Albireo Recognizes Rare Disease Day and Highlights Urgent Need to Support Families Affected by Progressive Familial Intrahepatic Cholestasis (PFIC)

February 28, 2019

Advances in research and efforts to build awareness of life-threatening, rare, pediatric liver disease offer new hope for patients and families

BOSTON, Feb. 28, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (NASDAQ: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced its support for Rare Disease Day, and reaffirmed its commitment to patients and families living with progressive familial intrahepatic cholestasis (PFIC) and other rare cholestatic liver diseases.

“The medical need in PFIC is substantial,” said Ronald J. Sokol, MD, Professor and Vice Chair of Pediatrics, Children’s Hospital Colorado. “As a clinician, you look into the eyes of a parent of one of these children and wish there were more that you could do to manage troubling symptoms, and control or reverse the progression of the disease. There are no approved pharmacologic treatments available today, but we are learning more about PFIC, and ongoing research is providing hope.”

PFIC is a severe, progressive, potentially life-threatening, ultra-rare liver disease characterized by pruritus (intense itching), jaundice (yellowing of the skin), and poor weight gain and growth. In many cases, people with PFIC progress to cirrhosis and liver failure by age 10. Children often undergo surgery to drain bile, liver transplant or both, which have significant risks and long-term challenges.

“It’s difficult to convey the full impact that a disease like PFIC can have on a family,” said Emily Ventura, President and Co-Founder of the PFIC Advocacy and Resource Network, whose 6-year-old daughter was diagnosed with PFIC. “You may watch helplessly, as your child scratches herself through the night, then struggles in school the next day. At the same time, you worry about liver disease and how long you have until she needs a new liver. We need treatment options that help with symptoms and protect the liver. In the meantime, there’s a huge need to help families learn about PFIC and available strategies to support their children affected by this devastating disease.”

“Rare Disease Day is an opportunity to shed light on the massive challenges that people affected by rare diseases and their families face, and to ally with them in building awareness and understanding,” said Ron Cooper, President and Chief Executive Officer of Albireo. “The children and parents we’ve met are constantly in our thoughts, as we work to develop a potential new treatment option for people with PFIC and, ultimately, other rare cholestatic liver diseases.”

In recognition of Rare Disease Day, Albireo this month provided an unrestricted grant to the American Liver Foundation in support of PFIC education. At the end of 2018, Albireo made unrestricted grants to the PFIC Advocacy & Resource Network and the Alagille Syndrome Alliance in support of their efforts to improve the lives of patients and families living with rare cholestatic liver diseases.

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.

About Rare Disease Day

Rare Disease Day is held on the last day of February every year to raise awareness of rare diseases. On this day, patient organizations and advocacy groups from countries and regions around the world hold awareness-raising activities focused on the theme set each year. 2019 marks the twelfth international Rare Disease Day coordinated by EURORDIS, the European Organisation for Rare Diseases. A rare, or orphan, disease is any disorder that affects a small percentage of the population. Although the disease may be rare, patients and families share common struggles and unique challenges in the treatment and care processes. For additional information on Rare Disease Day and the organization behind it, please visit https://www.rarediseaseday.org/ and https://www.eurordis.org/.

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.

Investor Contact:

Hans Vitzthum, LifeSci Advisors, LLC., 212-915-2568

Media Contact:
Kelly Wakelee, Berry & Company Public Relations, (212) 253-8881, kwakelee@berrypr.com

Source: Albireo Pharma, Inc.

Source: Albireo Pharma, Inc.