



## Albireo Presents Data on A4250 in Children with Biliary Atresia and Alagille Syndrome at The International Liver Congress 2019

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*- Reduction in serum bile acids and pruritus observed in both populations –  
- Alagille abstract selected for inclusion in 'Best of ILC' –  
- Albireo plans to initiate second A4250 pivotal program in biliary atresia second half of 2019 –*

BOSTON, April 13, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, announced today that results in Alagille syndrome and biliary atresia patients from its completed Phase 2 clinical trial evaluating A4250 in pediatric cholestasis were presented at the European Association for the Study of the Liver (EASL) The International Liver Congress (ILC) 2019 in Vienna, Austria.

Lead investigator Ulrich Baumann, M.D., presented the Alagille results today during an oral presentation.

"Liver damage caused by cholestasis is a hallmark of Alagille syndrome, a rare and life-threatening liver disease, and there is an urgent need for a non-invasive pharmacologic treatment option," said Dr. Baumann, Professor of Pediatric Gastroenterology and Hepatology, Hannover Medical School in Hannover, Germany. "The Alagille patient data from this Phase 2 trial, including the meaningful reductions in serum bile acids and improved pruritus and sleep scores, indicate that A4250 could be a promising therapy for cholestatic liver diseases like Alagille, and suggest that further investigation of A4250 in children with Alagille is warranted."

Investigator Ekkehard Sturm, M.D., Ph.D., presented the biliary atresia results yesterday in a poster session.

"Although biliary atresia is an orphan disease, it is the most common pediatric liver disease for which transplants are done, and there are no approved pharmacological treatments. The A4250 data, though limited, are consistent with results from the overall study population of pediatric patients with chronic CLD and are encouraging," said Dr. Sturm, Head of Pediatric Gastroenterology-Hepatology, Liver and Intestinal Transplantation, Children's Hospital, University of Tuebingen in Tuebingen, Germany. "I am pleased to see that Albireo plans to embark on a biliary atresia pivotal program as the unmet need for these patients is significant."

A4250 is a highly potent and selective inhibitor of the ileal bile acid transporter (IBAT) currently being studied in a Phase 3 clinical trial in children with progressive familial intrahepatic cholestasis (PFIC), a life-threatening, rare cholestatic liver disease. Albireo plans to initiate a second A4250 pivotal trial, in biliary atresia, in the second half of 2019. Albireo estimates that biliary atresia is one of the most common rare pediatric liver diseases, with roughly 15,000 to 20,000 patients in the U.S. and EU combined and about 80% of patients requiring liver transplant within their first two decades of life.

The open-label, multicenter, dose-finding Phase 2 clinical trial assessed the safety and tolerability of A4250 in 20 children with cholestatic liver diseases, including PFIC, Alagille syndrome and biliary atresia. Efficacy endpoints included change in serum bile acid (sBA) levels and pruritus. A4250 was administered orally in doses ranging from 10 µg/kg to 200 µg/kg once daily for 4 weeks.

A4250 demonstrated marked sBA reductions of up to 92% in the majority of Alagille patients. The majority of Alagille patients also showed improvement in pruritus as measured by three different scales. One patient had an elevation in bile acids versus baseline. The abstract was selected for inclusion in EASL's 'Best of ILC' summary resource highlighting the most noteworthy contributions to its scientific program, which will be available at <https://ilc-congress.eu/slide-decks/> following the Congress.

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. A4250 demonstrated significant sBA reductions of 57.6% and 50.8% in two patients with high baseline bile acids (> 130 µmol/L) and showed improvement in pruritus across two different pruritus scales. No effect was observed in a third patient with a low baseline sBA. These data, combined with the total dataset of n=24, form the basis for a pivotal development program in a second indication, biliary atresia.

In patients with Alagille and biliary atresia, A4250 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 to not dose escalate.

The data for patients with progressive familial intrahepatic cholestasis (PFIC) were presented at a previous meeting.

"Albireo's goal is to develop a pediatric cholestasis medicine that can benefit people across multiple rare diseases," said Ron Cooper, President and Chief Executive Officer of Albireo. "These data, and our key regulatory designations in biliary atresia, Alagille and PFIC in the U.S. and the EU, reinforce the potential of A4250 to achieve that goal."

Also at EASL, the NAPPED (NATural course and Prognosis of PFIC and Effect of biliary Diversion) consortium presented data on the natural history of PFIC: "Predicting long-term outcome after surgical biliary diversion in BSEP-deficiency patients: Results from the NAPPED consortium" (abstract number PS-195). Notably, the NAPPED consortium data showed that surgical biliary diversion profoundly decreased sBA in BSEP-deficient patients with mild or moderate PFIC Type 2 genetic severity. NAPPED is supported by an unrestricted grant from Albireo.

### About A4250

A4250 is a first-in-class 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),

A4250 has minimal systemic exposure and acts locally in the small intestine.

The PFIC A4250 program, or elements of it, have received fast track, rare pediatric disease and orphan drug designations in the United States. In addition, the FDA has granted orphan drug designation to A4250 for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis. The European Medicines Agency (EMA) has granted A4250 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 A4250 Pediatric Investigation Plan for PFIC. EMA also has granted orphan designation to A4250 for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis.

A4250 is currently being evaluated in a Phase 3 clinical program, PEDFIC 1, in patients with PFIC, subtype 1 or 2 ([NCT03566238](https://clinicaltrials.gov/ct2/show/study/NCT03566238)). The PEDFIC 1 clinical trial is currently recruiting in over 35 clinical trial sites in 14 countries worldwide. More information may be found on [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

#### **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, 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. For more information on Albireo, please visit [www.albireopharma.com](http://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 A4250 or any other Albireo product candidate or program, including regarding the Phase 3 clinical program for A4250 in patients with PFIC; the target indication(s) for development, the size, design, population, location, conduct, objective, 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 A4250; 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 A4250, or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential benefits of a rare pediatric disease designation, the potential benefits of an orphan drug designation, the potential benefits of a fast track designation, the pricing of A4250 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 A4250 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 A4250; 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 A4250 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 A4250, 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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