Albireo Initiates Global Phase 3 Clinical Trial of Odevixibat in Alagille Syndrome

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- Study represents Albireo's third global, Phase 3 trial in rare cholestatic liver diseases -
- ASSERT gold standard study design in Alagille syndrome -
- Product submission of once-daily odevixibat for patients with PFIC under review by FDA and EMA -

BOSTON, Dec. 17, 2020 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage rare liver disease company developing novel bile acid modulators, today announced the initiation of its global Phase 3 pivotal trial, ASSERT, Alagille Syndrome looking at Safety and Efficacy in a Randomized controlled Trial, which will evaluate odevixibat in patients with Alagille syndrome. Odevixibat is a potent, once-daily, non-systemic ileal bile acid transport inhibitor (IBATi) being investigated for the treatment of rare pediatric cholestatic liver diseases, including progressive familial intrahepatic cholestasis (PFIC), biliary atresia and Alagille syndrome (ALGS). ASSERT is Albireo’s third global trial in rare cholestatic liver conditions and furthers the Company’s efforts to deliver life-changing therapies to children and young adults living with these diseases.

ALGS is a rare multisystem genetic disorder that can affect the liver, heart and other parts of the body. Approximately 95% of patients with the condition present with chronic cholestasis, usually within the first three months of life, and as many as 88% also present with severe, intractable pruritus. Currently, there is no approved drug therapy for the treatment of ALGS.

ASSERT is a gold standard, prospective intervention trial. The double-blind, randomized, placebo-controlled trial is designed to evaluate the safety and efficacy of 120 µg/kg/day odevixibat for 24 weeks in relieving pruritus in patients with ALGS. Secondary endpoints will measure serum bile acid levels and safety and tolerability. Both the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) have agreed on the study design and have indicated that a single study demonstrating safety and efficacy of odevixibat would be sufficient for regulatory filings. The trial is expected to enroll approximately 45 patients aged 0 to 17 years of age with a genetically confirmed diagnosis of ALGS across 35 sites in North America, Europe, Middle East and Asia Pacific.

“Odevixibat is the first IBATi inhibitor to have demonstrated efficacy and tolerability in a Phase 3 randomized, placebo-controlled trial, and this gives us increased confidence for positive clinical outcomes in Alagille syndrome with ASSERT,” said Ron Cooper, President and Chief Executive Officer of Albireo. “We are pleased to initiate the ASSERT study within guidance and offer hope to children and young adults around the globe with Alagille syndrome who have no approved therapeutic options today.”

Odevixibat is also currently being evaluated in the ongoing PEDFIC 2 Phase 3 open-label trial in patients with PFIC, and the BOLD Phase 3 trial in patients with biliary atresia. The Company provides an Expanded Access Program for eligible patients with PFIC in the U.S., Canada, Australia and Europe.

About Alagille Syndrome
Alagille syndrome (ALGS) is a rare, multisystem genetic disorder that can affect the liver, heart, skeleton, eyes, central nervous system, kidneys and facial features. Liver damage is caused by a paucity of bile ducts preventing bile flow from the liver to the small intestine. Approximately 95% of patients with ALGS present with chronic cholestasis, usually within the first three months of life, and up to 88% also present with severe, intractable pruritus. Currently, there are no approved drug treatments.

About PFIC
Progressive familial intrahepatic cholestasis (PFIC) is a rare disorder that causes progressive, life-threatening liver disease. Patients have impaired bile flow, or cholestasis, caused by genetic mutations. The resulting bile build-up in liver cells causes liver disease and symptoms. The most prominent and problematic ongoing manifestation of the disease is pruritus, or intense itching, which often results in a severely diminished quality of life. Other symptoms include jaundice, poor weight gain and slowed growth. In many cases, PFIC leads to cirrhosis and liver failure within the first 10 years of life, and nearly all people with PFIC require treatment before age 30. There are no drugs currently approved for PFIC, only surgical options that include partial external biliary diversion (PEBD) and liver transplantation. Additional information on PFIC is available at https://www.pficvoices.com.

About Biliary Atresia
Biliary atresia is a rare pediatric liver disease with symptoms typically developing about two to eight weeks after birth. Damaged or absent bile ducts outside the liver result in bile and bile acids being trapped inside the liver, quickly resulting in cirrhosis and even liver failure. Children have clay-colored or no color in their stools and jaundice, among other things, and a few patients are pruritic. Biliary atresia is the most common pediatric cholestatic liver disease and is the leading cause of liver transplants among children as there are no approved drug treatments.

