# Data on Albireo's Odevixibat Presented at 2019 European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN) Annual Meeting

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**Data support further study of odevixibat across a range of rare cholestatic liver diseases**

BOSTON, June 07, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, announced that clinical data from a Phase 2 study of lead product candidate odevixibat (A4250), a highly potent and selective inhibitor of the ileal bile acid transporter (IBAT), in biliary atresia, Alagille syndrome and progressive familial intrahepatic cholestasis (PFIC) were presented today at the 2019 European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN) Annual Meeting in Glasgow, Scotland.

"The encouraging data presented at ESPGHAN support our further study of odevixibat as a potential treatment across a range of rare cholestatic liver diseases," said Ron Cooper, President and Chief Executive Officer of Albireo. "We look forward to advancing our IBAT inhibitors in new indications this year, and sharing topline Phase 3 data on odevixibat in PFIC by the end of 2019 or early 2020."

In the Phase 2 clinical trial in pediatric cholestasis, odevixibat demonstrated marked reductions in serum bile acids (sBA) in the majority of Alagille patients as high as 92 percent (presentation number H-O-006). The majority of Alagille patients also showed improvement in pruritus, as measured by three different scales. One patient out of the six had an elevation in bile acids versus baseline.

In the same trial, odevixibat demonstrated significant sBA reductions of 57.6 percent and 50.8 percent in two biliary atresia patients with high baseline bile acids (>130 µmol/L), and showed improvement in pruritus across two different pruritus scales (poster number H-P-029). No effect was observed in a third patient with low baseline sBA.

In both the Alagille and biliary atresia patients, odevixibat was generally well-tolerated. Adverse events, including some increased transaminases, were mild and transient. Two Alagille patients with high baseline transaminase levels experienced further increases, which informed the decision not to dose escalate above 200 ug.

"Alagille syndrome and biliary atresia are rare and life-threatening liver diseases with significant unmet medical need," said Ulrich Baumann, M.D., lead investigator and Professor of Pediatric Gastroenterology and Hepatology, Hannover Medical School in Hannover, Germany. "The Alagille and biliary atresia patient data from this Phase 2 trial, while limited, are promising and suggest that further studies are warranted."

To date, Albireo is the only company to generate positive data suggesting an effect on bile acids and pruritus in biliary atresia patients using a pharmacologic approach. Albireo plans to initiate a pivotal trial in biliary atresia in the second half of 2019.

In addition to the Phase 2 data, a case study evaluated a patient with PFIC type 2 who was treated with odevixibat in the Phase 2 trial in pediatric cholestasis (poster number H-P-130). After the study period ended, the patient's pruritus returned, and partial external biliary diversion (PEBD) was performed. The study found that, with both odevixibat and PEBD surgery, serum bile acids were reduced by ≥95 percent and returned to normal levels, and the patient showed improvement in pruritus and sleep.

PFIC is a serious disease characterized by an accumulation of bile acids that can cause debilitating pruritus. It often results in liver disease and failure before adulthood. PEBD is an invasive surgical approach commonly used to reduce serum bile acids and pruritus. This is the first case study comparing the effectiveness of a pharmacologic IBAT inhibitor to the current gold standard surgical intervention treatment.

Odevixibat is currently being studied in the PEDFIC Phase 3 clinical program in children with PFIC. In the PEDFIC studies, a proprietary measurement tool is being used to assess pruritus and sleep disturbance. Data on the development of the proprietary patient- and observer-reported outcome (PRO and ObsRO) tools also were presented at ESPHGAN (poster number H-P-138). Based on seven waves of interviews (N=36 sessions) with 24 patients and 29 caregivers, the tools were designed as diaries completed twice daily featuring a 5-point (0-4) pictorial response scale with numeric, color-coded and verbal anchors. The study tested patients' and caregivers' understanding of the measures as intended.

**About Odevixibat**
Odevixibat is a product candidate being developed to treat rare pediatric cholestatic liver diseases and is in Phase 3 development in its initial target indication, progressive familial intrahepatic cholestasis (PFIC). 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 a Phase 3 clinical trial, PEDFIC 1, in patients with PFIC subtype 1 or 2 (NCT03566238). The PEDFIC 1 clinical trial is recruiting at more than 40 clinical trial sites worldwide. More information may be found on www.clinicaltrials.gov.

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 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 through its operating subsidiary 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 (A4250), is being developed to treat rare pediatric cholestatic liver diseases and is in Phase 3 development in its initial target indication, progressive familial intrahepatic cholestasis (PFIC). Albireo's clinical pipeline also includes two Phase 2 product candidates. Albireo's elobixibat, approved in Japan for the treatment of chronic constipation, 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, Massachusetts, and its key operating subsidiary is located in Gothenburg, Sweden. The *Boston Business Journal* named Albireo one of the 2019 Best Places to Work in Massachusetts. 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, duration or results or timing for availability of results of, development of odevixibat, elobixibat or any other Albireo product candidate or program, including regarding the Phase 3 clinical program for odevixibat in patients with PFIC; the planned pivotal trial for odevixibat in biliary atresia, the planned Phase 2 clinical trial for elobixibat in NAFLD/NASH, the target indication(s) for development, the size, design, population, location, conduct, objective, enrollment, duration or endpoints of any clinical trial, or the timing for initiation or completion of or reporting of results from any clinical trial, including the double-blind Phase 3 PFIC trial for odevixibat, the planned pivotal trial for odevixibat in biliary atresia or the planned Phase 2 trial for elobixibat in NAFLD/NASH; the potential approval and commercialization of odevixibat; the size of the PFIC population, the biliary atresia population, the NASH population or any other disease population for indications that may be targeted by Albireo; 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 pricing of odevixibat if approved; 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: 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, 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; 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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