Travere Therapeutics Announces Successful Outcome from Type A Meeting with U.S. FDA for Sparsentan in Focal Segmental Glomerulosclerosis

September 7, 2021

Company and FDA align on a pathway to proceed with submission for accelerated approval in mid-2022 pending additional eGFR data

SAN DIEGO, Sept. 07, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today announced a successful outcome from the Company's Type A meeting with the U.S. Food and Drug Administration (FDA) in which alignment was reached on the Company's plan to submit additional estimated glomerular filtration rate (eGFR) data from the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) to support an application for accelerated approval in 2022.

"We are very pleased with the outcome of the Type A meeting which confirms our plan to provide FDA with additional eGFR data from the ongoing DUPLEX Study in the first half of 2022 to continue on the accelerated approval pathway for sparsentan in FSGS," said Eric Dube, Ph.D., chief executive officer of Travere Therapeutics. "If the additional data further strengthen the prediction of long-term benefit in the study as we expect, we anticipate submitting a New Drug Application for accelerated approval of sparsentan for FSGS in the middle of next year and furthering our preparations to deliver it as a potential new treatment standard for FSGS, if approved."

Following the outcome of the Type A meeting, the Company intends to continue with its plan to provide the FDA with additional eGFR data from the ongoing DUPLEX Study in the first half of 2022. At the time of the eGFR data cut, all patients remaining in the DUPLEX Study will have completed one year of treatment, and approximately 50 percent of patients will have completed two years of treatment. The Company plans to submit an application for accelerated approval in the U.S. in mid-2022, pending additional supportive eGFR data. The DUPLEX Study is continuing as planned with no changes to the statistical analysis plan, and patients will proceed in a blinded manner to assess the treatment effect on eGFR slope over 108 weeks in the confirmatory endpoint analysis.

About Sparsentan

Sparsentan, a Dual Endothelin Angiotensin Receptor Antagonist (DEARA), is a novel investigational product candidate. 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 focal segmental glomerulosclerosis (FSGS) and the pivotal Phase 3 PROTECT Study for the treatment of IgAN. In February 2021, the Company announced that the ongoing 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 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. 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 expected timing and plan for the data cut for the additional eGFR data from the DUPLEX Study; the Company's expectation that the additional data should further strengthen the prediction of long-term benefit in the DUPLEX study; the expectation of submitting an application for accelerated approval of sparsentan for FSGS in mid-2022, pending the additional eGFR data; the Company's expectations to further its preparations to deliver sparsentan as a potential new treatment standard for FSGS, if approved; expectations regarding the future conduct of the ongoing DUPLEX study and timing for topline results from the confirmatory endpoint analysis; 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, and the risk that the additional eGFR data will not support an accelerated approval submission. 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 planned eGFR data cut will provide sufficient additional support for an accelerated approval submission for sparsentan in FSGS, that the FDA will accept for filing a NDA for sparsentan for FSGS under the Subpart H approval pathway, that the FDA will grant accelerated approval of sparsentan for FSGS 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; 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-K, Form 10-Q and other filings with the Securities and Exchange Commission.

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Source: Travere Therapeutics, Inc.