Travere Therapeutics Provides Regulatory Update on its Sparsentan Program for IgA Nephropathy

October 13, 2022

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

SAN DIEGO, Oct. 13, 2022 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced that following late-cycle review interactions with the U.S. Food and Drug Administration (FDA), the Company expects the previously assigned Prescription Drug User Fee Act (PDUFA) target action date of November 17, 2022 for its New Drug Application (NDA) under Subpart H for accelerated approval of sparsentan for the treatment of IgA nephropathy to be extended by three months.

As part of its late-cycle review, the FDA has requested that the Company update its proposed Risk Evaluation Mitigation Strategy (REMS) to include liver monitoring for sparsentan consistent with certain other approved products in the endothelin receptor antagonist class. The Company anticipates submitting an updated REMS plan in the coming days. Based upon feedback from the FDA, the updated submission is likely to be considered a major amendment to the NDA which is expected to result in a three-month extension of the PDUFA target action date to allow sufficient time to review the information. No additional clinical data or studies have been requested as part of the application review process.

“While this request for additional monitoring within the REMS came unexpectedly, the strength of the clinical data supporting the profile of sparsentan and our confidence in the potential for sparsentan to be approved as a new therapy for IgA nephropathy remain unchanged,” said Eric Dube, Ph.D., president and chief executive officer of Travere Therapeutics. “Many people living with IgA nephropathy continue to face a progression of disease with no currently approved non-immunosuppressive treatments available. We will use the additional time to work collaboratively with the FDA as we continue the labeling process, and further prepare for launch with the goal of enabling sparsentan to ultimately become a new treatment standard, if approved.”

Conference Call Information

Travere Therapeutics will host a conference call and webcast today, October 13, 2022 at 4:30 p.m. ET to discuss the regulatory update. To participate in the conference call, dial +1-888-254-3590 (U.S.) or +1-929-477-0402 (International), confirmation code 4001801 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.

About Sparsentan

Sparsentan, a Dual Endothelin Angiotensin Receptor Antagonist (DEARA), is a novel investigational product candidate selectively targeting the endothelin A receptor (ET_AR) and the angiotensin II subtype 1 receptor (AT_1R). Pre-clinical data have shown that blockade of both endothelin type A and angiotensin II type 1 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. In February 2021, the Company announced that the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim FSGS partial remission of proteinuria endpoint (FPRE) 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. In August of 2021, the Company announced that the ongoing PROTECT Study in IgAN met its pre-specified interim primary efficacy endpoint with statistical significance, demonstrating a greater than threefold reduction of proteinuria from baseline after 36 weeks of treatment, compared to the active control irbesartan (p<0.0001). Preliminary results from the interim analysis suggest that at the time of the interim assessment, sparsentan had been generally well tolerated and performed consistent with the observed safety profile to date. In the Phase 2 DUET Study of sparsentan in FSGS, the combined treatment group met its primary efficacy endpoint, demonstrating a greater than two-fold reduction in proteinuria compared to irbesartan, and was generally well tolerated after the eight-week, double-blind treatment period. Irbesartan is part of a class of drugs used to manage FSGS and IgAN in the absence of an approved pharmacologic treatment. An NDA for accelerated approval of sparsentan in IgAN is currently being evaluated by the FDA under Priority Review designation. 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", “likely to be,” "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 expectation that the updated submission will be considered a major amendment to the NDA and that the PDUFA target action date will be extended by three months; the Company's anticipated timing for submitting an updated REMS plan; the likelihood of the FDA's potential approval of sparsentan for IgAN by the updated PDUFA target action date, or at all; the Company's goal of making sparsentan a new treatment standard, if approved; references to the Company's expectations of working with the FDA during the review process; references to the efficacy, safety and tolerability profile of sparsentan based on the preliminary data from the DUPLEX and PROTECT Studies' interim analyses; and the potential for sparsentan to become the first medicine approved for both FSGS and IgAN. 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 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; 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 traditional approval of sparsentan as planned; and the 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 updated PDUFA target date, once assigned by the FDA, will be three months from the original PDUFA target action date or that the review process for the sparsentan IgAN NDA will remain on track for the FDA's updated target action date, once assigned, that the FDA will grant accelerated approval of sparsentan for IgAN within the assigned target action date, or at all, or that the DUPLEX Study will support an application for traditional review or that sparsentan will be approved for FSGS. 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; 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 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 under the "Risk Factors" heading of the Company's quarterly report on Form 10-Q, as filed with the Securities and Exchange Commission on August 4, 2022, and other filings with the Securities and Exchange Commission.

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Source: Travere Therapeutics, Inc.