Bylvay® (odevixibat) Data Presented at AASLD The Liver Meeting® 2022, Demonstrating Native Liver Survival in Children Across PFIC Types

- *Early, rapid, and sustained efficacy across wide range of patients with evidence of disease modification*
- *Long-term preservation of native liver in PFIC, improvements in pruritus, bile acids, and increased growth over time*
- *Late-breaking oral presentation demonstrates restoration of bile acid secretion in PFIC patients receives "Best of Liver Meeting" distinction*
- *Late-breaking oral presentation of new positive Phase 3 ASSERT trial results in Alagille syndrome receives "Best of Liver Meeting" distinction*

BOSTON — November 7, 2022 — Albireo Pharma, Inc. (Nasdaq: ALBO), a rare disease company developing novel bile acid modulators to treat pediatric and adult liver diseases, presented new data at the American Association for the Study of Liver Diseases (AASLD) The Liver Meeting® 2022, being held November 4 – 8, 2022 in Washington, D.C. Across three oral presentations, including two late-breakers, and six posters, the Company provided evidence of early, rapid, and sustained efficacy with Bylvay (odevixibat) treatment in patients with progressive familial intrahepatic cholestasis (PFIC) and Alagille syndrome (ALGS).

Two key oral presentations on the PEDFIC trials provided evidence of the disease modifying effects of Bylvay in patients with PFIC. The first underscored that a decrease in serum bile acids was strongly associated with native liver survival for up to three years in PFIC patients treated with Bylvay. A second late-breaking oral presentation showed that Bylvay restored bile acid secretion in PFIC patients with bile salt export pump deficiency. The late-breaking abstract was selected by AASLD for inclusion as a key presentation in "The Best of The Liver Meeting" in the Pediatric Hepatology category.

"We are pleased to share long term data in PFIC, providing evidence that Bylvay could modify the course of disease, preserving patients' native liver and improving families' quality of life by alleviating disease burden," said Jan Mattsson, Ph.D., Chief Scientific Officer and Head of R&D at Albireo. "Furthermore, showing early, rapid, and consistent improvements in pruritus and bile acid levels in patients with Alagille syndrome, through the ASSERT body of evidence, makes for a big year at this year's AASLD congress. PFIC and Alagille syndrome are devastating childhood diseases and our goal at Albireo has always been to relieve the suffering of these young patients and their families."

"Patients with PFIC have long needed a disease modifying treatment," said Dr. Richard Thompson, Professor of Molecular Hepatology at King's College London, and principal investigator of the PEDFIC 1 and PEDFIC 2 trials. "Sadly, most children with PFIC end up having a liver transplant, with fewer than half of PFIC1 patients and less than a third of PFIC2 patients keeping their native liver through their eighteenth birthday. I am encouraged by long-term data that showed that in patients on odevixibat who reduced their serum bile acids after six months, there was a reduction in the need for liver transplantation.

This supports the importance of treating children with odevixibat so that they can benefit from the protective effects we are seeing on the liver."

Along with results observed with Bylvay treatment in PFIC patients, Albireo shared the first detailed results of the Phase 3 ASSERT trial in Alagille syndrome (ALGS) in a late-breaking oral presentation. The data showed that Bylvay provided early, rapid, clinically meaningful, and sustained improvements in pruritus, as well as significant reductions in bile acids and improvements in sleep quality in patients with ALGS. The ASSERT abstract was selected by AASLD for inclusion as a key presentation in "The Best of The Liver Meeting" in the Pediatric Hepatology category.

