Albireo to Present Data from Two Studies on Cholestatic Liver Disease at the 51st ESPGHAN Annual Meeting

April 19, 2018

BOSTON, April 19, 2018 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq:ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, announced today that findings from two studies that deepen the understanding of cholestatic liver disease will be presented during poster sessions at the 51st European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN) Annual Meeting, being held May 9-12, 2018, in Geneva, Switzerland.

- Data from a qualitative study illustrated the substantial impact that pediatric cholestatic liver diseases, such as progressive familial intrahepatic cholestasis (PFIC), have on quality of life. These findings were generated during development of the proprietary Patient Reported Outcome (PRO) and Observer Reported Outcome (ObsRO) tools that will be used to measure pruritus in Albireo’s planned Phase 3 study of A4250 in patients with PFIC.

- Research from a systematic literature review of partial external biliary diversion (PEBD) surgery in patients with PFIC found that changes in serum bile acids and bilirubin appear useful as biomarkers in predicting both early and long-term outcomes. Serum bile acid responder rate will be the primary endpoint in Europe and a key secondary endpoint in the United States in the planned A4250 Phase 3 study.

Details of the presentations are as follows:

**Title:** Symptoms and daily impacts associated with progressive familial intrahepatic cholestasis and other pediatric cholestatic liver diseases: a qualitative study with patients and caregivers

**Abstract Number:** A-968-0013-00837

**Date/Time:** Friday, May 11, 2-2:45 p.m. CEST

**Presenter:** Dr. Kristina Torfgård, Vice President, Global Project Head, Albireo AB, Gothenburg, Sweden

Data from the qualitative study examining symptoms and daily impacts on functioning of pediatric cholestatic liver diseases, including PFIC, Alagille syndrome, biliary atresia and primary sclerosing cholangitis, demonstrated that accompanying symptoms may greatly reduce quality of life. Pruritus was reported to be the most frequent and most disturbing symptom. Seventy-seven percent of respondents reported pruritus-related sleep disturbance, with PFIC patients and caregivers reporting the highest impact (7.8; 0-10 scale). Moreover, all respondents reported that pruritus occurred most frequently at night and all over the body, and occurred frequently upon waking and when tired or unwell. Fatigue (69%), difficulty focusing (46%) and scarring (46%) were other commonly experienced impacts, with fatigue and sleep disturbance similarly experienced across PFIC and non-PFIC patients.

**Title:** Systematic literature review of the effect of partial external biliary diversion surgery on clinical and biochemical outcomes in progressive familial intrahepatic cholestasis patients

**Abstract Number:** A-968-0013-00212

**Date/Time:** Friday, May 11, 2-2:45 p.m. CEST

**Presenter:** Dr. Henkjan Verkade, Department of Pediatrics, Beatrix Children's Hospital, Groningen, The Netherlands

Research from the systematic literature review of partial external biliary diversion (PEBD) surgery in patients with PFIC found that changes in serum bile acids and bilirubin after PEBD appear useful as biomarkers in predicting outcomes both early (e.g., relief of pruritus) and long term (e.g., need for liver transplant) following PEBD surgery.

Reductions in bile acids and bilirubin were independently highly associated with decreased aggregate need for a liver transplant following PEBD surgery. Prior to surgery, bile acids were 25-35 times greater than the upper limit of normal (mean: 322 µmol/L). Reduction in bile acids following PEBD surgery correlated with positive early clinical outcomes with high sensitivity and specificity (area under the curve [AUC], 0.99; p<0.0001). Baseline bilirubin levels were typically 5-8 times greater than the upper limit of normal (mean: 94 µmol/L); however, variability was high and several patients expressed levels close to normal. Reductions in bilirubin levels following PEBD surgery correlated with positive early clinical outcomes with good sensitivity and specificity (AUC, 0.87; p=0.003).

Alanine aminotransferase (ALT) levels were not associated with a decreased aggregate need for a liver transplant following PEBD surgery. Baseline ALT levels were typically 1-4 times greater than the upper limit of normal (mean: 168 U/L) and highly variable. ALT reductions did not significantly correlate with early positive outcomes (AUC, 0.74; p=NS).

For more information about the abstracts and meeting, visit [http://www.espghancongress.org/](http://www.espghancongress.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 directed 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: Sarah Hall, 6 Degrees, 215-313-5638, shall@6degreespr.com

Source: Albireo Pharma, Inc.