Albireo Announces Presentations at NASPGHAN 2019 Annual Meeting

September 30, 2019

Presentations highlight data on odevixibat's minimal systemic exposure and Albireo's proprietary pruritus assessment tools

BOSTON, Sept. 30, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced upcoming presentations at the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition (NASPGHAN) 2019 Annual Meeting, being held October 16-19, 2019, in Chicago. The presentations will include pharmacological data regarding the Company's lead product candidate, odevixibat, as well as background about the Company's proprietary patient- and observer-reported outcomes assessment tools for pruritus.

"When considering existing and emerging pediatric therapies, there is a strong desire among physicians, parents and patients for exposure levels to be concentrated in targeted areas of the body while minimizing broader systemic exposure," said Ron Cooper, President and Chief Executive Officer of Albireo. "At NASPGHAN, we will present data demonstrating this to be the case with odevixibat. Additionally, we are looking forward to sharing additional detail about the extensive efforts undertaken to develop the proprietary patient- and observer-reported outcomes tools that are being utilized to measure pruritus and sleep disturbance in our ongoing pivotal Phase 3 trial of odevixibat in progressive familial intrahapetic cholestasis (PFIC)."

Details of the NASPGHAN poster presentations are as follows:

- October 17 from 5-7 p.m. CDT: "Clinical pharmacology of odevixibat, a potent, selective, ileal bile acid transport inhibitor with minimal systemic exposure" – Abstract #167
- October 19 from 12-2:30 p.m. CDT: "Development of patient- and observer-reported outcome (PRO and ObsRO) measures for paediatric cholestatic liver diseases" – Abstract #592

The abstracts were published in the October issue of the *Journal of Pediatric Gastroenterology and Nutrition* and can be viewed here: https://journals.lww.com/jpgn/pages/default.aspx.

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 45 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 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 its initial target indication, progressive familial intrahepatic cholestasis (PFIC). 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 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 potential approval and commercialization of odevixibat; the size of the PFIC population or any other disease population for indications that may be targeted by Albireo; the potential benefits or competitive position of odevixibat, or any other Albireo product candidate or program or the commercial opportunity in any target indication. 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.

Investor Contact: Hans Vitzthum, LifeSci Advisors, LLC, 212-915-2568
Media Contact: Heather Anderson, 6 Degrees, 980-938-0260, handerson@6degreespr.com

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