Analyses of the ASSERT and PEDFIC trials and real-world data on Bylvay treatment, as well as data on the investigational therapy A3907, were highlighted in the following presentations at AASLD The Liver Meeting:

Early, Rapid, Sustained Effects in Alagille Syndrome

Oral, Late-Breaking Parallel Session Presentation (Abstract #5005; Publication #38786): *Efficacy and Safety of Odevixibat in Patients with Alagille Syndrome: Top-Line Results from ASSERT, a Phase 3, Double-Blind, Randomized, Placebo-Controlled Study*
Presenter: Dr. Nadia Ovchinsky, Children's Hospital at Montefiore, Albert Einstein College of Medicine, Bronx, NY, USA
Session: Late-Breaking Oral Abstract Session 1; Monday, November 7, 2022, 9:15 AM EST

Top-line, unblinded data from the ASSERT study demonstrated that Bylvay treatment led to significant and clinically meaningful improvements in pruritus, as well as reductions in bile acid levels and improvements in sleep parameters in patients with ALGS. Over 90% of patients were pruritus responders and the treatment effects were early, rapid, and sustained. Bylvay was generally well tolerated; the overall incidence of treatment emergent adverse events (TEAEs) was similar to placebo. No patients discontinued the study and 96% of patients rolled over into the open-label extension study.

Disease Modification in PFIC

Oral Presentation (Abstract #865): *Native Liver Survival in Odevixibat Serum Bile Acid Responders: Data from the PEDFIC Studies in Patients with Progressive Familial Intrahepatic Cholestasis*
Presenter: Dr. Richard J. Thompson, Institute of Liver Studies, King's College London
Session: Genes to Cures: What's New in Pediatric Liver Disease; Sunday, November 6, 2:10 PM EST

Pooled data analysis showed that PFIC patients who responded to Bylvay remained liver transplant free for up to three years. A decrease in serum bile acids (sBAs) at six months of treatment was strongly associated with native liver survival ($p=0.0050$) for sBA responders vs non-responders.

Oral, Late-Breaking Parallel Session Presentation (Abstract #5004; Publication #38801): *Odevixibat Treatment in Responsive Patients with Bile Salt Export Pump Deficiency Restores Biliary Bile Acid Secretion, as Indicated by Serum Bile Acid Composition*
Presenter: Dr. Mark Nomden, Department of Surgery and Pediatrics, University Medical Center Groningen, Groningen, the Netherlands
Session: Late-Breaking Oral Abstract Session 1; Monday, November 7, 2022, 10:00 AM EST

The analysis found that responders not only had a decrease in sBA concentration, but also alternations in sBA composition, more like that of a healthy individual. Data indicated that Bylvay restored biliary bile acid secretion in treatment-responsive patients with BSEP deficiency.

Durable Improvement Across Wide Range of PFIC Patients

Poster (Abstract #37939): *Long-term Efficacy and Safety of Odevixibat in Patients with Progressive Familial Intrahepatic Cholestasis: Results with 96 Weeks or More of Treatment*
Presenter: Dr. Richard J. Thompson, Institute of Liver Studies, King's College London
Session: Poster Session IV; Monday, November 7, 1:00 PM – 2:00 PM EST

Pooled data analysis showed Bylvay treatment for 96 weeks or more was associated with durable clinical benefits in patients with PFIC, with improvements over time in mean sBA and aminotransferase levels and growth. Mean levels of alanine aminotransferase (ALT) and aspartate aminotransferase (AST) decreased over time, while mean total bilirubin levels were relatively unchanged. Mean height and weight Z scores also increased over time and Bylvay was generally well tolerated.

Poster (Abstract #37254): *Serum Bile Acid Levels, Pruritus Scores, and Growth Over Time in Odevixibat Responders: Pooled Data from the PEDFIC Studies in Patients with Progressive Familial Intrahepatic Cholestasis*
Presenter: Dr. Lorenzo D'Antiga, Department of Paediatric Hepatology, Gastroenterology, and Transplantation, Azienda Ospedaliera Papa Giovanni XXIII, Bergamo, Italy
Session: Poster Session IV; Monday, November 7, 1:00 PM – 2:00 PM EST

This analysis of pooled data examined treatment effects in 49 Bylvay responders who had a mean duration of exposure of 110 weeks and showed mean reductions in sBAs and pruritus were sustained over time, across PFIC types in general. Patients also had mean improvements in growth.

