Albireo Announces FDA Clearance of IND to Commence Phase 2 Trial of Elobixibat for the Treatment of NAFLD/NASH

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– Initiation of Phase 2 clinical trial expected in Q2 2019 –
– IBAT inhibition has potential to impact key markers of NASH–

BOSTON, April 11, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced that its Investigational New Drug application (IND) has cleared the required 30 day review by the U.S. Food and Drug Administration and is in effect for a Phase 2 clinical trial of elobixibat, a first-in-class, once-daily, orally-available ileal bile acid transporter (IBAT) inhibitor, for the treatment of nonalcoholic fatty liver disease (NAFLD) and nonalcoholic steatohepatitis (NASH).

"The IND and subsequent trial initiation are an important step for our NASH program, as we apply our expertise in bile acids and leverage our novel IBAT platform to address a large unmet medical need," said Ron Cooper, President and Chief Executive Officer of Albireo. "We have a two-pronged approach for NASH development: advancing elobixibat, a well-characterized IBAT inhibitor, into a Phase 2 trial and continuing to develop novel preclinical compounds."

NAFLD affects about 25 percent of the world's population and is a major cause of liver disease. NAFLD may progress to NASH, which is characterized by liver inflammation and damage caused by a buildup of fat in the liver. NASH coincides with high obesity rates and diabetes and is projected to become the leading cause of liver transplants in the United States. NASH patients have a 10-fold greater risk of liver-related mortality, compared with the general population. There is currently no approved pharmacologic treatment for NASH.

There is a strong rationale for bile acid modulation as a therapeutic approach in NASH from an efficacy and safety/tolerability perspective. Key markers in NASH patients include elevated bile acid levels, elevated cholesterol, insulin sensitivity, liver inflammation and liver fibrosis. Clinical and preclinical data indicate that IBAT inhibitors may have a positive impact on these parameters. Elobixibat, an innovative IBAT inhibitor approved last year in Japan for chronic idiopathic constipation, has minimal systemic exposure and a safety database with more than 1,500 patient exposures. Elobixibat could have potential in NASH as either monotherapy or combination therapy.

The planned Phase 2 clinical trial is a randomized, double-blinded, placebo-controlled, multicenter trial designed to assess the efficacy and safety of elobixibat in approximately 46 adults with biopsy-confirmed NASH or suspected diagnosis of NAFLD/NASH based on metabolic syndrome definitions. Treated patients will receive 5 mg of elobixibat once daily for 16 weeks. The primary endpoint will be the assessment of change in low-density lipoprotein (LDL) cholesterol, and secondary endpoints include the assessment of change in liver fat by imaging, and alanine transaminase (ALT) and serum bile acids levels. The study should be initiated in the second quarter of 2019, with results planned for mid-2020.

About Elobixibat
The first ileal bile acid transporter (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, 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 (PFIC). 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.

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 elobixibat or any other Albireo product candidate or program; the target indication(s) for development, the size, design, population, location, conduct, objective, 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 Phase 2 trial for elobixibat in NAFLD/NASH; the size of the NASH population, or any other disease population for indications that may be targeted by Albireo; the potential benefits or competitive position of elobixibat, or any other Albireo product candidate or program or the commercial opportunity in any target indication; 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: the timing for initiation or completion of, or for availability of data from, clinical trials, including the Phase 2 trial of elobixibat in NAFLD/NASH, 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, clinical trials, including the Phase 2 clinical trial of elobixibat in NAFLD/NASH; 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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