



## Albireo Reports Data on Pharmacodynamic Marker for A4250 in Children with Cholestatic Liver Disease at The International Liver Congress™ 2018

April 14, 2018

— Correlation observed between reduction in serum bile acids and autotaxin with A4250 —

— Autotaxin has potential to be important biomarker of cholestatic pruritus —

BOSTON, April 14, 2018 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq:ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, announced that data on a pharmacodynamic marker measured in its completed Phase 2 clinical trial evaluating lead product candidate A4250 in children with cholestatic liver disease and pruritus were presented today by trial investigator Emmanuel Gonzalès, M.D., Ph.D., during a poster session at the European Association for the Study of the Liver (EASL) The International Liver Congress 2018 in Paris.

The lysophospholipase autotaxin (ATX) and its product, lysophosphatic acid, have been shown to be increased in patients with pruritus due to cholestasis. In this study, treatment with A4250 decreased ATX levels in most patients, and there was a statistically significant correlation with a reduction in serum bile acids (sBA) ( $r=0.60$ ;  $p=0.003$ ). Pruritus intensity (VAS-itch) at baseline was significantly correlated with baseline levels of ATX ( $r=0.67$ ;  $p=0.001$ ). Correlations between reduction in ATX and reduction in pruritus did not reach statistical significance ( $r=0.39$ ;  $p=0.075$ ).

"Improvements in pruritus have been reported previously in this study to be correlated with reductions in serum bile acids, but it is notable that there was a correlation between reductions in serum bile acids and reductions in ATX with an IBAT inhibitor in this patient population," said Dr. Gonzalès, Professor of Pediatric Hepatology and Liver Transplantation at University Hospitals of Paris-Sud in France. "Further studies to investigate the role and importance of ATX as a biomarker of cholestatic pruritus in children are warranted."

"We believe that these data demonstrating an association between a reduction in serum bile acids and a decrease in autotaxin levels are encouraging, particularly given the correlation between reduction in serum bile acids and pruritus in this study," said Paresh Soni, M.D., Ph.D., Albireo's Chief Medical Officer. "We look forward to commencing our Phase 3 clinical trial of A4250 for the treatment of patients with PFIC this spring, as we work to help children with cholestatic liver disease."

A4250 is a highly potent and selective IBAT inhibitor that has minimal systemic exposure and acts locally in the gut. The open label, multicenter, Phase 2 clinical trial evaluated changes in serum bile acid levels, pruritus, sleep disturbance and explorative markers, such as ATX protein levels. Twenty patients, ages 1 to 17 years old with a pediatric cholestatic liver disease, including progressive familial intrahepatic cholestasis (PFIC subtype 1, 2 or 3), Alagille syndrome or biliary atresia were administered A4250 orally once daily for 4 weeks. Five different doses of A4250 were evaluated, ranging from 10 µg/kg to 200 µg/kg.

As previously reported, A4250 also reduced sBA and improved pruritus, a common and debilitating manifestation of cholestatic liver disease, in most patients during a 4-week treatment period. A statistically significant correlation between reduction in sBA levels and improvement in pruritus scores was observed, as well as improvement in sleep disturbance. Pharmacodynamic markers measured in addition to autotaxin were: ALP, GT, AST, ALT, conjugated and total bilirubin, FGF19 and C4.

A4250 exhibited a favorable overall tolerability profile in the study, with all patients completing the 4-week treatment period and no reports of diarrhea associated with multiple dose therapy. There were no serious adverse events reported in the study that were considered to be drug related. Most adverse events, including some increased transaminases, were mild, transient and assessed as either unrelated to study drug or the relationship was unclear.

### About A4250

A4250 is a first-in-class product candidate directed to treat rare pediatric cholestatic liver diseases and is entering 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 gut.

A4250 has been granted orphan drug designation for PFIC in the United States and European Union. The European Medicines Agency (EMA) has also granted A4250 access to the PRiority MEDicines (PRIME) program for the treatment of PFIC, and its Paediatric Committee has agreed to Albireo's A4250 Pediatric Investigation Plan.

### 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 directed to treat rare pediatric cholestatic liver diseases and is in Phase 3 development in its initial target indication, progressive familial intrahepatic cholestasis. 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).

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