Travere Therapeutics Provides Regulatory Update on Sparsentan Development Program for Focal Segmental Glomerulosclerosis

May 25, 2021

Conference call and webcast to be held at 4:30p.m. ET

SAN DIEGO, May 25, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today provided a regulatory update for its sparsentan program in focal segmental glomerulosclerosis (FSGS). Following achievement of the interim proteinuria endpoint in the ongoing Phase 3 DUPLEX Study of sparsentan in FSGS, the Company conducted pre-New Drug Application (NDA) interactions with the U.S. Food and Drug Administration in pursuit of an accelerated approval submission in the U.S., and Marketing Authorization Application (MAA) pre-submission interactions with the European Medicines Agency (EMA) for conditional marketing authorization and accelerated assessment consideration in Europe.

In recently received final pre-NDA meeting minutes, the FDA acknowledged the high unmet need for approved therapies for the treatment of FSGS but indicated the available data from the interim assessment of the DUPLEX Study would not be adequate to support an accelerated approval at this time. Based upon this feedback, the Company no longer expects to submit for accelerated approval for FSGS in the U.S. during the second half of 2021. The FDA has indicated that it may be possible to submit an application for accelerated approval after additional data accrue in the study. Subject to further discussion with the FDA, the Company believes that it may be possible to provide sufficient additional estimated glomerular filtration (eGFR) data from the DUPLEX Study in the first half of 2022. The FDA has encouraged the Company to request a follow-up meeting to further explore this option in greater detail, and a Type A meeting is expected to occur in the third quarter of 2021.

“We remain very confident in the profile of sparsentan and believe it supports the ability to ultimately become a new treatment standard for FSGS, but we are disappointed that we will not be able to submit for accelerated approval this year and deliver it to patients on our original targeted timeline,” said Eric Dube, Ph.D., chief executive officer of Travere Therapeutics. “Based upon our ongoing dialogue with FDA, we believe the eGFR data need to further mature to support our New Drug Application for accelerated approval in this indication. We remain optimistic that we still have a potential opportunity to pursue a path to an accelerated approval submission in the U.S. next year. We look forward to continuing our collaborative discussions with the FDA and providing a further update in the third quarter of this year.”

The Company also conducted initial MAA pre-submission interactions with the EMA. In that meeting, the Company received administrative support for proceeding as planned with a conditional marketing authorization submission in the second half of 2021. The agency also provided direction on administrative and regulatory topics, as well as guidance for upcoming discussions with assigned rapporteurs and co-rapporteurs. As a result of these interactions, the Company will continue its preparations for an MAA submission of sparsentan in FSGS and seek confirmation of its plans with the assigned rapporteurs and co-rapporteurs in an upcoming meeting.

Conference Call Information

Travere Therapeutics will host a conference call and webcast today, May 25, 2021 at 4:30 p.m. ET to discuss the regulatory update. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 6134997 shortly before 4:30 p.m. ET. The webcast can be accessed at travere.com, in the Events and Presentations section of the Investor Relations page and will be archived for at least 30 days. A replay of the call will be available from 7:30 p.m. ET, May 25, 2021 to 7:30 p.m. ET, June 1, 2021. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 6134997.

About the DUPLEX Study

In February 2021, the Company announced that the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim FPRE endpoint with statistical significance. FPRE is a clinically meaningful endpoint defined as urine protein-to-creatinine ratio (UP/C) ≤1.5 g/g and a >40 percent reduction in UP/C from baseline. After 36 weeks of treatment, 42.0 percent of patients receiving sparsentan achieved FPRE, compared to 26.0 percent of irbesartan-treated patients (p=0.0094). Preliminary results from the interim analysis suggest that at the time of the interim assessment, sparsentan had been generally well-tolerated and shown a comparable safety profile to irbesartan. The DUPLEX Study is fully enrolled with 371 participants and is scheduled to continue as planned on a blinded basis to assess the confirmatory estimated glomerular filtration rate (eGFR) endpoint after 108 weeks of treatment. Topline results from the confirmatory endpoint are expected in the first half of 2023.

About Focal Segmental Glomerulosclerosis

FSGS is a rare proteinuric kidney disorder that is estimated to affect up to 40,000 patients in the U.S. with similar prevalence in Europe. The disorder is defined by progressive scarring of the kidney and often leads to end-stage kidney disease (ESKD). FSGS is characterized by proteinuria, where protein leaks into the urine due to a breakdown of the normal filtration mechanism in the kidney. Once in the urine, protein is considered to be toxic to other parts of the kidney, especially the tubules, and is believed to contribute to further disease progression. Other common symptoms include swelling in parts of the body, known as edema, as well as low blood albumin levels, abnormal lipid profiles and hypertension.

Reduction in proteinuria appears to be beneficial in the treatment of FSGS and may be associated with a decreased risk of progression to ESKD. Achieving FPRE appears to be associated with long-term preservation of renal function in patients with FSGS. FSGS is currently managed with angiotensin receptor blockers, angiotensin converting enzyme inhibitors, steroids or calcineurin inhibitors.

About Sparsentan

Sparsentan is a novel investigational product candidate, that functions as a high affinity dual-acting antagonist of both the endothelin type A and angiotensin II type 1 receptors, in a single molecule. Pre-clinical data have shown that blockade of both pathways in forms of rare chronic kidney

disease, reduces proteinuria, protects podocytes and prevents glomerulosclerosis and mesangial cell proliferation. Sparsentan has been granted Orphan Drug Designation for the treatment of FSGS and IgAN in the U.S. and Europe.

Sparsentan is currently being evaluated in the pivotal Phase 3 DUPLEX Study for the treatment of FSGS and the pivotal Phase 3 PROTECT Study for the treatment of IgAN. If approved for both indications, sparsentan could potentially be the first medicine approved for both FSGS and IgAN.

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 include, but are not limited to, references to the Company's current expectations around the timelines for anticipated future meetings with regulators and for providing a further regulatory update; the possibility, following further engagement with the FDA, of being able to submit an application for accelerated approval of sparsentan for FSGS after additional data accrue in the DUPLEX study; the Company's beliefs regarding maturation of the eGFR data; the potential to generate sufficient additional eGFR data from the DUPLEX Study in the first half of 2022 and to reach alignment with the FDA that such additional data would enable a filing under the accelerated approval pathway; beliefs regarding the profile of sparsentan and references to the ability of sparsentan to ultimately become a new treatment standard for FSGS. 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, including the Subpart H accelerated approval pathway in the United States and the conditional marketing authorization (CMA) pathway in the Europe Union. Specifically, the Company faces the risk that the Phase 3 DUPLEX Study 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 PROTECT Study 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 Company's 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. There is no guarantee that the Company will be able to reach alignment with the FDA around a future NDA submission for sparsentan for FSGS under the Subpart H approval pathway, that the FDA will grant accelerated approval of sparsentan for FSGS or that sparsentan will be approved at all. 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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