Travere Therapeutics Completes Sale of Bile Acid Product Portfolio to Mirum Pharmaceuticals

September 5, 2023

• Travere received $210 million at closing and is eligible for up to $235 million in potential sales-based milestone payments
• Mirum has acquired Travere's rights and assets related to Cholbam ® and Chenodal®
• Advances Travere's strategy to deliver new treatment standards from its pipeline of innovative medicines for rare diseases and strengthens financial foundation
• Expands Mirum's leadership in rare liver disease with two additional commercial products and a near-term Phase 3 label expansion opportunity

SAN DIEGO and FOSTER CITY, Calif., Sept. 05, 2023 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) and Mirum Pharmaceuticals, Inc. (NASDAQ: MIRM) today announced the completion of the previously announced sale of Travere's bile acid product portfolio that includes Cholbam® (cholic acid) and Chenodal® (chenodiol), two medications addressing rare diseases in high-need settings (the "Asset Purchase"). In connection with the closing of the Asset Purchase, Travere received an upfront payment of $210 million from Mirum, and remains eligible to receive up to $235 million in potential sales-based milestone payments.

Mirum has acquired Travere's rights to Cholbam ®, indicated for the treatment of bile acid synthesis disorders due to single enzyme deficiencies and adjunctive treatment of peroxisomal disorders in patients who show signs or symptoms of liver disease, and Chenodal®, indicated for the treatment of radiolucent stones in the gallbladder, which is also under Phase 3 clinical evaluation for cerebrotendinous xanthomatosis (CTX). The closing of the Asset Purchase was subject to the satisfaction of customary closing conditions, including the expiration of the waiting period under the Hart-Scott-Rodino (HSR) Antitrust Improvements Act of 1976. In connection with the closing, Travere will provide certain transitional services to Mirum.

About Cholbam® (cholic acid)
The FDA approved Cholbam® (cholic acid) capsules in March 2015, the first FDA-approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisome biogenesis disorder-Zellweger spectrum disorder. The effectiveness of Cholbam® has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. An estimated 200 to 300 patients are current candidates for therapy.

CHOLBAM® (cholic acid) Indication
Cholbam is a bile acid indicated for

- Treatment of bile acid synthesis disorders due to single enzyme defects.
- Adjunctive treatment of peroxisomal disorders, including Zellweger spectrum disorders, in patients who exhibit manifestations of liver disease, steatorrhea, or complications from decreased fat-soluble vitamin absorption.

LIMITATIONS OF USE
The safety and effectiveness of CHOLBAM on extrahepatic manifestations of bile acid synthesis disorders due to single enzyme defects or peroxisomal disorders, including Zellweger spectrum disorders, have not been established.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS – Exacerbation of liver impairment

- Monitor liver function and discontinue CHOLBAM in patients who develop worsening of liver function while on treatment.
- Concurrent elevations of serum gamma glutamyltransferase (GGT) and alanine aminotransferase (ALT) may indicate CHOLBAM overdose.
- Discontinue treatment with CHOLBAM at any time if there are clinical or laboratory indicators of worsening liver function or cholestasis.

ADVERSE REACTIONS

- The most common adverse reactions (≥1%) are diarrhea, reflux esophagitis, malaise, jaundice, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy.

Please see full Prescribing Information for additional Important Safety Information.

About Chenodal® (chenodiol)
Chenodal® is a synthetic oral form of chenodeoxycholic acid (CDCA), a naturally occurring primary bile acid synthesized from cholesterol in the liver. The FDA approved Chenodal for the treatment of people with radiolucent stones in the gallbladder. In 2010, Chenodal was granted orphan drug designation for the treatment of cerebrotendinous xanthomatosis (CTX), a rare autosomal recessive lipid storage disease.

While Chenodal® is not currently approved for CTX, it received a medical necessity determination in the U.S. by the FDA and has been used as the standard of care for more than three decades. Efforts are being made to obtain FDA approval of Chenodal for the treatment of CTX and a Phase 3

clinical trial for this indication was initiated in January 2020. The prevalence of CTX is estimated in the literature to be as high as 1 in 70,000 in the overall population.

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 Mirum Pharmaceuticals, Inc.
Mirum Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to transforming the treatment of rare liver diseases. Mirum's approved medication is LIVMARLI® (maralixibat) oral solution which is approved in the U.S. for the treatment of cholestatic pruritus in patients with Alagille syndrome three months of age and older. LIVMARLI is also the only approved IBAT inhibitor approved by the European Commission for the treatment of cholestatic pruritus in patients with ALGS two months and older, and by Health Canada for the treatment of cholestatic pruritus in ALGS. For more information for U.S. residents, please visit LIVMARLI.com.

