Albireo to Present New Data at the 54th Annual Meeting of ESPGHAN

June 7, 2022

– Four abstracts accepted of Phase 3 data from PEDFIC 1 and PEDFIC 2 trials of Bylvay™ (odevixibat)

– Three oral presentations discuss long-term safety, tolerability and improved quality of life, hepatic biochemical markers, sleep in children with PFIC

– Company-sponsored symposium to feature case-based expert discussion on real-life experience of treating PFIC with an IBAT inhibitor

BOSTON, June 07, 2022 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a rare liver disease company developing novel bile acid modulators, today announced the presentation of data at the European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN), being held June 22 – 25, 2022 in Copenhagen, Denmark. Data will be presented in three oral sessions and one poster, sharing analyses of the landmark Phase 3 PEDFIC 1 study and PEDFIC 2 long-term extension study of Bylvay in children with progressive familial intrahepatic cholestasis (PFIC). These studies represent the largest Phase 3 clinical trials ever completed in PFIC. The full list of Albireo presentations is listed below with abstracts on the ESPGHAN website here.

Oral Presentation: *Efficacy and Safety Outcomes in Patients with Progressive Familial Intrahepatic Cholestasis Who Had an Odevixibat Dose Escalation: Pooled Results from the PEDFIC 1 and PEDFIC 2 Studies*
Presenter: Dr. Tassos Grammatikopoulos, Consultant in Paediatric Hepatology & Honorary Clinical Senior Lecturer, Institute of Liver Studies, King's College London
Session Title: Parallel Session: Hepatology 1 – Around PFIC and Hyperbilirubinemia
Date & Time: Thursday, 23 June, 17:00 CEST

Oral Presentation: *Analysis of Quality of Life, Hepatic Biochemical Markers, and Sleep in Patients With Progressive Familial Intrahepatic Cholestasis Who Had a Pruritus Response With Odevixibat Treatment*
Presenter: Dr. Girish Gupte, Liver Unit and Small Bowel Transplantation, Birmingham Women's and Children's NHS Foundation Trust
Session Title: Parallel Session: Hepatology 1 – Around PFIC and Hyperbilirubinemia
Date & Time: Thursday, 23 June, 17:10 CEST

Oral Presentation: *Changes in Hepatic Parameters, Growth, Sleep, and Biochemical Markers With Odevixibat Treatment Across Patients With Various Types of Progressive Familial Intrahepatic Cholestasis*
Presenter: Dr. Lorenzo D'Antiga, Department of Paediatric Hepatology, Gastroenterology, and Transplantation, Azienda Ospedaliera Papa Giovanni XXIII
Session Title: Plenary Session: Hepatology
Date & Time: Friday, 24 June, 09:15 CEST

e-Poster Abstract #363: *Total, Primary, and Secondary Serum Bile Acid Concentrations in Patients With Progressive Familial Intrahepatic Cholestasis With Serum Bile Acid Response or Not With Odevixibat Treatment: Assessing the Contribution of Ursodeoxycholic Acid Concentration*
Lead Author: Dr. Henkjan J. Verkade, Department of Paediatrics, University of Groningen, Beatrix Children's Hospital/University Medical Centre Groningen
Date & Time: Friday, 24 June, 14:00 CEST

Breakfast Symposium: Real-life experience of treating PFIC with an IBAT inhibitor
Expert panel: Prof. Patrick McKiernan, Birmingham Children's Hospital, NHS Foundation Trust, UK, Dr Christoph Leiskau, Hannover Medical School, Germany, Dr Angelo Di Giorgio, Hospital Papa Giovanni XXIII, Bergamo, Italy
Date & Time: Saturday, 25 June, 7:15-8:15 CEST

About Bylvay (odevixibat)

Bylvay is the first drug approved in the U.S. for the treatment of pruritus in patients 3 months of age and older in all types of progressive familial intrahepatic cholestasis (PFIC). Limitation of Use: Bylvay may not be effective in PFIC type 2 patients with ABCB11 variants resulting in non-functional or complete absence of bile salt export pump protein (BSEP-3). The European Commission (EC) and UK Medicines and Healthcare Products Regulatory Agency (MHRA) have also granted marketing authorization of Bylvay for the treatment of PFIC in patients aged 6 months or older. Bylvay is available in Germany and the UK and will be available for sale in other European countries following pricing and reimbursement approval. A potent, once-daily, non-systemic ileal bile acid transport inhibitor, Bylvay acts locally in the small intestine. Bylvay can be taken as a capsule for patients that are able to swallow capsules, or opened and sprinkled onto food, which is a factor of key importance for adherence in a pediatric patient population. The most common adverse reactions for Bylvay are diarrhea, liver test abnormalities, vomiting, abdominal pain, and fat-soluble vitamin deficiency. The medicine can only be obtained with a prescription. For more information about using Bylvay, see the package leaflet or contact your doctor or pharmacist. For full prescribing information, visit www.bylvay.com.

