Albireo Launches Expanded Access Program for Odevixibat for Patients in the U.S., Canada, Australia and Europe

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— Program expands access to odevixibat for patients with progressive familial intrahepatic cholestasis (PFIC), an ultra-rare pediatric cholestatic liver disease —

BOSTON, July 20, 2020 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced the launch of an Expanded Access Program (EAP) for investigational lead product candidate odevixibat, a highly potent and selective inhibitor of the ileal bile acid transporter (IBAT), for patients with progressive familial intrahepatic cholestasis (PFIC) in the U.S., Canada, Australia and Europe.

“A tremendous need remains for a pharmacologic treatment option for PFIC, a life-threatening, ultra-rare, pediatric liver disease with serious and debilitating symptoms. Through our open-label PEDFIC 2 trial, and now our Expanded Access Program (EAP), we hope to increase access to odevixibat for all eligible patients to the greatest extent possible until odevixibat is commercially available,” said Ron Cooper, President and Chief Executive Officer of Albireo. “We are pleased to launch the program in the U.S., Canada, Australia and Europe, and we are working closely with local regulators in other countries to offer access and continue to provide hope for PFIC families worldwide.”

Odevixibat is being evaluated in patients with PFIC in the PEDFIC 1 Phase 3 trial (NCT03566238), for which topline results are expected in mid-2020, and the PEDFIC 2 open-label trial (NCT03659916) evaluating the long-term efficacy and safety of odevixibat in patients with PFIC. The PEDFIC 2 trial is the preferred route of access for all patients who qualify, as determined by their physician. Cohort 1 of the trial allows patients from the PEDFIC 1 clinical trial to continue treatment with odevixibat until it is commercially available. Cohort 2 comprises patients who did not participate in the PEDFIC 1 trial and includes other forms of PFIC not included in PEDFIC 1.

The EAP is available to patients in the U.S., Canada, Australia and Europe but may be limited depending on drug supply. Timing of availability in Europe will vary due to country-specific and local regulations. This program is available for patients with a clinical diagnosis of PFIC who have no other therapeutic options and do not qualify for, or have access to, PEDFIC 2 Cohort 2. The EAP inclusion criteria allow participation by patients with reduced pruritus and serum bile acid levels compared to our existing studies. All forms of PFIC (even episodic forms – e.g., benign recurrent intrahepatic cholestasis, commonly known as BRIC) are allowed, genetic confirmation of PFIC is not required, and patients who have had a prior liver transplant may be eligible for this program, as determined by their physician. For more information on Albireo’s odevixibat Expanded Access Program, visit www.albireopharma.com/patients-families/expanded-access-policy.

If you are a physician in the U.S. or Canada who would like to request EAP access for your patient, contact Odevixibat@clinigengroup.com. If you are a physician in Australia or Europe who would like to request EAP access for your patient, contact medicineaccess@clinigengroup.com.

Our aim is to expand this program to other geographies over time. If you are a physician, patient or caregiver of a patient outside of the U.S., Canada, Australia, or Europe who may be interested in odevixibat therapy access, email medinfo@albireopharma.com with your interest.

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 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 (NCT03566238), as well as a Phase 3 clinical trial, BOLD, 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 U.S. Food and Drug Administration (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 progressive familial intrahepatic cholestasis (PFIC) and biliary atresia, with a third Phase 3 trial being planned in Alagille syndrome. 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 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, elobixibat 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 potential availability of odevixibat through the EAP, whether the FDA continues to allow odevixibat to be administered through the EAP, the pivotal trial for odevixibat in biliary atresia (BOLD), the planned pivotal trial for odevixibat in Alagille syndrome, and the Phase 2 clinical trial for elobixibat in NAFLD/NASH; 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 Phase 3 PFIC trial for odevixibat, and the long-term open-label extension study, the pivotal trial for odevixibat in biliary atresia, the planned pivotal trial for odevixibat in Alagille syndrome, or the Phase 2 trial for elobixibat in NAFLD/NASH; 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 benefits of an orphan drug designation; 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 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, 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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