Mirum has also submitted LIVMARLI for approval in the U.S. in cholestatic pruritus in PFIC patients three months of age and older and in Europe in PFIC for patients two months of age and older.

Mirum's late-stage pipeline includes two investigational treatments for debilitating liver diseases affecting children and adults. LIVMARLI, an oral ileal bile acid transporter (IBAT) inhibitor, is currently being evaluated in clinical trials for pediatric liver diseases and includes the EMBARK Phase 2b clinical trial for patients with biliary atresia. In addition, Mirum has an expanded access program open across multiple countries for eligible patients with ALGS and PFIC.

Mirum's second investigational treatment, volixibat, an oral IBAT inhibitor, is being evaluated in two potentially registrational studies including the VISTAS Phase 2b clinical trial for adults with primary sclerosing cholangitis and the VANTAGE Phase 2b clinical trial for adults with primary biliary cholangitis.

Travere Therapeutics, Inc. 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 "look forward to", "will," "may", "might", "believes", "anticipates", "plans", "expects", "intends," "potential" or similar expressions. In addition, expressions of strategies, intentions or plans are also forward-looking statements. Such forward-looking statements include, but are not limited to, references to: the potential sales-based milestone payments under the purchase agreement; estimated patient populations; and expectations regarding Travere's products, pipeline, and strategy to deliver new treatment standards. 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. Travere faces the risk that disruptions from the Asset Purchase and the transition of the business to the acquiror will harm Travere's business, including current plans and operations; potential adverse reactions or changes to business relationships resulting from the announcement or completion of the Asset Purchase; and the risk that Travere will not receive some or all of the potential sales-based milestone payments under the purchase agreement. Travere also faces the risk that its cash runway will not extend as far as anticipated and that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates, including as a result of macroeconomic conditions; the risk that the results from the Phase 3 DUPLEX Study of sparsentan in FSGS will not serve as a basis for a regulatory submission for approval of sparsentan for FSGS; the 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 further approval of sparsentan; risks relating to Travere'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 Travere's products, and technological changes that may limit demand for Travere's products. Travere also faces additional risks associated with global and macroeconomic conditions, including health epidemics and pandemics, including risks related to potential disruptions to clinical trials, commercialization activity, supply chain, and manufacturing operations. 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. Travere 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, including under the heading "Risk Factors", as included in Travere's most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

Mirum Pharmaceuticals, Inc. Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Generally, the words "anticipate," "estimate," "expect," "project," "intend," "plan," "contemplate," "predict," "forecast," "likely," "believe," "target," "will," "could," "would," "should," "potential," "may" and similar expressions or their negative, may, but are not necessary to, identify forward-looking statements. Such forward-looking statements, including those regarding the timing, and consummation and anticipated benefits of, the transactions described herein, the potential sales-based milestone payments under the purchase agreement, estimated patient populations, and expectations regarding Mirum's products, pipeline, and strategy to deliver new treatment standards involve risks and uncertainties. Mirum's experience and results may differ materially from the experience and results anticipated in such statements. The accuracy of such statements is subject to a number of risks, uncertainties and assumptions including, but are not limited to, the following factors: litigation relating to the Asset Purchase; risks that the Asset Purchase disrupts the current plans or operations of Mirum; Mirum's ability to retain and hire key personnel; competitive responses to the Asset Purchase; unexpected costs, charges or expenses resulting from the Asset Purchase; potential adverse reactions or changes to relationships with customers, suppliers, distributors and other business partners resulting from the completion of the Asset Purchase; Mirum's ability to achieve the synergies expected from the Asset Purchase, as well as delays, challenges and expenses associated with integrating the commercialization of Chenodal and Cholbam; the impact of overall industry and general economic conditions, including inflation, interest rates and related monetary policy by governments in response to inflation; geopolitical events, and regulatory, economic and other risks associated therewith; and continued uncertainty resulting from broader macroeconomic conditions. Other factors that might cause such a difference include those discussed in Mirum's filings with the SEC, which include its Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K.

All forward-looking statements made herein are based on information currently available to Mirum as of the date of this press release. Mirum undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

Contacts

Travere Therapeutics, Inc.

Media:
Nivi Nehra
Vice President, Corporate Communications
888-969-7879
mediarelations@travere.com

Investors:
Naomi Eichenbaum
Vice President, Investor Relations
888-969-7879
ir@travere.com

Mirum Pharmaceuticals, Inc.

Media:
Erin Murphy
510-508-6521
media@mirumpharma.com

Investors:
Andrew McKibben
ir@mirumpharma.com

Sam Martin
Argot Partners
ir@mirumpharma.com

Source: Travere Therapeutics, Inc.