



Albireo Announces Launch of “PFIC Voices”

June 20, 2019

New Initiative to Create Awareness and Understanding of Life-Threatening Pediatric Liver Disease

Announced new PFIC Voices website, www.PFICvoices.com

BOSTON, June 20, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, announced today the launch of *PFIC Voices*, a new global campaign to build awareness and understanding of the burden of *progressive familial intrahepatic cholestasis* (PFIC), an ultra-rare, genetic, pediatric liver disease. Through *PFIC Voices*, Albireo is joining with leaders from the patient advocacy community, families and clinicians to share perspectives about the impact of PFIC, and the critical need for education, treatment and support.

“Imagine the heartbreak of trying to console a child who is scratching herself so much day and night that she tears through her skin to the point of bleeding,” said Emily Ventura, leader of PFIC Network (www.pfic.org), and mother to a PFIC patient. “This is the experience of patients living with PFIC, and it affects the entire family. We are confronted with life-altering challenges in the present and an uncertain future. While we have made important progress in raising awareness in recent years, we must all join in the effort to support research that could lead to treatment. *PFIC Voices* is a way for patient and parent voices to be heard.”

PFIC is rare but devastating, and those affected are desperate for relief—for their child and for the whole family. It is an inherited liver disease in which normal bile flow is disrupted, elevating bile acids to toxic levels. In many cases, patients diagnosed with PFIC progress to cirrhosis and experience end-stage liver disease by 10 years old. PFIC is estimated to affect one in every 50,000 to 100,000 children born worldwide. It is characterized by pruritus (intense itching), jaundice (yellowing of the skin), nutrient malabsorption, and poor weight gain and growth. PFIC is disruptive to sleep, leading to irritability and daytime fatigue for all family members. Itching may be so severe that it drives the decision to seek liver transplant. Liver transplant and other surgical interventions may fail, have complications, or bring psychological and social issues.

“There are no approved treatments for PFIC, and healthcare providers have only limited options available to help manage this disease,” said Dr. Patrick McKiernan, Director, Pediatric Hepatology Program, UPMC Children’s Hospital of Pittsburgh. “These children usually need disfiguring surgeries or a liver transplant in addition to managing severe symptoms. Efforts to increase awareness of the impact of PFIC can play an important role in both improving treatment outcomes for patients, and driving research that could lead to new treatments in the years ahead.”

Albireo has been talking with parents, patients and physicians around the world to understand their experiences living with, and managing, this debilitating condition. Many are eager to share their stories. *PFIC Voices* includes a new website, www.PFICvoices.com, which seeks to provide a glimpse into life with the disease. The site currently features one family’s story, with more information to come. In addition, Albireo is sponsoring the first-ever PFIC Family Conference to be held on June 21, 2019, in Cincinnati, Ohio, in conjunction with the Alagille Syndrome Alliance’s 8th International Symposium and Scientific Meeting on Alagille Syndrome. Albireo is a supporter of the PFIC Advocacy and Resource Network, the only patient advocacy organization specifically serving the PFIC community. As part of the *PFIC Voices* effort, Albireo also provided an unrestricted educational grant to the American Liver Foundation (www.liverfoundation.org) in support of a PFIC webinar, which will be hosted on Tuesday, July 9 at 1 p.m. EDT.

“Albireo is committed to research that can lead to new and innovative treatments for rare cholestatic liver diseases,” said Ron Cooper, President and Chief Executive Officer of Albireo. “We are committed to supporting patients, parents and treaters and, through *PFIC Voices*, bring their experiences to light.”

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, odevixibat (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 in Boston, Massachusetts, 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, duration or endpoints of the double-blind Phase 3 PFIC trial for odevixibat; the size of the PFIC population; or the potential benefits or competitive position of odevixibat. 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 will be predictive of results from the trials

comprising the Phase 3 PFIC program for odeixibat 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 odeixibat 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 odeixibat, 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, enrollment, 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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