Travere Therapeutics Provides Corporate Update and 2022 Outlook

January 10, 2022

Company positioned for multiple NDA and MAA submissions for accelerated approval in 2022

SAN DIEGO, Jan. 10, 2022 (GLOBE NEWSWIRE) -- Travere Therapeutics (NASDAQ: TVTX) today announced that, based on preliminary and unaudited financial data, the Company expects net product sales for the fourth quarter of 2021 to be approximately $55 million. For the fiscal year 2021, the Company expects total revenue of $227 million, inclusive of approximately $211 million in net product sales and approximately $16 million in licensing and collaboration revenue. The Company also provided a general update on its development programs, including anticipated milestones for 2022.

“Following three positive readouts from our pipeline and continued strong execution from our commercial business last year, we enter 2022 with great confidence in our ability to deliver new life-changing therapies to people living with rare disease,” said Eric Dube, Ph.D., chief executive officer of Travere Therapeutics. “We are driven by the potential to make sparsentan a new treatment standard for people living with rare kidney diseases IgA nephropathy and FSGS, if approved. We remain on track for our planned NDA and MAA submissions, the first of which could result in an approval of sparsentan for IgA nephropathy in the U.S. as early as the end of this year. Additionally, with recent clinical proof of concept supporting pegtibatinase, we are in position to engage with regulators and establish the next steps for a pivotal program to further advance this therapy as the first potential treatment targeting the underlying deficiency in classical homocystinuria.”

Program Updates and Anticipated Upcoming Milestones

- The Company continues to advance its investigational Dual Endothelin Angiotensin Receptor Antagonist (DEARA) sparsentan for the treatment of IgA nephropathy (IgAN) and focal segmental glomerulosclerosis (FSGS) following previously reported positive topline interim results from the ongoing pivotal Phase 3 PROTECT and DUPLEX studies. The following upcoming milestones are anticipated in 2022:
    - In the first quarter of 2022, the Company expects to submit a New Drug Application (NDA) for accelerated approval of sparsentan for IgAN in the U.S.
    - In the first half of 2022, the Company plans to provide additional estimated glomerular filtration rate (eGFR) data from the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS to the U.S. Food and Drug Administration (FDA) to support a potential NDA submission for accelerated approval. Should additional eGFR data from the study be supportive as expected, the Company plans to submit an NDA for accelerated approval of sparsentan for FSGS in the U.S. in mid-2022.
    - In collaboration with its partner Vifor Pharma, the Company expects to submit a combined IgAN and FSGS Marketing Authorisation Application (MAA) in mid-2022 for conditional marketing authorization of sparsentan in Europe.
- In December 2021, the Company reported positive topline results from the ongoing Phase 1/2 COMPOSE Study of pegtibatinase, a novel investigational enzyme replacement therapy with the potential to become the first disease modifying therapy for people living with classical homocystinuria (HCU). The following upcoming milestones are anticipated in 2022:
    - The Company will engage with regulators to establish next steps for a pivotal development program to ultimately support potential approvals of pegtibatinase for the treatment of HCU.
    - Additional detailed study results from the first five dosing cohorts in the ongoing Phase 1/2 COMPOSE Study are expected to be presented at a medical meeting in the first half of 2022.
    - In 2022, the Company expects to provide an update on the Phase 1/2 COMPOSE Study following completion of a sixth cohort to further evaluate formulation refinement and pegtibatinase dosing.
- In 2022, the Company will continue to build upon its successful commercialization capabilities to support the potential future launch of sparsentan, if approved.

In late February, the Company expects to announce complete full year 2021 financial results and provide a corporate update.

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

About Preliminary Financial Results

The preliminary results set forth above are unaudited, are based on management's initial review of the Company's results for the quarter and year ended December 31, 2021 and are subject to revision based upon the Company's year-end closing procedures and the completion and external audit of the Company's year-end financial statements. Actual results may differ materially from these preliminary unaudited results following the completion of year-end closing procedures, final adjustments or other developments arising between now and the time that the Company's financial results are finalized. In addition, these preliminary unaudited results are not a comprehensive statement of the Company's financial results for the year ended December 31, 2021, should not be viewed as a substitute for full, audited financial statements prepared in accordance with generally accepted accounting principles, and are not necessarily indicative of the Company's results for any future period.

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 expectations regarding net product sales for the fourth quarter of 2021 and total revenues for fiscal year 2021 based on preliminary and unaudited financial data; expected sparsentan regulatory submissions in 2022, including the timing for the planned IgAN accelerated approval submission, the ability to submit for accelerated approval in FSGS, pending additional supportive eGFR data, as well as expectations and the timing for submitting a joint marketing authorization application in Europe for both FSGS and IgAN; expectations that additional eGFR data from the DUPLEX Study will support an accelerated approval submission; references to the Company's pipeline of potential first-in-class therapies and the Company's ability to deliver new life-changing therapies to people living with rare disease; the potential to make sparsentan a new treatment standard for people living with rare kidney diseases IgA nephropathy and FSGS, if approved; the potential future regulatory approval of sparsentan for FSGS and IgAN and references to the potential timing of such approval; the Company's expectations around engagement with regulatory authorities regarding next steps for a future pivotal trial of pegtibatinase in HCU, future plans for the pegtibatinase program and the potential for the program to deliver the first therapy targeting the underlying deficiency in HCU. 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 the Europe Union, as well as risks and uncertainties associated with the Company's business and finances in general, success of its commercial products and 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 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 FDA will grant accelerated approval of sparsentan for IgAN or FSGS or that sparsentan will be approved at all. There is also no guarantee that the results from ongoing or future clinical studies of pegtibatinase will be positive. 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 under the "Risk Factors" heading of the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, as filed with the Securities and Exchange Commission ("SEC") on October 29, 2021, and other filings with the SEC.

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