FDA Grants Rare Pediatric Disease Designation to A4250; Albireo Eligible to Apply for Priority Review Voucher

June 12, 2018

BOSTON, June 12, 2018 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq:ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation to lead product candidate A4250, an ileal bile acid transporter (IBAT) inhibitor, for the treatment of progressive familial intrahepatic cholestasis (PFIC), a rare and life-threatening liver disease with no approved pharmacologic treatment option.

“This designation affirms Albireo’s eligibility to apply for a rare pediatric disease priority review voucher upon submission of a new drug application for A4250 and highlights the serious, life-threatening manifestations of PFIC,” said Ron Cooper, President and Chief Executive Officer of Albireo. “A priority review voucher is a very valuable and important component of the incentives to develop products for rare, life-threatening diseases.”

Albireo recently announced the enrollment of the first patient in PEDFIC-1, a single, randomized, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate A4250 in 60 patients aged 6 months to 18 years with PFIC (subtype 1 or 2) who have elevated serum bile acid (sBA) levels and pruritus. If successful, data from the Phase 3 trial and an open-label extension study are expected to form the basis of the drug approval applications for A4250 in the U.S. and E.U. for the treatment of patients with PFIC.

The FDA grants rare pediatric disease designation for diseases with serious or life-threatening manifestations that primarily affect people aged from birth to 18 years, and that affect fewer than 200,000 people in the U.S. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application or biologics license application for a product for the prevention or treatment of a rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain priority review for any subsequent marketing application, and may be sold or transferred.

In addition to the rare pediatric disease designation, A4250 received orphan drug designation for PFIC in the U.S. and E.U. in 2012, and Albireo was granted access in 2016 to the EMA's PRIority MEdicines (PRIME) program for the treatment of PFIC.

PFIC is estimated to affect between one in every 50,000 to 100,000 children born worldwide and causes progressive, life-threatening liver disease. Moderate to severe pruritus is a common and problematic clinical presentation of PFIC that can severely diminish quality of life. In many cases, PFIC leads to cirrhosis and liver failure within the first 10 years of life, and nearly all patients with PFIC require treatment before age 30. There are currently no approved pharmacological treatment options for PFIC.

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 gut.

A4250 has been granted rare pediatric disease and orphan drug designations for PFIC in the United States. The European Medicines Agency (EMA) has also granted A4250 orphan drug designation, as well as 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. A4250 is currently being evaluated in a Phase 3 clinical program in patients with PFIC (subtype 1 or 2).

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 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 or any other disease population for indications that may be targeted by Albireo; the potential benefits or competitive position of A4250, elobixibat or any other Albireo product candidate or program or the commercial opportunity in any target indication; 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 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; 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.

Investor Contact:
Hans Vitzthum
LifeSci Advisors, LLC.
212-915-2568

Media Contact:
Heather Anderson
6 Degrees
980-938-0260
handerson@6degreespr.com

Source: Albireo Pharma, Inc.

Primary Logo

Source: Albireo Pharma, Inc.