Albireo Announces First Patients Dosed in Two New Studies

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– Dosed first patients in Phase 1 study with new product candidate A3907 –

– First patient dosed in ASSERT global Phase 3 study of odevixibat for Alagille syndrome –

– EMA & FDA reviewing odevixibat for PFIC, currently no plans for FDA advisory committee meeting –

– Achievement of important clinical milestones tracks to guidance, demonstrates expansion beyond PFIC –

BOSTON, March 25, 2021 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage rare liver disease company developing novel bile acid modulators, today announced progress in two key clinical trials designed to further the Company's efforts to deliver life-changing drugs to children and adults living with rare liver diseases. Furthering the Company's goal of advancing multiple approaches for modulating bile acids, Albireo initiated a Phase 1 study with a new compound, A3907, an oral systemic apical sodium-dependent bile acid transporter (ASBT) inhibitor. A3907 is being developed for adult cholestatic liver diseases such as primary sclerosing cholangitis (PSC) and primary biliary cholangitis (PBC).

Due to high oral bioavailability, A3907 can inhibit ASBT in the intestine and kidney, with the potential to increase elimination of bile acids by both fecal and urinary excretion. By using dual pathway diversion of bile acids, next generation modulators like A3907 seek to increase efficacy without the dose limiting diarrhea seen with bile acid transport inhibitors today.

"Reaching a new milestone with the first patients dosed in our Phase 1 A3907 study represents an important pipeline development for Albireo, reinforcing our scientific leadership in bile acid modulation and ambition to expand into adult liver disease," said Ron Cooper, President and Chief Executive Officer of Albireo. "Simultaneously, we are focused on our ambition of building odevixibat into a globally available billion-dollar product by the end of the decade, and by dosing our first patient in the ASSERT study we've shown great progress in the mission to provide a new drug option to treat rare cholestatic liver diseases."

The Phase 1 study is a first-in-human, double-blind, single and multiple ascending dose study in healthy adult subjects to investigate the safety, tolerability, pharmacokinetics and pharmacodynamics of an A3907 oral formulation. In pre-clinical studies, A3907 showed high systemic exposure and increased level of urinary bile acid secretion in mice, and in a mice model of cholestasis and sclerosing cholangitis, A3907 decreased serum bile acids, and reduced plasma levels of transaminases as well as markers for cell damage and fibrosis. Topline data for the Phase 1 study is anticipated in the second half of 2021, with subsequent initiation of Phase 2 in 2022. Beyond A3907, the Company also recently selected new development candidate A2342, an oral systemic sodium-taurocholate co-transporting peptide (NTCP) inhibitor for viral disease and cholestatic diseases and is moving ahead with IND-enabling studies.

Additionally, the Company enrolled its first patient in the ASSERT Study, a global Phase 3 pivotal trial of odevixibat in patients with Alagille syndrome (ALGS). 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 ALGS. This milestone is in keeping with Albireo's plans to achieve full site activation by mid-year, with topline data expected in 2022.

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.

"I am proud of the work and great progress we are making in our studies of odevixibat in rare cholestatic diseases," said Patrick Horn, Chief Medical Officer of Albireo. "Enrolling our first Alagille patient and continuing enrollment of biliary atresia patients in the BOLD study with 46 sites active, while also progressing A3907 from concept to the clinic, are significant steps in developing our wholly owned pipeline of products to address multiple liver indications."

The U.S. Food and Drug Administration (FDA) granted Priority Review to Albireo's New Drug Application for odevixibat in PFIC with a Prescription Drug User Fee Act (PDUFA) goal date of July 20th of this year, and the Company was informed that there are no plans for an FDA advisory committee meeting. The European Medicines Agency (EMA) has granted odevixibat accelerated assessment and Orphan Designation, as well as access to the PRIority MEdicines (PRIME) scheme for the treatment of PFIC. The EMA's Pediatric Committee has also agreed to Albireo's odevixibat Pediatric Investigation Plans for PFIC and biliary atresia. In addition to PFIC, odevixibat has Orphan Drug Designations for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis. Albireo anticipates potential regulatory approvals, issuance of a rare pediatric disease Priority Review Voucher and launch in the second half of 2021.