About Odevixibat
Odevixibat is an investigational product candidate being developed to treat rare pediatric cholestatic liver diseases, including progressive familial...
intrahepatic cholestasis (PFIC), biliary atresia and Alagille syndrome. A potent, once-daily, non-systemic ileal bile acid transport inhibitor (IBATi), odevixibat acts locally in the small intestine. Odevixibat does not require refrigeration and can be taken as a capsule for older children, or opened and sprinkled onto food, which are factors of key importance for adherence in a pediatric patient population. Odevixibat is currently being evaluated in the ongoing PEDFIC 2 open-label trial the BOLD Phase 3 trial in patients with biliary atresia, and the global Phase 3 ASSERT trial for ALGS.

About Albireo
Albireo Pharma is a clinical-stage biopharmaceutical company focused on the development of novel bile acid modulators to treat rare pediatric and adult liver diseases, and other liver diseases and disorders. Albireo’s lead product candidate, odevixibat, is being developed to treat rare pediatric cholestatic liver diseases with Phase 3 pivotal trials in PFIC, Alagille syndrome and biliary atresia. The Company completed IND-enabling studies for new preclinical candidate A3907 this year and plans to advance development in adult liver disease. Albireo was spun out from AstraZeneca in 2008 and is headquartered in Boston, Massachusetts, with its key operating subsidiary in Gothenburg, Sweden. The Boston Business Journal named Albireo one of the 2020 Best Places to Work in Massachusetts for the second consecutive year. For more information on Albireo, please visit www.albireopharma.com.

Forward-Looking Statements
This press release includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include statements, other than statements of historical fact, regarding, among other things: the plans for, or progress, scope, cost, initiation, duration, enrollment, results or timing for availability of results of, development of odevixibat or any other Albireo product candidate or program, including regarding expectations regarding the impact of COVID-19 on our business and our ability to adapt our approach as appropriate; the Phase 3 clinical program for odevixibat in patients with PFIC, the pivotal trial for odevixibat in biliary atresia (BOLD), and the pivotal trial for odevixibat in Alagille syndrome (ASSERT); the target indication(s) for development or approval, the size, design, population, location, conduct, cost, objective, enrollment, duration or endpoints of any clinical trial, or the timing for initiation or completion of or availability or reporting of results from any clinical trial, including the long-term open-label extension study for odevixibat in PFIC, the pivotal trial for odevixibat in biliary atresia, the pivotal trial for odevixibat in Alagille syndrome; the potential approval and commercialization of odevixibat; discussions with the FDA or EMA regarding our programs; the potential benefits or competitive position of odevixibat or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential effects of odevixibat of the treatment of PFIC patients and its potential to improve the current standard of care; the potential benefits of an orphan drug designation; the potential issuance of a rare pediatric disease priority review voucher; or Albireo’s plans, expectations or future operations, financial position, revenues, costs or expenses. Albireo often uses words such as “anticipates,” “believes,” “plans,” “expects,” “projects,” “future,” “intends,” “may,” “will,” “should,” “could,” “estimates,” “predicts,” “potential,” “planned,” “continue,” “guidance,” and similar expressions to identify forward-looking statements. Actual results, performance or experience may differ materially from those expressed or implied by any forward-looking statement as a result of various risks, uncertainties and other factors, including, but not limited to: negative impacts of the COVID-19 pandemic, including on manufacturing, supply, conduct or initiation of clinical trials, or other aspects of our business; whether favorable findings from clinical trials of odevixibat to date, including findings in indications other than PFIC, will be predictive of results from other clinical trials of odevixibat; whether either or both of the FDA and EMA will determine that the primary endpoint for their respective evaluations and treatment duration of the double-blind Phase 3 trial in patients with PFIC are sufficient to support approval of odevixibat in the United States or the European Union, to treat PFIC, a symptom of PFIC, a specific PFIC subtype(s) or otherwise; the outcome and interpretation by regulatory authorities of the ongoing third-party study pooling and analyzing of long-term PFIC patient data; the timing for initiation or completion of, or for availability of data from, clinical trials of odevixibat, including the pivotal program in biliary atresia or the pivotal program in Alagille syndrome, and the outcomes of such trials; Albireo’s ability to obtain coverage, pricing or reimbursement for approved products in the United States or European Union; delays or other challenges in the recruitment of patients for, or the conduct of, company’s clinical trials; and Albireo’s critical accounting policies. These and other risks and uncertainties that Albireo faces are described in greater detail under the heading “Risk Factors” in Albireo’s most recent Annual Report on Form 10-K or in subsequent filings that it makes with the Securities and Exchange Commission. As a result of risks and uncertainties that Albireo faces, the results or events indicated by any forward-looking statement may not occur. Albireo cautions you not to place undue reliance on any forward-looking statement. In addition, any forward-looking statement in this press release represents Albireo’s views only as of the date of this press release and should not be relied upon as representing its views as of any subsequent date. Albireo disclaims any obligation to update any forward-looking statement, except as required by applicable law.

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