Travere Therapeutics Announces Voluntary Pause of Enrollment in the Phase 3 HARMONY Study of Pegtibatinase Related to Commercial Manufacturing Scale-Up

September 26, 2024

Company to host conference call today at 4:30 p.m. ET

SAN DIEGO, Sept. 26, 2024 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc., (Nasdaq: TVTX) today announced a voluntary pause of enrollment in the Phase 3 HARMONY Study evaluating pegtibatinase for the treatment of classical homocystinuria (HCU). The voluntary enrollment pause enables the Company to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. Patients currently enrolled in pegtibatinase studies continue to receive study medication from small scale batches which are unaffected by the scale-up process. Currently enrolled patients will be able to continue on study medication as scheduled for the duration of the trials they are participating in.

The voluntary enrollment pause was enacted following the Company's determination that the desired drug substance profile was not achieved in the recent scale-up process. The Company is in the process of notifying all study investigators of the decision to pause enrollment of new patients into the study until additional material is available.

"We believe the potential for pegtibatinase to become the first disease-modifying therapy for classical HCU remains unchanged. Our internal team and external manufacturing partners have extensive biologics expertise, and we are confident that we will be able to make the necessary manufacturing process improvements to continue with this pivotal program. We remain committed to supporting the patients currently enrolled in our clinical trials while working with our manufacturing partners to optimize our commercial-scale manufacturing process," said Bill Rote, Ph.D., senior vice president of research and development at Travere Therapeutics.

The Company expects to further evaluate the necessary commercial process improvements to enable the continuation of the Phase 3 program and anticipates the earliest date to restart enrollment in the Phase 3 HARMONY Study will be in 2026. Previously planned investments related to clinical enrollment in HARMONY and large-scale production are expected to be delayed beyond 2025. Together with the expected decline in costs associated with the development of sparsentan as the Phase 3 programs advance towards completion, the Company now estimates that research and development expenses may be reduced by more than $30 million in 2025 compared to 2024. The Company continues to anticipate that its cash, cash equivalents and marketable securities of $325.4 million as of June 30, 2024 can support its operations into 2028.

Conference call information

Travere Therapeutics will host a conference call and webcast today, Thursday, September 26, 2024 at 4:30 p.m. ET to discuss the voluntary pause in enrollment. To participate in the conference call, dial +1 (323) 994-2093 (U.S.) or +1 (888) 394-8218 (International), confirmation code 5880214. The webcast can be accessed on the Investor page of Travere's website at ir.travere.com/events-presentations. Following the live webcast, an archived version of the call will be available for 30 days on the Company's website.

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 preclinical 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: the potential for pegtibatinase to become the first disease-modifying therapy for classical HCU; statements and expectations regarding future process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study, and related timing expectations; statements regarding ongoing clinical trials, including statements regarding medication for patients currently enrolled in such trials; and statements and expectations related to future research and development expenses and the expected timing thereof, including with respect to investments related to clinical enrollment in HARMONY and large scale production, and the expected decline in costs associated with the development of sparsentan as the Phase 3 programs advance towards completion; and expectations regarding cash runway. 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 manufacturing processes and improvements, and risks related to the regulatory review and approval process, as well as risks and uncertainties associated with the Company's business and finances in general, the success of its commercial products and risks and uncertainties associated with its preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with the challenges of manufacturing scale-up, the ongoing commercial launch of FILSPARI, 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. The Company also faces the risk that its cash runway might not last as long as currently anticipated and 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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