Albireo Recognizes PFIC Awareness Day and Highlights Urgent Needs of Progressive Familial Intrahepatic Cholestasis Families

October 3, 2019

Grassroots PFIC community advocates to build awareness of rare, life-threatening liver disease

BOSTON, Oct. 03, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (NASDAQ: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, announced today its support for PFIC Awareness Day, and reaffirmed its commitment to patients and families living with progressive familial intrahepatic cholestasis (PFIC).

PFIC is an ultra-rare, life-threatening, pediatric liver disease characterized by pruritus (intense itching), jaundice (elevated serum bilirubin levels that may manifest as yellowing of the skin), and poor weight gain and growth. People with PFIC are often diagnosed in infancy or early childhood and can progress to cirrhosis and liver failure by age 10 without surgical intervention. Children even undergo liver transplant due to the relentless pruritus, and this has risks and long-term challenges.

On October 5, 2019, grassroots organization PFIC Advocacy and Resource Network, Inc. ("PFIC Network," www.PFIC.org) will hold the first ever PFIC Awareness Day. Members of the PFIC community all over the world will host lemonade stand fundraisers to raise awareness in their communities. In collaboration with Life Gave Lemons, the PFIC Network is providing families with lemonade stand fundraising toolkits that empower them to become advocates. Among other resources, it includes PFIC brochures, stickers and advocacy bracelets.

"These fundraising events bring our community closer and really empower the kiddos—those that are living with this unbelievably challenging condition," said Lisa Crompton, Co-Director of PFIC Network. "Our children who are affected by PFIC are resilient, gritty and motivated to build a strong support system for families while we wait for urgently-needed treatments."

Albireo shares perspectives about the impact of PFIC and the critical need for education and treatment through *PFIC Voices*, www.PFICvoices.com. Families affected by the condition tell their perspectives about life with this debilitating condition as part of the global initiative to build awareness of PFIC.

"PFIC Awareness Day is an opportunity to stand in support of the PFIC community, as we work to develop a potential new treatment option for people with PFIC," said Ron Cooper, President and Chief Executive Officer of Albireo.

Albireo's Phase 3 PEDFIC clinical trial program for lead investigational product candidate odevixibat in PFIC consists of two clinical trials. PEDFIC 1 is a randomized, double-blind, placebo-controlled, global multicenter clinical trial designed to enroll approximately 60 patients with PFIC type 1 or type 2. PEDFIC 2 is a long-term, open-label extension study of PEDFIC 1 and includes a second cohort of PFIC patients who do not meet eligibility criteria for PEDFIC 1, but have elevated serum bile acid levels and pruritus. This includes patients with all types of PFIC, and patients younger than 6 months or older than 18 years of age. The company expects topline data in mid-2020. There are currently 45 sites actively recruiting for the PEDFIC program.

About PFIC

Progressive familial intrahepatic cholestasis (PFIC) is a rare genetic disorder that is estimated to affect between one in every 50,000 to 100,000 children born worldwide and causes progressive, life-threatening liver disease. People diagnosed with PFIC have impaired bile flow, or cholestasis, caused by genetic mutations. The resulting bile build-up in liver cells causes liver disease and symptoms. The most prominent and problematic ongoing manifestation of the disease is pruritus (intense itching), which often results in a severely diminished quality of life. PFIC is also characterized by jaundice (yellowing of the skin), and poor weight gain and growth. In many cases, PFIC leads to cirrhosis and liver failure within the first 10 years of life, and nearly all people with PFIC require treatment before age 30. There are no medicines currently approved for PFIC, only surgical options, including a procedure known as partial external biliary diversion (PEBD), and liver transplantation. These options carry substantial risks. Additional information on PFIC is available at https://www.pfic.org and https://www.pficvoices.com

About Albireo

Albireo Pharma is a clinical-stage biopharmaceutical company focused 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, 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 was spun out from AstraZeneca in 2008. Albireo Pharma is located in Boston, Mass., 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, including regarding the Phase 3 clinical program for odevixibat in patients

with PFIC;, 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 potential approval and commercialization of odevixibat; 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 odevixibat or the commercial opportunity in any target indication. 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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