Albireo Granted Orphan Designation by European Commission For Lead Product Candidate A4250 for Treatment of Biliary Atresia

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Expands lead product candidate's development potential in rare cholestatic liver diseases

Provides 10 years market exclusivity upon approval plus 2 years upon completion of PIP

BOSTON, Dec. 18, 2018 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced the European Commission has granted orphan designation to lead product candidate A4250, an ileal bile acid transporter (IBAT) inhibitor, for the treatment of biliary atresia, a rare and life-threatening liver disease with no approved pharmacologic treatment option.

A4250 also holds orphan drug designations in both the U.S. and EU for the treatment of progressive familial intrahepatic cholestasis (PFIC), Alagille syndrome and primary biliary cholangitis (PBC).

The European Commission grants orphan designation to medicines intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically disabling, and that has a prevalence of not more than 5 in 10,000 in the EU. Orphan designation allows for protocol assistance, and streamlined regulatory review processes and registration in addition to 10 years of market exclusivity upon regulatory approval. Albireo could be eligible for an additional 2 years of market exclusivity following completion of a pediatric investigation plan (PIP).

"We are pleased to receive orphan designation from the European Commission for A4250 for the treatment of biliary atresia," said Ron Cooper, President and Chief Executive Officer of Albireo. "This latest regulatory milestone builds on our recent fast track designation in PFIC and orphan drug designation in Alagille syndrome in the U.S., and underscores the widespread need for new therapies to treat rare cholestatic liver diseases and the potential of A4250. We plan to expand the development of A4250 in rare cholestatic liver diseases in 2019."

Biliary atresia is a rare disease of the liver and bile ducts with symptoms developing about 2-8 weeks after birth. In biliary atresia, damaged or absent bile ducts result in bile and bile acids being trapped inside the liver, quickly resulting in damage and scarring of the liver cells (cirrhosis), and even liver failure. About 80 percent of patients with biliary atresia (with or without Kasai hepatoportoenterostomy) ultimately need a liver transplant within the first two decades after birth. Biliary atresia is estimated to affect between 4.5 and 8.5 in every 100,000 children born worldwide.

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 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 Paediatric 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 in patients with PFIC, subtype 1 or 2 (NCT03566238).

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. 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.

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, 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 Alagille population, the biliary atresia population or any other disease population for indications that may be targeted by Albireo; the potential benefits or competitive position of A4250; the potential benefits of any regulatory designation, including rare pediatric disease designation, orphan drug designation, orphan designation or fast track designation. 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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