Poster (Abstract #37273): *Effect of Odevixibat in Patients with Progressive Familial Intrahepatic Cholestasis Type 2 with at Least 1 Severe Mutation (BSEP3 Compound Heterozygotes): Pooled Data from the PEDFIC 1 and PEDFIC 2 Studies*
Presenter: Dr. Henkjan J. Verkade, Department of Paediatrics, University of Groningen, Beatrix Children's Hospital/University Medical Centre Groningen, Groningen, the Netherlands
Session: Poster Session IV**,** Monday; November 7, 1:00 PM – 2:00 PM EST

Partial external biliary diversion (PEBD) is not effective in PFIC2 patients with BSEP 3 mutations, but this pooled data analysis showed that treatment with Bylvay provided substantial reductions in sBAs and/or pruritus severity in most patients with BSEP2/BSEP3 mutations, demonstrating that Bylvay may provide an alternative to surgical intervention for these patients.

Poster (Abstract #37608): *Odevixibat Therapy in Patients with FIC1-Deficient Progressive Familial Intrahepatic Cholestasis and Diarrhea Following Liver Transplantation That Impacted Daily Activities: A Retrospective Case Series*
Presenter: Georg-Friedrich Vogel, Assistant Professor, Medical University of Innsbruck, Innsbruck, Austria
Session: Poster Session IV; Monday, November 7, 1:00 PM – 2:00 PM EST

Chologenic diarrhea can be a frequent and severe symptom after liver transplant in patients with FIC1 deficiency. Real-world data in three PFIC1 patients indicated that treatment with Bylvay can improve diarrhea and quality of life in PFIC1 patients with severe diarrhea after transplant.

Poster (Abstract #850): *Odevixibat Treatment in Patients with Recurrent Episodic Cholestasis and Biallelic Mutations in ATP8B1: A Retrospective Case Series*
Presenter: Dr. Angelo Di Giorgio, Hospital Papa Giovanni XXIII, Bergamo, Italy
Session: Poster Session IV; Monday, November 7, 1:00 PM – 2:00 PM EST

Recurrent episodic cholestasis is a rare disease characterized by episodes of cholestasis followed by periods of remission. In a case series of six patients treated with Bylvay during a cholestasis episode, most patients experienced clinical improvement, including improvements in sBA levels, hepatic laboratory parameters, pruritus, sleep disturbances and impacts on daily life. Before starting Bylvay treatment, all 6 patients had high sBAs and severe pruritus leading to sleep or mood disturbances and/or the inability to attend school, play sports or work.

ASBTi A3907 for Cholestatic Liver Diseases

The apical sodium-dependent bile acid transporter (ASBT) plays an important role in regulation of bile acid homeostasis by promoting the reuptake of bile acids in the ileum, bile ducts, and proximal tubuli of the kidney. A3907 is an investigational therapy that is designed to inhibit ASBT to potentially benefit people with cholestatic liver diseases.

The study showed that A3097 improved the general condition and liver phenotype of mice with induced obstructive cholestasis by promoting urinary secretion of bile acids, demonstrating that A3907 may have therapeutic potential for patients with cholestatic liver diseases. Treatment with A3907 resulted in marked decreases in serum and bile levels of bile acids relative to vehicle. The A3907 study was selected among the four posters for the Cholestatic and Autoimmune Liver Diseases SIG (Special Interest Group) debrief.

