPELLA Study
Poster B18
Poster Section: Clinical Studies, Patient Reported Outcome Measures

Clinical Burden of Classical Homocystinuria in the United States: A Retrospective Analysis of Optum Market Clarity
Poster B36
Poster Section: Clinical Studies, Patient Reported Outcome Measures
August 30, 7:11 p.m. IDT, Exhibitions Hall (ICC) – Poster Walk & Highest Ranked Posters

Validation of a Patient Identification Algorithm to Estimate the Prevalence of Classical Homocystinuria (HCU) in the United States (US)
Poster B24
Poster Section: Clinical Studies, Patient Reported Outcome Measures

Understanding the Burden of Classical Homocystinuria (HCU) from the Patient's Perspective: A Qualitative Study
Poster B102
Poster Section: Inborn Errors of Metabolism in Adults

RESTORE, a Phase 3 Study to Evaluate the Effects of Chenodeoxycholic Acid in Adults and Pediatric Patients with Cerebrotendinous Xanthomatosis
Poster B13
Poster Section: Clinical Studies, Patient Reported Outcome Measures

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 supplemental 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 homocystinuria HCU. In preclinical studies, pegtibatinase has demonstrated an ability to reduce total homocysteine levels and improve clinical parameters. Pegtibatinase is currently advancing in the ongoing Phase 1/2 COMPOSE Study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. 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 US 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 "may", "might", "believes", "thinks", "anticipates", "plans", "expects", "intends" or similar expressions. In addition, expressions of our strategies, intentions or plans are also forward-looking statements. Such forward-looking statements include, but are not limited to, references to pegtibatinase being designed to address the underlying cause of classical homocystinuria and references to the Phase 1/2 COMPOSE Study. 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 and uncertainties associated with the regulatory review and approval process, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. The Company faces 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 its other products and products in development, 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.

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