Travere Therapeutics Enters Agreement to Sell its Bile Acid Product Portfolio for the Treatment of Rare Liver Diseases to Mirum Pharmaceuticals

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• Mirum to acquire all of Travere's rights and assets related to Cholbam ® and Chenodal®

• Travere to receive up to $445 million with $210 million upfront and up to $235 million in potential sales-based milestones

• 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 commercial products and a near-term Phase 3 label expansion opportunity

SAN DIEGO and FOSTER CITY, Calif., July 17, 2023 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) and Mirum Pharmaceuticals, Inc. (NASDAQ: MIRM) today announced that they have entered into a definitive agreement for the 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.

Under the terms of the definitive agreement, Mirum will purchase Travere's bile acid product portfolio for up to $445 million, consisting of $210 million upfront and $235 million in potential sales-based milestone payments. Mirum will acquire 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).

"This agreement is an important step forward in Travere's strategy to deliver our pipeline of innovative medicines to patients living with rare diseases," said Eric Dube Ph.D., president and chief executive officer of Travere Therapeutics. "The sale of the bile acid portfolio will enable us to further focus our efforts on the ongoing, and successful launch of FILSPARI™ for IgA nephropathy, pursuing a potential regulatory path forward for sparsentan in FSGS, and the development of pegtibatinase for the treatment of classical homocystinuria, all of which we believe have the potential to be future treatment standards in their respective indications. This divestment will also strengthen our financial foundation by meaningfully extending our cash runway and allow us to maximize our growth potential. We look forward to working with Mirum to ensure a seamless transition and continuing the commitment to delivering these important medicines to patients in the rare liver disease community."

"The addition of the bile acid replacement therapies from Travere will strengthen our pipeline and offer an opportunity to leverage our unique expertise in the development and commercialization of treatments in rare and underserved liver diseases," said Chris Peetz, president and chief executive officer at Mirum. "This synergistic acquisition of the bile acid portfolio along with the opportunity to sponsor the genetic testing program will help to reinforce our leadership position in pediatric hepatology. We look forward to building on the meaningful work initiated by the talented Travere team and delivering on our commitment to advancing research and bringing treatments to rare liver disease patients in need."

Transaction Details

Per the terms of the agreement, Mirum will acquire Travere's rights to the bile acid product portfolio consisting of Cholbam ® and Chenodal®. Travere will receive an upfront payment of $210 million and be eligible for up to $235 million in sales-based milestone payments based on annual net sales thresholds tiered from $125 to $500 million. Travere has also agreed to provide certain transitional services. The transaction is expected to close in the third quarter of 2023, subject to regulatory clearance and customary closing conditions.

For Travere, Lazard is acting as financial advisor and Cooley is acting as legal advisor. For Mirum, Evercore is advising on the acquisition and Gibson, Dunn & Crutcher is acting as legal advisor.

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.

DRUG INTERACTIONS

- Inhibitors of Bile Acid Transporters: Avoid concomitant use of inhibitors of the bile salt efflux pump (BSEP) such as cyclosporine. Concomitant medications that inhibit canalicular membrane bile acid transporters such as the BSEP may exacerbate accumulation of conjugated bile salts in the liver and result in clinical symptoms. If concomitant use is deemed necessary, monitoring of serum transaminases and bilirubin is recommended.
- Bile Acid Binding Resins: Bile acid binding resins such as cholestyramine, colestipol, or colesevelam adsorb and reduce bile acid absorption and may reduce the efficacy of CHOLBAM. Take CHOLBAM at least 1 hour before or 4 to 6 hours (or at as great an interval as possible) after a bile acid binding resin.
- Aluminum-based Antacids: Aluminum-based antacids have been shown to adsorb bile acids *in vitro* and can reduce the bioavailability of CHOLBAM. Take CHOLBAM at least 1 hour before or 4 to 6 hours (or at as great an interval as possible) after an aluminum-based antacid.

PREGNANCY
No studies in pregnant women or animal reproduction studies have been conducted with CHOLBAM. Women who become pregnant during CHOLBAM treatment are encouraged to call 1-844-202-6262.

LACTATION
Endogenous cholic acid is present in human milk. Clinical lactation studies have not been conducted to assess the presence of CHOLBAM in human milk, the effects of CHOLBAM on the breastfed infant, or the effects of CHOLBAM on milk production. The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for CHOLBAM and any potential adverse effects on the breastfed infant from CHOLBAM or from the underlying maternal condition.

GERIATRIC USE
It is not known if elderly patients respond differently from younger patients.

HEPATIC IMPAIRMENT

- Discontinue treatment with CHOLBAM if liver function does not improve within 3 months of the start of treatment.
- Discontinue treatment with CHOLBAM at any time if there are clinical or laboratory indicators of worsening liver function or cholestasis. Continue to monitor laboratory parameters of liver function and consider restarting at a lower dose when the parameters return to baseline.

OVERDOSAGE
Concurrent elevations of serum GGT and serum ALT may indicate CHOLBAM overdose. In the event of overdose, the patient should be monitored and treated symptomatically. Continue to monitor laboratory parameters of liver function and consider restarting at a lower dose when the parameters return to baseline.

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 labeled for CTX, it received a medical necessity determination in the US by the FDA and has been used as the standard of care for more than three decades. Travere is working to obtain FDA approval of Chenodal for the treatment of CTX and initiated a Phase 3 clinical trial for this indication 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, and in Europe for the same indication in patients two months of age and older.

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.

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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: expectations regarding the ability to obtain regulatory and other approvals and satisfy other closing conditions for the contemplated transaction, successfully close the contemplated transaction and successfully transition the business to the acquiror; the expected timing of the contemplated transaction and related matters; expectations regarding future sales of Chenodal and Cholbam and the outcome of continuing studies, potential future approvals, and sales of such products, and the related impact on the potential sales-based milestone payments under the purchase agreement; expectations regarding Travere's other products and products in development, including continued progress with the FILSPARI launch and a potential regulatory path forward for sparsentan in FSGS; the timing and achievement of additional development and regulatory milestones; the advancement of pipeline products; expectations regarding and results of Travere's ongoing and future trials, studies and analyses related to its various products including but not limited to Chenodal, Cholbam, sparsentan and pegtibatinase; Travere's cash runway; and future growth prospects. 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 the contemplated transaction will not close on the planned timeline or at all due to a delay or failure to obtain regulatory approval or for any other reason; the possible occurrence of any event, change or other circumstance or condition that could give rise to the termination of the purchase agreement for the proposed transaction; the incurrence of significant transaction costs whether or not the proposed transaction is consummated; the potential for litigation relating to the proposed transaction; and the risk that disruptions from the proposed transaction 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 proposed transaction; 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.

Contacts

Travere Therapeutics

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

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