Travere Therapeutics Announces European Commission Has Granted Orphan Designation to Sparsentan for the Treatment of IgA Nephropathy

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SAN DIEGO, Feb. 18, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced the European Commission (EC) has granted orphan designation to sparsentan for the treatment of IgA nephropathy (IgAN), a rare kidney disorder and a leading cause of end-stage kidney disease (ESKD). Sparsentan is an investigational product candidate currently being evaluated in a pivotal Phase 3 clinical study for the treatment of IgAN, as well as a pivotal Phase 3 clinical study for the treatment of focal segmental glomerulosclerosis (FSGS). The Company recently reported that the ongoing DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim proteinuria endpoint and that preliminary results from the interim analysis suggest that to date in the study, sparsentan has been generally well-tolerated and has shown a comparable safety profile to irbesartan. Topline data from the interim proteinuria assessment in the ongoing PROTECT Study of sparsentan in IgAN remain on track to be reported in the third quarter of 2021.

“The limited and non-specific therapeutic strategies used for IgAN today are often associated with long-term tolerability challenges, and for many are not enough to slow the progression to ESKD,” said Noah Rosenberg, M.D., chief medical officer of Travere Therapeutics. “We are pleased to receive orphan designation in Europe which further supports our goal of ultimately delivering sparsentan as a potential new treatment standard for IgAN.”

Orphan designation from the EC provides incentives for companies to develop medicines intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating and where no satisfactory treatment is currently authorized. The prevalence of the condition must not exceed more than five in 10,000 people in the European Union (EU). In addition to being eligible for a 10-year period of marketing exclusivity in the EU upon product approval, orphan designation provides fee waivers, protocol assistance, and eligibility for marketing authorization under the centralized procedure granting approval in all EU countries. Travere Therapeutics was previously granted Orphan Drug designation for IgAN in the U.S., and it also holds Orphan Drug designation in the U.S. and orphan designation in Europe for sparsentan for the treatment of FSGS.

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 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 FSGS. In February of 2021, the Company reported that the ongoing DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim proteinuria endpoint and that preliminary results from the interim analysis suggest that to date in the study, sparsentan has been generally well-tolerated and has shown a comparable safety profile to irbesartan. 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. 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 timelines for reporting top-line data from the proteinuria endpoint in the PROTECT study, the goal of ultimately delivering sparsentan as a potential new treatment standard for 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 commercial 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 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 for each of its development programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing clinical

trials may not proceed on expected timelines or may be delayed for safety, regulatory or other reasons and risk that the product candidates will not be approved for efficacy, safety, regulatory or other reasons. 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 potential 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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