Travere Therapeutics to Present Abstracts at the Society for Inherited Metabolic Disorders 43rd Annual Meeting and the Genetic Metabolic Dieticians International Conference 2022

April 6, 2022

SAN DIEGO, April 06, 2022 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced that the Company will present data from the ongoing 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 Inherited Metabolic Disorders (SIMD) 43rd Annual Meeting, and the 2022 Genetic Metabolic Dieticians International (GMDI) Conference. In December 2021, the Company announced positive topline results from the COMPOSE Study. The Company and its collaborators will also present real-world evidence from metabolic centers of excellence on current challenges in the dietary management of classical HCU.

SIMD 43rd Annual Meeting in Orlando, FL – April 10-13, 2022

Pegtibatinase, an Investigational Enzyme Replacement Therapy for the Treatment of Classical Homocystinuria: Initial Results from the Phase 1/2 COMPOSE Study
Poster #: 55
Date & Time: April 11, 8-10 p.m. ET

2022 GMDI Conference in Lake Las Vegas, NV – May 4-7, 2022

Pegtibatinase, an Investigational Enzyme Replacement Therapy for the Treatment of Classical Homocystinuria: Initial Results from the Phase 1/2 COMPOSE Study
Oral Session: Building Evidence for Evidence-Based Practice Clinical Research
Date & Time: May 4, 3:30-5:30 p.m. PT

Dietary goals and current challenges in the management of classical homocystinuria: insights from multinational real-world experience
Poster #: 9
Date & Time: May 5, 6:15-7:00 p.m. PT

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 and heart attacks, 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 the COMPOSE Study

The ongoing Phase 1/2 COMPOSE Study is a randomized, multicenter, placebo controlled, double-blind dose escalation trial evaluating the safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects of pegtibatinase in up to 40 patients with classical homocystinuria. Patients in COMPOSE are randomized 3:1 to receive subcutaneous doses of either pegtibatinase or placebo, and patients are eligible to continue in an open-label extension after the initial blinded twelve-week treatment period. In the highest dose cohort to date evaluating 1.5mg/kg of pegtibatinase twice weekly, treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy) through 12 weeks of treatment, including a 55.1% mean relative reduction in tHcy from baseline as well as maintenance of tHcy below a clinically meaningful threshold of 100 µmol. To date in the study, pegtibatinase has been generally well-tolerated.

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 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 Company's business and finances in general, success of its commercial products as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with market acceptance of its marketed products including efficacy, safety, price, reimbursement and benefit over competing therapies. 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 future clinical trials will not proceed as planned. Specifically, the Company faces the risk that the Phase 3 clinical trial of sparsentan in FSGS will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan as planned; risk that the Phase 3 clinical trial of sparsentan in IgAN will not demonstrate that sparsentan is safe or effective or serve as the basis for accelerated approval of sparsentan as planned; and risk that sparsentan will not be approved for efficacy, safety, regulatory or other reasons, and for each of the programs, 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; risk 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; 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 faces additional risks associated with the potential impacts the COVID-19 pandemic may have on its business, including, but not limited to (i) the Company's ability to continue its ongoing development activities and clinical trials, (ii) the timing of such clinical trials and the release of data from those trials, (iii) the Company's and its suppliers' ability to successfully manufacture its commercial products and product candidates, and (iv) the market for and sales of its commercial products. 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 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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