Albireo Phase 3 Trial Meets Both Primary Endpoints for Odevixibat in PFIC

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-Highly statistically significant reductions in both pruritus and serum bile acids-

Albireo President and CEO Ron Cooper

-Well tolerated with very low incidence of diarrhea-

-Similar efficacy in children with PFIC1 or PFIC2-

-Pivotal trial results substantiate potential for odevixibat to be first drug for PFIC patients-

-Regulatory submissions for approval on track-

-Conference call to be held today at 8:30 a.m. EDT-

BOSTON, Sept. 08, 2020 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced positive topline results from PEDFIC 1, a global Phase 3 clinical trial evaluating the efficacy and safety of odevixibat and the largest study ever conducted in PFIC1 and PFIC2. PEDFIC 1 met its two primary endpoints, demonstrating that odevixibat reduced serum bile acid responses (sBAs) (p=0.003) and improved pruritus assessments (p=0.004) with a single digit diarrhea rate. Odevixibat is a highly potent, non-systemic ileal bile acid transport inhibitor (IBATi), for the treatment of progressive familial intrahepatic cholestasis (PFIC) patients.

In the primary analysis, the study met the U.S. regulatory primary endpoint with the proportion of positive pruritus assessments being 53.5% in the odevixibat arms compared to 28.7% in the placebo arm (p=0.004). As a secondary endpoint, 42.9% of patients in the odevixibat arms had a clinically meaningful improvement in the pruritus score, defined as a drop from baseline of 1.0 point or more on the 0-4 point scale, at week 24 compared to 10.5% in the placebo arm (p=0.018). The study also met the EU regulatory primary endpoint with 33.3% of subjects in the odevixibat arms experiencing either a 70% reduction in sBAs or reaching a level of 70 µmol/L compared to no patients in the placebo arm (p=0.003). As a secondary endpoint, mean reduction of bile acids was 114.3 µmol/L in the odevixibat arms compared to an increase of 13.1 µmol/L in the placebo arm (p=0.002). Both doses of odevixibat were statistically significant for each of the endpoints. Odevixibat was well tolerated, with an overall adverse event incidence similar to placebo. There were no drug-related serious adverse events (SAEs) reported during the study. Diarrhea/frequent bowel movements were the most common treatment-related gastrointestinal adverse events which occurred in 9.5% of odevixibat treated patients vs. 5.0% of placebo patients. Full results from the Phase 3 clinical trial will be presented at a future scientific meeting.

“The successful clinical application of IBAT inhibition is all about the ability to lower bile acids and reduce diarrhea rates. Odevixibat reduced bile acids in both PFIC1 and PFIC2 patients and demonstrated a clinically meaningful outcome in pruritus. This is exciting news for children suffering from PFIC who, if odevixibat is approved, may soon have an easy to take, once-daily drug for their life-threatening liver disease,” said Ron Cooper, President and Chief Executive Officer of Albireo. “These strong results from PEDFIC 1 increase our confidence in the ongoing BOLD pivotal trial in biliary atresia and

the Alagille syndrome study planned for later this year."

"The results of the PEDFIC 1 Phase 3 trial represent the potential for a paradigm shift in the treatment of PFIC," said Richard Thompson, MD, Professor of Molecular Hepatology at King's College London and principal investigator of the study. "These data demonstrate that odevixibat reduced serum bile acids and improved pruritus in patients with PFIC. Coupled with the favourable safety and tolerability profile odevixibat exhibited, these data underscore the potential to improve upon the current standard of care, which typically consists of off-label medications or invasive surgeries including transplant."

"PFIC is a devastating and progressive disease that impacts every facet of a person's life. These positive results represent an important milestone for the PFIC community, many of whose only option is a liver transplant or other invasive surgeries," said Emily Ventura, President & Co-Founder of the PFIC Network. "We appreciate Albireo's vital work in this area, and believe these positive data could bring us one step closer to a safe, effective drug therapy to make a real difference for patients with PFIC and their families."

PEDFIC 1 Key Topline Results

	Placebo n=20	**Odevixibat n=42**	**P-value**
Proportion of positive pruritus assessments	28.7%	53.5%	0.004
Clinically meaningful improvement in pruritus score	10.5%	42.9%	0.018
Protocol defined bile acid reduction	0%	33.3%	0.003
Absolute change in serum bile acid	13.1	-114.3	0.002
Low rate of drug-related diarrhea/frequent bowel movements	5.0%	9.5%	----

The randomized, double-blind, placebo-controlled, global multicenter PEDFIC 1 clinical trial evaluated odevixibat in 62 patients, ages 6 months to 15.9 years, with PFIC type 1 or type 2. Patients were randomized to receive either a 40 µg/kg/day or 120 µg/kg/day oral dose of the planned commercial formulation of odevixibat or placebo once daily for 24 weeks. Patients randomized to odevixibat were treated with once-daily oral capsules or sprinkles, which did not require refrigeration. PEDFIC 2, an open-label extension study (NCT03659916), is designed to assess long-term safety and durability of response. Cohort 1 of the trial allowed patients from the PEDFIC 1 clinical trial to continue treatment with odevixibat. Cohort 2 consists of patients who did not participate in the PEDFIC 1 trial, and includes other forms of PFIC not included in PEDFIC 1.

