Albireo Enrolls First Patient in Phase 3 Clinical Trial of Odevixibat in Biliary Atresia

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—BOLD is first-ever pivotal trial for biliary atresia —

—Trial expands odevixibat's development program into second rare cholestatic liver disease —

BOSTON, July 14, 2020 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced the first patient has been enrolled in the BOLD (**B**iliary atresia and the use of **O**devixibat in treating **L**iver **D**isease) global Phase 3 clinical trial of odevixibat, a highly potent and selective inhibitor of the ileal bile acid transporter (IBAT), for the treatment of biliary atresia. There are no approved pharmacological treatments for biliary atresia which is the most common pediatric cholestatic liver disease and the leading cause of pediatric liver transplant across all diseases. BOLD is the largest, prospective intervention trial ever conducted in biliary atresia, expanding the development of odevixibat to a second rare cholestatic liver disease indication. Two clinical sites in the United States are currently active for patient enrollment in the second odevixibat Phase 3 pivotal trial.

Biliary atresia is a rare pediatric liver disease with symptoms typically developing about two to eight weeks after birth and no approved pharmacological therapies. 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. The disease impacts an estimated 15,000-20,000 people in the United States and European Union and is the leading cause of liver transplants among children. Odevixibat has received orphan drug designation for biliary atresia in the United States and European Union.

"The initiation of the BOLD trial is encouraging progress for biliary atresia patients, their families and clinicians, as it is the first-ever pivotal trial of a pharmacological therapy for this devastating rare disease," said Saul Karpen, M.D., Ph.D., Pediatric Hepatologist at Children's Healthcare of Atlanta and lead investigator of the BOLD trial. "There is an incredible unmet need for these patients with biliary atresia, since there are no effective therapies to stem the expected progression of disease. This lack of effective therapeutics leads to the current state of the field where approximately 70 percent of biliary atresia patients will need a liver transplant during childhood. I look forward to seeing how a targeted therapeutic, odevixibat, one that specifically addresses the accumulation of bile acids in the liver, may delay or prevent liver transplant for babies with this serious disease."

BOLD (NCT04336722) is a double-blind, randomized, placebo-controlled trial to evaluate the efficacy and safety of odevixibat in children who have biliary atresia and have undergone a Kasai procedure before age three months. Children in the treatment arm will receive odevixibat and escalate to 120 µg/kg orally once daily for 24 months. The primary efficacy endpoint is improvement in the proportion of patients who are alive and have not undergone a liver transplant after two years of treatment compared to placebo, and secondary outcome measures include time to onset of any sentinel events, total bilirubin levels and serum bile acid levels. The trial will enroll approximately 200 patients at up to 75 sites globally.

"We're pleased to expand the evaluation of odevixibat into a second pivotal trial with the initiation of the precedent-setting BOLD trial for patients with biliary atresia, the most common rare pediatric liver disease," said Ron Cooper, President and Chief Executive Officer of Albireo. "We look forward to continuing our work to realize the potential of odevixibat as a much-needed treatment option for patients across multiple cholestatic liver diseases with BOLD and our PEDFIC 1 Phase 3 trial of odevixibat in progressive familial intrahepatic cholestasis, which we continue to expect topline data from in mid-2020."

In addition to the BOLD and PEDFIC 1 trials, Albireo is finalizing a third pivotal trial of odevixibat in Alagille syndrome and anticipates initiating the trial by the end of 2020.

About Odevixibat
Odevixibat is a product candidate being developed to treat rare pediatric cholestatic liver diseases, including progressive familial intrahepatic cholestasis (PFIC), biliary atresia and Alagille syndrome. A highly potent and selective inhibitor of the ileal bile acid transporter (IBAT), odevixibat has minimal systemic exposure and acts locally in the small intestine. Odevixibat is being evaluated in the PEDFIC 1 Phase 3 clinical trial in patients with PFIC (NCT03566238), as well as the BOLD Phase 3 clinical trial in patients with biliary atresia (NCT04336722).

The odevixibat PFIC program, or elements of it, have received fast track, rare pediatric disease and orphan drug designations in the United States. In addition, the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to odevixibat for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis. The European Medicines Agency (EMA) has granted odevixibat orphan designation, as well as access to the PRIority MEdicines (PRIME) scheme for the treatment of PFIC. Its Pediatric Committee has agreed to Albireo's odevixibat Pediatric Investigation Plan for PFIC. EMA also has granted orphan designation to odevixibat for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis.

About Albireo
Albireo Pharma is a clinical-stage biopharmaceutical company focused on the development of novel bile acid modulators to treat orphan pediatric liver diseases, and other liver and gastrointestinal diseases and disorders. Albireo's lead product candidate, odevixibat, is being developed to treat rare pediatric cholestatic liver diseases and is in Phase 3 development in progressive familial intrahepatic cholestasis (PFIC) and biliary atresia, with a third Phase 3 trial being planned in Alagille syndrome. Albireo's clinical pipeline also includes two Phase 2 product candidates. Elobixibat is in Phase 2 development in NAFLD and NASH. Approved in Japan for the treatment of chronic constipation, elobixibat is the first ileal bile acid transporter (IBAT) inhibitor approved anywhere in the world.

Albireo was spun out from AstraZeneca in 2008. Albireo Pharma is located in Boston, Mass., and its key operating subsidiary is located

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, elobixibat 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), the planned pivotal trial for odevixibat in Alagille syndrome, the Phase 2 clinical trial for elobixibat in NAFLD/NASH, and another Phase 3 trial for elobixibat being conducted by EA Pharma in Japan; 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 Phase 3 PFIC trial for odevixibat, and the long-term open-label extension study, the pivotal trial for odevixibat in biliary atresia, the planned pivotal trial for odevixibat in Alagille syndrome, or the Phase 2 trial for elobixibat in NAFLD/NASH; the potential approval and commercialization of odevixibat; discussions with the FDA or EMA regarding our programs; the potential benefits or competitive position of odevixibat, elobixibat, or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential benefits of an orphan drug designation; the period for which Albireo's cash resources will be sufficient to fund its operating requirements (runway); 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 the trials comprising the Phase 3 PFIC program or any 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, even if the primary endpoint is met with statistical significance, 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 trials comprising the Phase 3 PFIC program, the pivotal program in biliary atresia or the planned 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, the double-blind Phase 3 trial or other pivotal 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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