Travere Therapeutics Announces Completion of Patient Enrollment in Pivotal Phase 3 DUPLEX Study of Sparsentan in Focal Segmental Glomerulosclerosis

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Topline data from interim 36-week proteinuria endpoint on track for first quarter of 2021

SAN DIEGO, Nov. 30, 2020 (GLOBE NEWSWIRE) -- Travere Therapeutics (NASDAQ: TVTX) today announced completion of patient enrollment in the Phase 3 DUPLEX Study. The pivotal DUPLEX Study is evaluating the safety and efficacy of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS), a rare kidney condition that often leads to end-stage kidney disease (ESKD). Topline efficacy data from the interim 36-week proteinuria endpoint analysis are expected in the first quarter of 2021.

“Many people living with FSGS are unable to delay progression to end-stage kidney disease and ultimately face transplant or dialysis,” said Noah Rosenberg, M.D., chief medical officer of Travere Therapeutics. “New treatment options are desperately needed. By completing enrollment in the DUPLEX Study, we have achieved the next milestone on our path to potentially delivering sparsentan as the first therapy indicated for the treatment of FSGS. We are maintaining focus on high quality trial conduct over the course of the study and we look forward to reporting interim topline results in the first quarter of next year.”

The DUPLEX Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial of approximately 300 patients with FSGS. Patients are randomized to receive either sparsentan or irbesartan, the active control. The DUPLEX Study protocol provides for an unblinded analysis of at least 190 patients to be performed after 36 weeks of treatment to evaluate the interim efficacy endpoint – the proportion of patients achieving a FSGS partial remission of proteinuria endpoint (FPRE), which is defined as urine protein-to-creatinine ratio (Up/C) ≤1.5 g/g and a >40 percent reduction in Up/C from baseline, at Week 36. Successful achievement of the interim 36-week proteinuria endpoint is expected to serve as the basis for submission of a New Drug Application (NDA) under the Subpart H accelerated approval pathway in the U.S. and Conditional Marketing Authorization (CMA) consideration in Europe. The confirmatory endpoint of the study is the slope of estimated glomerular filtration rate (eGFR) from baseline to Week 108, in approximately 300 patients.

About Sparsentan

Sparsentan is an investigational product candidate in Phase 3 clinical development that has a dual mechanism of action combining endothelin type A receptor antagonism with angiotensin II receptor blockade. Travere Therapeutics is developing sparsentan for the treatment of FSGS and IgA nephropathy (IgAN), rare kidney disorders that often lead to ESKD. In several forms of chronic kidney disease, such as FSGS and IgAN, endothelin receptor blockade has been shown to have an additive beneficial effect on proteinuria in combination with renin-angiotensin blockade via angiotensin receptor blockers or angiotensin converting enzyme inhibitors. Sparsentan has been granted orphan drug designation for the treatment of FSGS by the FDA and European Commission.

The Phase 2 DUET Study of sparsentan in FSGS met its primary efficacy endpoint for the combined treatment group, demonstrating a greater than two-fold reduction in proteinuria compared to irbesartan, 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. Travere Therapeutics is currently advancing the pivotal Phase 3 DUPLEX Study of sparsentan for the treatment of FSGS and continuing to enroll patients in the pivotal Phase 3 PROTECT Study of sparsentan for the treatment of IgAN (IgANprotect.com). Both studies contain 36-week proteinuria-based interim endpoints, which if successfully achieved, are expected to support submission of an NDA under the Subpart H accelerated approval pathway in the U.S. as well as an application for CMA consideration in Europe. If approved for both indications, sparsentan could potentially be the first medicine approved for 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 timeline for reporting top-line data from the proteinuria endpoint in the DUPLEX study, expectations regarding potential regulatory submissions for sparsentan under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe, the Company’s path to potentially delivering sparsentan as the first therapy indicated for the treatment of FSGS and the potential future regulatory approval of sparsentan for 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 Company’s business and finances in general, success of its commercial products as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with market acceptance of its marketed products including efficacy, safety, price, reimbursement and benefit over competing therapies. 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 future clinical trials will not proceed as planned. Specifically, the Company faces the risk that the Phase 3 clinical trial 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 clinical trial 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 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. Also, there is no guarantee that the positive results from the DUET Study of sparsentan in FSGS will be repeated in the currently ongoing Phase 3 DUPLEX study. 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 potential generic competition with certain of the Company's products, and technological changes that may limit demand for the Company's 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.

Contact:
Chris Cline, CFA
Senior Vice President, Investor Relations & Corporate Communications
888-969-7879
IR@travere.com