In the U.S. and Europe, Bylvay has orphan exclusivity for its approved PFIC indications, and orphan designations for the treatment of ALGS, biliary atresia and primary biliary cholangitis. Bylvay is being evaluated in the ongoing PEDFIC 2 open-label trial in patients with PFIC, in the BOLD Phase 3 study for patients with biliary atresia and the ASSERT Phase 3 study for ALGS.

Important Safety Information

- The most common adverse reactions for Bylvay are diarrhea, liver test abnormalities, vomiting, abdominal pain, and fat-soluble vitamin deficiency.
- Liver Test Abnormalities: Patients should obtain baseline liver tests and monitor during treatment. Dose reduction or treatment interruption may be required if abnormalities occur. For persistent or recurrent liver test abnormalities, consider treatment discontinuation.
- Diarrhea: Treat dehydration. Treatment interruption or discontinuation may be required for persistent diarrhea.
- Fat-Soluble Vitamin (FSV) Deficiency: Patient should obtain baseline vitamin levels and monitor during treatment. Supplement if deficiency is observed. If FSV deficiency persists or worsens despite FSV supplementation, discontinue treatment.

About Albireo

Albireo Pharma is a rare disease company focused on the development of novel bile acid modulators to treat rare pediatric and adult liver diseases. Albireo's lead product, Bylvay, was approved by the U.S. FDA as the first drug for the treatment of pruritus in all types of progressive familial intrahepatic cholestasis (PFIC), and it is also being developed to treat other rare pediatric cholestatic liver diseases with Phase 3 trials in Alagille syndrome (ALGS) and biliary atresia, as well as Open-label Extension (OLE) studies for PFIC and ALGS. In Europe, Bylvay has been approved for the treatment of PFIC with pricing listing in Germany and guidance from the National Institute for Health and Care Excellence (NICE) recommending Bylvay for use in the National Health Service in England, Wales and Northern Ireland. The Company has also completed a Phase 1 clinical trial for A3907 to advance development in adult cholestatic liver disease, with IND-enabling studies progressing with A2342 for viral and cholestatic liver disease. Albireo was spun out from AstraZeneca in 2008 and is headquartered in Boston, Massachusetts, with its key operating subsidiary 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: Albireo's commercialization plans; the plans for, or progress, scope, cost, initiation, duration, enrollment, results or timing for availability of results of, development of Bylvay, A3907, A2342 or any other Albireo product candidate or program; the PEDFIC 2 open-label trial in patients with PFIC; the pivotal trial for Bylvay in biliary atresia (BOLD); the pivotal trial for Bylvay in Alagille syndrome (ASSERT); the Phase 2 study for A3907 the IND-enabling or clinical studies for A2342; the target indication(s) for development or approval; the timing for initiation or completion of or availability or reporting of results from any clinical trial, including the long-term open-label extension study for Bylvay in PFIC, the BOLD and ASSERT trials, the Phase 2 study for A3907, and the IND-enabling and clinical studies for A2342; potential regulatory approval and plans for potential commercialization of Bylvay in additional countries; the potential benefits or competitive position of Bylvay or any other Albireo product candidate or program or the commercial opportunity in any target indication; future price listings and reimbursement approvals of Bylvay; the length of time for which Albireo's cash resources are expected to be sufficient; 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," or the negative of these terms or other 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: results achieved in Bylvay in the treatment of patients with PFIC may be different than observed in clinical trials, and may vary among patients; potential negative impacts of the COVID-19 pandemic, including on manufacturing, supply, conduct or initiation of clinical trials, or other aspects of our business; whether favorable findings from clinical trials of Bylvay to date, including findings in indications other than PFIC, will be predictive of results from other clinical trials of Bylvay; ; the timing for initiation or completion of, or for availability of data from, clinical trials of Bylvay, including BOLD and ASSERT and the Phase 2 clinical trial of A3907, and the outcomes of such trials; Albireo's ability to obtain coverage, pricing or reimbursement for approved products in the United States or Europe; delays or other challenges in the recruitment of patients for, or the conduct of, the Company's clinical trials; and the Company'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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