Poster (Abstract #37733): *Systemic ASBT Inhibition with A3907 Stimulates Urinary Excretion of Bile Acids and Halts Liver Disease Progression in Bile-Duct–Obstructed Mice*
Presenters: Drs. Peter Åkerblad and Erik Lindström, Albireo, Boston, MA, USA
Session: Poster Session III; Sunday, November 6, 1:00 PM – 2:00 PM EST

About the Phase 3 PEDFIC & ASSERT Studies

The PEDFIC trials represent the largest studies ever completed in children with PFIC, or progressive familial intrahepatic cholestasis, a rare genetic disorder that causes progressive, life-threatening liver disease. PEDFIC 1 was a randomized, double-blind, placebo-controlled Phase 3 trial that evaluated the efficacy and tolerability of Bylvay in reducing pruritus and serum bile acids (sBAs) in children with PFIC, and PEDFIC 2 is a long-term, open-label Phase 3 extension study. Patients with PFIC have impaired bile flow, or cholestasis, and the resulting bile build-up in liver cells causes liver disease and symptoms, such as intense itching, poor sleep, delayed growth, and diminished quality of life. The harmful impacts of the disease extend to parents and caregivers, as the 2022 multinational PICTURE study revealed that PFIC negatively affects caregivers' quality of life, relationships, and career prospects.

ASSERT is a gold standard, prospective intervention trial in Alagille syndrome, or ALGS, a rare, multisystem genetic disorder that can affect the liver, heart, skeleton, eyes, central nervous system, kidneys, and facial features. With 32 sites across North America, Europe, Middle East, and Asia Pacific, the double-blind, randomized, placebo-controlled ASSERT trial was designed to evaluate the safety and efficacy of 120 µg /kg/day Bylvay for 24 weeks in relieving pruritus in patients with Alagille syndrome (ALGS). Key secondary endpoints measure serum bile acid levels, safety, and tolerability. The Company estimates that ALGS impacts 25,000 people globally. In people with ALGS, liver damage is caused by a paucity of bile ducts preventing bile flow from the liver to the small intestine. Approximately 95% of patients with the condition present with chronic cholestasis, usually within the first three months of life, and as many as 88 percent also present with severe, intractable pruritus.

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. A potent, once-daily, non-systemic ileal bile acid transport inhibitor, Bylvay has minimal systemic exposure and 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 open-label trial 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 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 a completed Phase 3 trial in Alagille syndrome (ALGS), an ongoing Phase 3 study in biliary atresia, as well as Open-label Extension (OLE) studies for PFIC and ALGS. In Europe, Bylvay is reimbursed for the treatment of PFIC in Germany, England, Wales & Northern Ireland, Scotland, Italy, and Belgium. 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 expected cash runway; 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 target indication(s) for development or approval; ;discussions with the FDA or EMA regarding our programs; potential regulatory approval and plans for potential commercialization of Bylvay in biliary atresia or ALGS or Albireo's other product candidates; the potential benefits or competitive position of Bylvay or any other Albireo product candidate or program or the commercial opportunity in any target indication; 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: whether the regulatory filings to be made for Bylvay in patients with ALGS will be made on the timelines we expect and be approved by the FDA and EMA; whether the FDA and EMA will complete their respective reviews within target timelines, once determined; whether the FDA and EMA will require additional information, whether we will be able to provide in a timely manner any additional information that the FDA and EMA request, and whether such additional information will be satisfactory to the FDA and EMA; there are no guarantees that Bylvay will be commercially successful; we may encounter issues, delays or other challenges in commercializing Bylvay; whether Bylvay receives adequate reimbursement from third-party payors; the degree to which Bylvay receives acceptance from patients and physicians for its approved indication; challenges associated with execution of our sales activities, which in each case could limit the potential of our product; challenges associated with supply and distribution activities, which in each case could limit our sales and the availability of our product; results achieved in Bylvay in the treatment of patients with PFIC or other approved indications 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 PFIC, ALGS and other indications, will be predictive of results from other clinical trials of Bylvay; there is no guarantee that Bylvay will be approved in jurisdictions or for indications (such as biliary atresia or ALGS) beyond the jurisdictions in which or indications for which Bylvay is currently approved; there is no guarantee that our other product candidates will be approved; estimates of the addressable patient population for target indications may prove to be incorrect; 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 Bylvay, including BOLD, 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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