Travere Therapeutics to Present Abstracts at the Society for Inherited Metabolic Disorders 44th Annual Meeting and the American College of Medical Genetics and Genomics Annual Clinical Genetics Meeting 2023

March 14, 2023

SAN DIEGO, March 14, 2023 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced that the Company will present a quantitative systems pharmacology (QSP) model for predicting the effects of the investigational enzyme replacement therapy pegtibatinase, in classical homocystinuria (HCU), as well as real-world evidence on the prevalence and potential underdiagnosis and/or underreporting of HCU in the United States, at the 2023 Society for Inherited Metabolic Disorders (SIMD) 44th Annual Meeting in Salt Lake City, UT, March 18-21, 2023. The Company and its collaborators will also provide presentations from the Company's ongoing longitudinal natural history study of people living with HCU, and HCU incidence estimates based on gnomAD database evaluation at the American College of Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting in Salt Lake City, UT, March 14-18, 2023.

ACMG Annual Meeting, Salt Palace Convention Center, Salt Lake City, UT – March 14-18, 2023

Insights from the First Genetic Evaluation of a Longitudinal Natural History Study in Classical Homocystinuria (HCU)
Platform presentation: #O06
Date & Time: March 16, 11 a.m.-12:30 p.m. MT, Room 250

Population-Based Incidence Estimates of Classical Homocystinuria Using the Genome Aggregation Database (gnomAD)
Poster #: P561
Date & Time: March 16, 10:30 a.m.-12 p.m. MT, Exhibit Hall BCD

SIMD Annual Meeting, Hyatt Regency, Salt Lake City, UT – March 18-21, 2023

Development of A Patient Identification Algorithm to Estimate Prevalence of Homocystinuria (HCU) in the United States (US)
Poster #: 54
Attended poster session: March 19, 7-8 p.m. MT, Exhibit Hall

A Quantitative Systems Pharmacology (QSP) Model for Classical Homocystinuria Predicting Efficacy of Treatment
Poster #: 79
Attended poster session: March 19, 8-9 p.m. MT, Exhibit Hall

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 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. Pegtibatinase has been granted Breakthrough Therapy, 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 “on-track”, “positioned”, “look forward to”, "may", "might", "believes", "anticipates", "plans", "expects", "intends," “potential” 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 regulatory review and approval process, as well as risks and uncertainties associated with the Company's business and finances in general, success of its commercial products and risks and uncertainties associated with the Company's

preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with 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. 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 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 or abandoned for safety, regulatory, program assessment or other reasons. There is also no guarantee that the results from the ongoing clinical study of pegtibatinase will be positive, or that the Company will be able to align with regulators on the design of, or ultimately proceed with, a pivotal program for pegtibatinase for HCU. 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; 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 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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