Travere Therapeutics Completes Enrollment in Pivotal Phase 3 PROTECT Study of Sparsentan in IgA Nephropathy

June 2, 2021

Topline data from interim 36-week proteinuria endpoint expected in August 2021

SAN DIEGO, June 02, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced completion of patient enrollment in the Phase 3 PROTECT Study. The pivotal PROTECT Study is evaluating the safety and efficacy of sparsentan for the treatment of IgA nephropathy (IgAN), a rare kidney disorder that often progresses to end-stage kidney disease (ESKD). Topline efficacy data from the interim 36-week proteinuria endpoint analysis are expected in August 2021.

“The current treatment goal for people living with IgAN is to preserve kidney function, but with limited treatment options available many patients remain at high risk of progression towards end-stage kidney disease,” said Noah Rosenberg, M.D., chief medical officer of Travere Therapeutics. “Completing enrollment in PROTECT marks a significant milestone on our path to potentially establishing sparsentan, if approved, as a new, non-immunosuppressant based treatment option to slow the progression of IgAN. We are grateful for the continued strong support from patients and investigators in this important trial, and we look forward to the topline results from the 36-week proteinuria analysis in August of this year.”

The PROTECT Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled pivotal Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 380 patients with IgAN. The PROTECT Study protocol provides for an unblinded analysis of at least 280 patients to be performed after 36 weeks of treatment to evaluate the primary efficacy endpoint – the change in proteinuria (urine protein-to-creatinine ratio, or UPCR) at Week 36 from baseline. The interim assessment of the PROTECT Study is designed to support potential submissions under the Subpart H pathway for accelerated approval in the United States, and potential Conditional Marketing Authorization in Europe. Secondary efficacy endpoints include the rate of change in eGFR following the initiation of randomized treatment over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment in approximately 380 patients.

About IgA Nephropathy

IgA nephropathy (IgAN), also called Berger's disease, is a rare kidney disorder characterized by the buildup of immunoglobulin A (IgA), a protein that helps the body fight infections, in the kidneys. The deposits of IgA cause a breakdown of the normal filtering mechanisms in the kidney, leading to blood in the urine (hematuria), and protein in the urine (proteinuria). Other symptoms of IgAN may include kidney pain, swelling (edema) and high blood pressure.

IgAN is the most prevalent primary chronic glomerular disease worldwide and a leading cause of ESKD. IgAN is estimated to affect more than 100,000 people in the U.S. and is one of the leading causes of acute nephritis in Europe and Japan. There are currently no approved treatments indicated for IgAN.

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 IgAN and FSGS in the U.S. and Europe.

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

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 timing for reporting top-line data from the interim proteinuria endpoint in the PROTECT Study; the Company’s path to potentially establishing sparsentan, if approved, as a new, non-immunosuppressant based treatment option to slow the progression of IgAN; the potential for the interim assessment of the PROTECT Study to support submissions under the Subpart H pathway for accelerated approval in the United States and Conditional Marketing Authorization in Europe; 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 regulatory review and approval process, including the Subpart H accelerated approval pathway in the United States and the conditional marketing authorization (CMA) pathway in Europe. 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 interim assessment from the PROTECT Study will support an NDA submission for sparsentan for IgAN under the Subpart H approval pathway, that the FDA will grant accelerated approval of sparsentan for IgAN 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; 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 in the Company's most recent Form 10-Q, Form 10-K and other filings with the Securities and Exchange Commission.

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