Travere Therapeutics to Present Abstracts at the Society for Inherited Metabolic Disorders and Genetic Metabolic Dieticians International

April 4, 2024

Posters to be presented highlighting research investigating pegtibatinase as the first potential disease-modifying treatment for classical homocystinuria

SAN DIEGO, April 04, 2024 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc., (Nasdaq: TVTX) today announced that the Company will present eight posters in classical homocystinuria (HCU) at Society for Inherited Metabolic Disorders (SIMD) in Charlotte, North Carolina from April 14-17, 2024, and Genetic Metabolic Dieticians International (GMDI) in Charlotte, North Carolina from April 17-20, 2024.

At SIMD and GMDI, the Company will present the trial design of the pivotal Phase 3 HARMONY Study of pegtibatinase, a novel investigational enzyme replacement therapy for the treatment of classical HCU. Additionally, the Company will share insights on the development of an innovative tool used for dietary management and monitoring in the Phase 3 HARMONY Study and open-label extension ENSEMBLE Study, and the positive results from cohort 6 in the placebo-controlled Phase 1/2 COMPOSE Study of pegtibatinase in classical HCU.

“Classical HCU is an isolating and devastating rare disorder with very limited treatment options, including adherence to highly restrictive diets, leaving patients and their caregivers with immense challenges,” said Jula Inrig, M.D., chief medical officer of Travere Therapeutics. “We look forward to participating at both of these prestigious metabolic medical meetings, and we are excited about our work pioneering the first potential disease-modifying treatment for the thousands of people affected by classical HCU.”

SIMD Poster Presentations

Latest Results From the COMPOSE Phase 1/2 Trial For the Treatment of Classical Homocystinuria (HCU) Using Pegtibatinase, a Novel Investigational Enzyme Replacement Therapy
Poster: 139
Abstract Category: Innovative Therapies
Symphony Ballrooms 4-7; April 15, 2024, 8-9 p.m. ET

Pegtibatinase, an Investigational Enzyme Replacement Therapy, For The Treatment of Classical Homocystinuria (HCU): Design of the HARMONY Phase 3 Study
Poster: 147
Abstract Category: Innovative Therapies
Symphony Ballrooms 4-7; April 15, 2024, 8-9 p.m. ET

Economic Burden of Classical Homocystinuria in the United States
Poster: 48
Abstract Category: Clinical Care/Research
Symphony Ballrooms 4-7; April 15, 2024, 7-8 p.m. ET

Clinical Burden of Classical Homocystinuria in the United States: A Retrospective Claims Analysis
Poster: 64
Abstract Category: Clinical Care/Research
Symphony Ballrooms 4-7; April 15, 2024, 7-8 p.m. ET

Association Between Homocysteine and Clinical Outcomes in Patients with Classical Homocystinuria: A Systematic Literature Review
Poster: 68
Abstract Category: Clinical Care/Research
Symphony Ballrooms 4-7; April 15, 2024, 7-8 p.m. ET

GMDI Poster Presentations

Latest Results from the COMPOSE Phase 1/2 Trial of Pegtibatinase, a Novel Investigational Enzyme Replacement Therapy for Classical Homocystinuria (HCU)
Poster: 10
April 18, 2024, 4:30-5:15 p.m. ET

Novel Tool for Dietary Management and Monitoring in Clinical Trials of Pegtibatinase, an Investigational Enzyme Replacement Therapy for Classical Homocystinuria
Poster: 16
April 18, 2024, 4:30-5:15 p.m. ET

Novel Dietary Management Strategies for Classical Homocystinuria (HCU) in HARMONY/ENSEMBLE Phase 3 Studies of Pegtibatinase, an

Investigational Enzyme Replacement Therapy
Poster: 33
April 18, 2024, 5:15-6 p.m. ET

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 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. In May 2023, the Company announced that data from four patients treated with the highest dose of pegtibatinase 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 “anticipate,” “believe,” “expect,” “intend,” “may,” “might,” “objective,” “plan,” “will” 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, statements regarding pegtibatinase as the first potential disease-modifying treatment for classical homocystinuria, references to the HARMONY study and the other studies described herein, including statements regarding expectations related thereto, and statements regarding the development of an innovative tool used for dietary management and monitoring. 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 HARMONY study and the other studies described herein; risks and uncertainties associated with the regulatory review and approval process, risks 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 faces risks related to its business and finances in general; risks related to the commercial launch of a new product; risks associated with market acceptance of FILSPARI and other current and future products, including efficacy, safety, price, reimbursement and benefit over competing therapies; and the 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 FDA’s potential acceptance for filing and review of the sNDA submission for full approval of FILSPARI in IgAN, and the timing and potential outcome of the European Commission’s decision regarding conditional marketing authorization of sparsentan for IgAN. There is no guarantee that the FDA will accept the sNDA submission for filing, that the European Commission will grant conditional marketing authorization of sparsentan for IgAN, or 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.

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