Travere Therapeutics Announces Orphan Drug Designation for Sparsentan for the Treatment of IgA Nephropathy

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SAN DIEGO, Jan. 12, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug 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 for the treatment of IgAN, as well as focal segmental glomerulosclerosis (FSGS) in pivotal Phase 3 clinical trials that are expected to report topline data from interim proteinuria assessments in 2021.

“People living with IgAN face a significant unmet need with limited treatment options available,” said Noah Rosenberg, M.D., chief medical officer of Travere Therapeutics. “Obtaining Orphan Drug Designation is another milestone in our development program as we continue to advance towards the goal of delivering sparsentan as a potential new treatment standard for people living with IgAN. We continue to look forward to topline data from the interim proteinuria assessment in the ongoing Phase 3 PROTECT Study in IgAN during the third quarter of this year.”

The Orphan Drug Designation program is intended to encourage the development of therapeutics for diseases that affect fewer than 200,000 individuals in the United States. Prior to FDA approval, Orphan Drug Designation qualifies sponsors for certain incentives, such as tax credits toward the cost of clinical trials and prescription drug user fee waivers. Orphan Drug Designation may also convey seven years of marketing exclusivity for sparsentan if approved by the FDA for the treatment of IgAN. Travere Therapeutics previously received a positive opinion from the European Medicines Agency Committee for Orphan Medicinal Products on the company’s application for Orphan Drug Designation for IgAN in Europe, and it holds Orphan Drug Designation for sparsentan for the treatment of FSGS in the U.S. and Europe.

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 in a single molecule. Travere Therapeutics is developing sparsentan for the treatment of IgAN and FSGS, rare kidney disorders that often lead to ESKD. In several forms of chronic kidney disease, such as IgAN and FSGS, 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.

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, 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. 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 timelines for reporting top-line data from the proteinuria endpoints in the DUPLEX and PROTECT studies, expectations regarding potential regulatory submissions for sparsentan under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe , the potential future regulatory approval of sparsentan for FSGS and IgAN and the goal of delivering sparsentan as a potential new treatment standard for people living with 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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