Albireo Provides Mid-Year Clinical Development Update

July 9, 2019

— Expects Odevixibat PEDFIC 1 Phase 3 Topline Data Mid-2020—
— Site Activated for Odevixibat PEDFIC 2 Expanded Open-Label Cohort—
— Odevixibat Biliary Atresia Pivotal Trial Expected to Begin in 2020—
— Moves Into NASH with Initiation of Elobixibat Phase 2 trial —

BOSTON, July 09, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, provided an update on the progress of the development of odevixibat for pediatric cholestatic liver diseases and elobixibat for NASH.

The odevixibat PEDFIC program in progressive familial intrahepatic cholestasis (PFIC) consists of two clinical trials. PEDFIC 1 is a randomized, double-blind, placebo-controlled, global multicenter clinical trial designed to enroll approximately 60 patients with PFIC type 1 or type 2. PEDFIC 2 is an open-label extension study designed to assess long-term safety and durability of response in a cohort of patients rolled over from PEDFIC 1, and it includes a second cohort of PFIC patients who are not eligible for PEDFIC 1.

"We are extremely encouraged with the recruiting efforts by the participating sites in our Phase 3 trial for odevixibat in PFIC, as we have now enrolled over half of the study. We also have a potentially sufficient number of patients currently in screening and prescreening to complete the study, and we expect to have topline data mid-2020. Approximately one third of the recruited patients to-date have been screen failures, which is higher than expected, but importantly, this provides greater homogeneity of the PFIC type 1 or type 2 patients who ultimately enroll in the trial, which preserves the integrity of the study," said Ron Cooper, President and Chief Executive Officer of Albireo. "In addition, we're excited to have screened the first patients in the Phase 2 study with once-daily elobixibat in NAFLD/NASH, as we believe elobixibat has the potential to find a significant place in the emerging NASH treatment landscape."

PEDFIC 1 is a global study with 44 sites actively recruiting. The company expects topline data in mid-2020 and will provide a further update when the study has been fully enrolled.

PEDFIC 2 is an open-label extension study for patients to roll into after completing PEDFIC 1. Many of the patients not randomized into PEDFIC 1 will potentially be eligible for the PEDFIC 2 expanded cohort, which has a broader entry criteria. Patients are given high-dose odevixibat, 120 (µg/kg/day) and will be studied for 18 months. Sites are active for this global trial, which is designed to assess long-term safety and durability of response.

PEDFIC 2 includes another cohort for an expanded range of PFIC patients. Patients are given high-dose odevixibat, 120 (µg/kg/day) and will be studied for 18 months. The first site has been activated.

Odevixibat is being developed for multiple pediatric cholestatic liver diseases, and Albireo announced biliary atresia as the next indication for study. There is no approved treatment for biliary atresia, and the disease is the number one reason for pediatric liver transplants. Productive discussions on the final protocol continue with regulatory agencies, and the expected start of a biliary atresia pivotal trial will be in 2020. Albireo expects the final study design to have a different size, length and endpoint compared to the PFIC program, and the company will provide further updates once the trial protocol and overall timeline for the trial is established.

Bile acid modulation through IBAT inhibition holds promise as a therapeutic approach for the treatment of NASH based on measures of efficacy, safety and convenience. In clinical and nonclinical studies, IBAT inhibitors have demonstrated decreases in serum bile acids, cholesterol, liver inflammation and liver fibrosis, as well as an increase in GLP1— all effects that are of potential benefit in the treatment of NAFLD/NASH. IBAT inhibitors can be given orally once a day, and they have low systemic exposure, reducing the potential for off-target effects and raising the possibility they could be used concomitantly with other NASH or cardiovascular risk medicines.

Albireo's NASH program includes a Phase 2 trial with elobixibat, as well as development of investigational preclinical novel bile acid modulators. The first patients were screened in the elobixibat Phase 2 trial, which will assess the safety and efficacy of elobixibat 5 mg in 46 NASH/NAFLD patients over 16 weeks, as measured by liver steatosis and various biomarkers. Topline data are expected mid-2020.

About Odevixibat
Odevixibat is a product candidate being developed to treat rare pediatric cholestatic liver diseases and is in Phase 3 development in its initial target indication, progressive familial intrahepatic cholestasis (PFIC). A highly potent and selective inhibitor of the ileal bile acid transporter (IBAT), odevixibat has minimal systemic exposure and acts locally in the small intestine.

Odevixibat is being evaluated in a Phase 3 clinical trial, PEDFIC 1, in patients with PFIC subtype 1 or 2 (NCT03566238). The PEDFIC 1 clinical trial is recruiting at more than 40 clinical trial sites worldwide. More information may be found on www.clinicaltrials.gov.

The odevixibat PFIC program has received fast track, rare pediatric disease and orphan drug designation 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 European Medicines Agency (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 Elobixibat

Elobixibat is a first-in-class, once-daily, orally-available ileal bile acid transporter (IBAT) inhibitor currently being evaluated in a Phase 2 clinical trial in nonalcoholic fatty liver disease (NAFLD) and nonalcoholic steatohepatitis (NASH). In clinical studies, elobixibat demonstrated a decrease in LDL/H cholesterol, as well as decreased insulin resistance through an increase in GLP-1. The first IBAT inhibitor approved globally, elobixibat is approved in Japan for the treatment of patients with chronic constipation (excluding constipation caused by organic disease). It is marketed and sold in Japan under the trade name GOOFICE®.

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, odevixibat (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. Elobixibat is in Phase 2 development in NAFLD and NASH. Approved in Japan for the treatment of chronic constipation, elobixibat 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, Mass., and its key operating subsidiary is located in Gothenburg, Sweden. The *Boston Business Journal* named Albireo one of the 2019 Best Places to Work in Massachusetts. 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, duration or results or timing for availability of results of, development of odevixibat, elobixibat or any other Albireo product candidate or program, including regarding the Phase 3 clinical program for odevixibat in patients with PFIC; the planned pivotal trial for odevixibat in biliary atresia, the Phase 2 clinical trial for elobixibat in NAFLD/NASH, the target indication(s) for development, the size, design, population, location, conduct, objective, enrollment, duration or endpoints of any clinical trial, or the timing for initiation or completion of or reporting of results from any clinical trial, including the double-blind Phase 3 PFIC trial for odevixibat, the planned pivotal trial for odevixibat in biliary atresia or the Phase 2 trial for elobixibat in NAFLD/NASH; the potential approval and commercialization of odevixibat; the size of the PFIC population, the biliary atresia population, the NASH population or any other disease population for indications that may be targeted by Albireo; 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 benefits of an orphan drug designation 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: whether favorable findings from clinical trials of odevixibat to date, including findings in indications other than PFIC, will be predictive of results from the trials comprising the Phase 3 PFIC program or any 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, even if the primary endpoint is met with statistical significance, 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 trials comprising the Phase 3 PFIC program, 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; 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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