Travere Therapeutics Presents Abstracts at the 15th International Congress of Inborn Errors of Metabolism

2025-09-03

Presentation details long-term safety and efficacy data on pegtibatinase as a potential treatment for classical HCU

SAN DIEGO--(BUSINESS WIRE)-- Travere Therapeutics, Inc., (Nasdaq: TVTX) today announced that the Company shared two oral presentations in classical homocystinuria (HCU) at the International Congress of Inborn Errors of Metabolism, taking place September 2-6 in Kyoto, Japan.

New long-term data from Cohort 6 of the Phase 1/2 COMPOSE open-label extension (OLE) Study demonstrated that at the target dose of 2.5 mg/kg twice weekly, participants treated with pegtibatinase in the OLE maintained significant reductions in disease-related metabolite levels, including a 53.5% relative reduction in total homocysteine and a 67.1% relative reduction in methionine over 50 weeks of treatment. Importantly, within the observation period, homocysteine remained significantly below the clinical guidelines of 100 µM. These reductions are clinically meaningful, as the accumulation of these metabolites is associated with toxicity and disease progression. Pegtibatinase was generally well-tolerated, and no new safety signals were observed.

“We are pleased to see sustained and clinically meaningful reductions in toxic homocysteine and methionine levels over nearly a year of treatment with pegtibatinase, particularly within the context of an open-label study,” said Jula Inrig, M.D., chief medical officer of Travere Therapeutics. “People living with classical HCU continue to face complications from this rare disease with limited therapeutic options, and we look forward to furthering our development of pegtibatinase as the potential first disease-modifying therapy to address these unmet needs.”

In the double-blind treatment period of COMPOSE, participants in Cohort 6 received placebo (n=1) or the target dose of 2.5 mg/kg twice weekly (n=4) over a 12-week period. In the OLE portion of the trial, the patient on placebo was titrated up to the target dose and one patient discontinued after entering the OLE.

The Company remains on track to restart enrollment in the Phase 3 HARMONY Study evaluating pegtibatinase for the treatment of classical HCU in 2026.

International Congress of Inborn Errors of Metabolism Presentations

Long-Term Safety and Efficacy of Pegtibatinase for Treatment of Classical Homocystinuria (HCU): Data from the Phase 1/2 COMPOSE Open-Label Extension Study

Presentation #: OP12-5
Date: September 3, 2025
Location: Room 3 (Kyoto International Conference Center)
Time: 16:00-17:30 JST

An Open-Label, Prospective, Interventional Study to Determine the Optimal Treatment of Classical Homocystinuria (HCU) in Infants Identified through Newborn Screening in Qatar

Presentation #: OP14-5
Date: September 3, 2025
Location: Room 3 (Kyoto International Conference Center)
Time: 16:00-17:30 JST

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 pre-clinical 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: statements relating to the clinical studies and data described herein; statements regarding the potential for pegtibatinase to be the first disease-modifying therapy for classical HCU; and statements and expectations regarding the Company’s ability to restart enrollment in the Phase 3 HARMONY Study, and the expected timing thereof. 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 related to the Company’s sNDA for FILSPARI in FSGS, including the timing and outcome thereof. There is no guarantee that the FDA will grant approval of FILSPARI for FSGS on the anticipated timeline, or at all. The Company also faces risks and uncertainties related to its business and finances in general, the success of its commercial products, risks and uncertainties associated with its preclinical and clinical stage pipeline, risks and uncertainties associated with the regulatory review and approval process, 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. Specifically, the Company faces risks associated with the ongoing commercial launch of FILSPARI in IgAN, the timing and potential outcome of its and its partners’ clinical studies, market acceptance of its commercial products including efficacy, safety, price, reimbursement, and benefit over competing therapies, risks related to the challenges of manufacturing scale-up, risks associated with the successful development and execution of commercial strategies for such products, including FILSPARI, and risks and uncertainties related to the new administration, including but not limited to risks and uncertainties related to tariffs and the funding, staffing and prioritization of resources at government agencies including the FDA. 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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