About A3907
A3907 is a selective inhibitor of the apical sodium-dependent bile acid transporter (ASBT) with a dual mechanism of action. Due to oral bioavailability, A3907 acts on both renal and ileal transporters to increase elimination of bile acids by both fecal and urinary excretion. This dual inhibition approach may yield increased efficacy without the dose limiting diarrhea seen with bile acid transport inhibitors today.

About Cholestatic Adult Liver Diseases
Adult cholestatic diseases are a diverse group of disorders known for the appearance of jaundice, fatigue, pruritus and/or complications of cirrhosis. The most common adult cholestatic liver diseases are primary biliary cholangitis and primary sclerosing cholangitis. Primary biliary cholangitis is a

chronic disease in which the bile ducts in the liver are slowly destroyed. When the bile ducts are damaged, bile can back up in the liver and sometimes lead to irreversible scarring of liver tissue (cirrhosis). Primary sclerosing cholangitis is a disease of the bile ducts where inflammation causes scars within the bile ducts. These scars make the ducts hard and narrow, gradually causing serious liver damage that leads to liver failure, repeated infections and tumors of the bile duct or liver.

About ASSERT
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 FDA and 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. An additional exploratory cohort of patients ≥18 years of age with genetically confirmed diagnosis will be enrolled, not to exceed 18 patients in total. Primary efficacy endpoint is a change from baseline in scratching to Month 6 (Weeks 21 to 24) as measured by the Albireo ObsRO caregiver instrument. Key secondary efficacy endpoint is a change in serum bile acid levels from baseline to the average of Week 20 and Week 24.

About Odevixibat
Odevixibat is an investigational product candidate being developed to treat rare pediatric cholestatic liver diseases, including PFIC, biliary atresia and ALGS. 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. The FDA has granted Priority Review and set a PDUFA goal date of July 20, 2021. In Europe, the EMA validated MAA. Odevixibat is the only IBATi granted accelerated assessment by the EMA.

The MAA and NDA filings are supported by results from PEDFIC 1 and PEDFIC 2 Phase 3 studies. PEDFIC 1 was the first and largest, global, pivotal Phase 3 study conducted in PFIC, which evaluated the efficacy and tolerability of odevixibat in reducing pruritus and serum bile acids in a randomized, double-blind, placebo-controlled trial. In the PEDFIC 1 study, odevixibat met both primary endpoints and was well tolerated with very low incidence of diarrhea/frequent bowel movements (9.5% of odevixibat treated patients vs. 5.0% of placebo patients). ir.albireopharma.com/news-releases/news-release-details/albireo-phase-3-trial-meets-both-primary-endpoints-odevixibat. PEDFIC 2 is a long-term, open-label Phase 3 extension study. The Company also provides an Expanded Access Program (EAP) for eligible patients with PFIC in the U.S., Europe, Canada and Australia. Odevixibat is also currently being evaluated in 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. Albireo's lead product candidate, odevixibat, is being developed to treat rare pediatric cholestatic liver diseases with Phase 3 trials in PFIC, Alagille syndrome and biliary atresia. The Company has initiated a Phase 1 clinical trial for A3907 to advance development in adult cholestatic liver disease, with IND-enabling studies moving ahead with A2342 for viral and cholestatic 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 expectations regarding the impact of the COVID-19 pandemic on our business and our ability to adapt our plans and activities as appropriate; the pivotal trial for odevixibat in biliary atresia (BOLD), and the pivotal trial for odevixibat in Alagille syndrome (ASSERT); the Phase 1 trial for A3907, 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 Phase 1 trial for A3907, the potential approval and commercialization of odevixibat; the potential for odevixibat to become the first approved drug for PFIC patients; discussions with the FDA or EMA regarding our programs; the potential benefits or competitive position of odevixibat, A3907, A2342 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," or the negative of these terms or other 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: whether the NDA for odevixibat for the treatment of pruritus in patients with PFIC will be approved by the FDA and whether the MAA for odevixibat in PFIC will be approved by the EMA; whether the FDA or EMA will complete their respective reviews within the target timelines, including the FDA's PDUFA goal date, as a potential result of the impact of the COVID-19 pandemic or otherwise; the risk that the NDA will not be approved despite the FDA's acceptance of the NDA for review; whether the FDA will require additional information, whether we will be able to provide in a timely manner any additional information that the FDA requests, and whether such additional information will be satisfactory to the FDA; other potential 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 A3907 or 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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