"I want to extend my deepest thanks to the Albireo team, and to the families and clinicians whose participation was integral to obtaining today's exciting odevixibat PEDFIC 1 Phase 3 data," added Cooper.

Odevixibat has the potential to become the first approved pharmacologic treatment for patients with PFIC. The Company intends to complete regulatory filings in the EU and in the U.S. no later than early 2021, in anticipation of potential regulatory approval, issuance of a rare pediatric disease priority review voucher and launch in the second half of 2021, if approved. The Company also plans to initiate a pivotal Phase 3 trial of odevixibat in Alagille syndrome by the end of 2020, and to continue enrolling patients in the BOLD pivotal Phase 3 trial of odevixibat in biliary atresia. Albireo continues to progress its pipeline, and expects to complete IND-enabling studies for a new preclinical candidate this year.

Conference Call
Albireo will host a conference call and live audio webcast at 8:30 a.m. EDT. To access the live conference call by phone, please dial 877-407-0792 (domestic) or 201-689-8263 (international), and provide the access code 13709929. The live audio webcast will be accessible from the Albireo Media & Investors page: http://ir.albireopharma.com/. To ensure a timely connection to the webcast, it is recommended that participants register at least 15 minutes prior to the scheduled start time. An archived version of the webcast will be available for replay in the Events & Presentations section of the Media & Investors page of Albireo's website for two weeks following the event.

About Odevixibat
Odevixibat is a product candidate being developed to treat rare pediatric cholestatic liver diseases, including progressive familial intrahepatic cholestasis (PFIC), biliary atresia and Alagille syndrome. A highly potent, non-systemic ileal bile acid transport inhibitor (IBATi), odevixibat has minimal systemic exposure and acts locally in the small intestine. Odevixibat does not require refrigeration and can be taken as a capsule for older children, or opened and sprinkled onto food, which are factors of key importance for adherence in a pediatric patient population. Odevixibat was evaluated in a Phase 3 clinical trial, PEDFIC 1, in patients with PFIC (NCT03566238), and is being evaluated in the ongoing PEDFIC 2 open-label trial (NCT03659916), and the BOLD Phase 3 clinical trial in patients with biliary atresia (NCT04336722). The Company anticipates initiating a pivotal trial of odevixibat for Alagille syndrome by the end of this year.

The odevixibat PFIC program, or elements of it, have received fast track, rare pediatric disease and orphan drug designations in the United States. In addition, the FDA has granted orphan drug designation to odevixibat for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis. The EMA has granted odevixibat orphan designation, as well as access to the PRIority MEdicines (PRIME) scheme for the treatment of PFIC. Its Pediatric Committee has agreed to Albireo's odevixibat Pediatric Investigation Plan for PFIC. EMA also has granted orphan designation to odevixibat for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis.

About PFIC
Progressive familial intrahepatic cholestasis (PFIC) is a rare genetic disorder that 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, or intense itching, which often results in a severely diminished quality of life. PFIC is also characterized by jaundice, 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 drugs 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.pficvoices.com.

About Albireo
Albireo Pharma is a clinical-stage biopharmaceutical company focused 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, is being developed to treat rare

pediatric cholestatic liver diseases and is in Phase 3 development in progressive familial intrahepatic cholestasis (PFIC) and biliary atresia, with a third Phase 3 trial being planned in Alagille syndrome.

Albireo was spun out from AstraZeneca in 2008. Albireo Pharma is located in Boston, Mass., and its key operating subsidiary is located in Gothenburg, Sweden. The *Boston Business Journal* named Albireo one of the 2020 Best Places to Work in Massachusetts for the second consecutive year. 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, initiation, duration, enrollment, results or timing for availability of results of, development of odevixibat or any other Albireo product candidate or program, including regarding expectations regarding the impact of COVID-19 on our business and our ability to adapt our approach as appropriate; the Phase 3 clinical program for odevixibat in patients with PFIC, the pivotal trial for odevixibat in biliary atresia (BOLD), and the planned pivotal trial for odevixibat in Alagille syndrome; the target indication(s) for development or approval, the size, design, population, location, conduct, cost, objective, enrollment, duration or endpoints of any clinical trial, or 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 odevixibat in PFIC, the pivotal trial for odevixibat in biliary atresia, the planned pivotal trial for odevixibat in Alagille syndrome; the potential approval and commercialization of odevixibat; discussions with the FDA or EMA regarding our programs; the potential benefits or competitive position of odevixibat, elobixibat, or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential effects of odevixibat of the treatment of PFIC patients and its potential to improve the current standard of care; the potential benefits of an orphan drug designation; the potential issuance of a rare pediatric disease priority review voucher; the period for which Albireo's cash resources will be sufficient to fund its operating requirements (runway); 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," 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: 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 odevixibat to date, including findings in indications other than PFIC, will be predictive of results from other clinical trials of odevixibat; 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 to support approval of odevixibat 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 odevixibat, including the the pivotal program in biliary atresia or the planned pivotal program in Alagille syndrome, 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, or the conduct of, the double-blind Phase 3 trial or other pivotal trials; 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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Videos accompanying this announcement are available at

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A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/31d2662e-d87e-4599-bcd1-05e7b13437da

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