Albireo Reports Fourth Quarter and Year-End 2019 Financial Results, and Provides Business Update

March 2, 2020

— Phase 3 odevixibat PFIC trial fully enrolled —

— Initiating pivotal trials in biliary atresia first half of 2020 and Alagille syndrome by year end—

— Management to host conference call and webcast today at 10:00 a.m. ET —

BOSTON, March 02, 2020 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today provided a business update and reported financial results for the fourth quarter and year ended December 31, 2019.

“In 2019, we took major steps forward in multiple development programs, advanced commercial preparations and evolved as an organization, laying the groundwork for Albireo to enter the next stage,” said Ron Cooper, President and Chief Executive Officer of Albireo. “We have always believed in odevixibat’s potential across a range of cholestatic liver diseases, and 2020 will be a transformational year, as we expect results from our Phase 3 trial in PFIC, look forward to initiating a pivotal trial in biliary atresia and plan to begin a pivotal trial in Alagille syndrome following discussions with the FDA this quarter. Meanwhile, we eagerly anticipate the results from the elobixibat NASH/NAFLD study and are excited to have selected a lead candidate to advance from our robust library of preclinical compounds.”

Recent Highlights

Odevixibat

- Completed full randomization of the PEDFIC 1 Phase 3 study in progressive familial intrahepatic cholestasis (PFIC). The PEDFIC 1 trial is studying odevixibat in both PFIC type 1 and type 2 patients aged 6 months to 18 years. Patients randomized to odevixibat are being treated with high- (120µg/kg) or low- (40µg/kg) dose once-daily oral capsules or sprinkles. The trial uses Albireo’s planned commercial formulation of odevixibat, which does not require refrigeration. Albireo expects topline results mid-2020, and anticipates a potential first regulatory approval and launch in the second half of 2021.

- Continued enrolling patients in PEDFIC 2, the long-term, open-label extension study of PEDFIC 1. Cohort 1 of PEDFIC 2 is composed of patients who have rolled over from the PEDFIC 1 study. Albireo now has patients who have been treated with odevixibat for more than 1 year. Cohort 2 is expected to broaden the evidence base for odevixibat by including PFIC patients who were ineligible for PEDFIC 1. This includes patients with all types of PFIC, and patients younger than 6 months or older than 18 years of age.

- Received clearance from the U.S. Food and Drug Administration (FDA) for the company’s Investigational New Drug (IND) application to initiate a precedent-setting global pivotal trial in biliary atresia following agreement on a study design. Biliary atresia is a rare pediatric liver disease that is the leading cause of liver transplants among children, and for which there is no approved pharmacological therapy. The double-blind, placebo-controlled trial is designed to enroll approximately 200 patients at 70 sites. Patients will receive either placebo or high-dose (120µg/kg) odevixibat once daily. The primary endpoint is survival with native liver after 2 years of treatment. The FDA and European Commission have granted orphan designations for odevixibat in the treatment of biliary atresia. Albireo plans to initiate the trial in 1H 2020.

- Plans to commence an additional pivotal program in Alagille syndrome by the end of 2020, following scheduled FDA interactions in the first quarter. The FDA and the European Commission have granted orphan designations for odevixibat in the treatment of Alagille syndrome.

- Advanced preparations for commercialization across physician, patient and payer communities. The company initiated activities to develop a detailed understanding of treatment centers, continued to build the PFIC Voices initiative in collaboration with patients and families, and progressed the odevixibat value story and economic models for reimbursement.

Pipeline

- Nearing completion of enrollment in the company’s Phase 2 multicenter, placebo-controlled clinical trial of elobixibat 5mg in non-alcoholic fatty liver disease (NAFLD)/nonalcoholic steatohepatitis (NASH). Albireo expects topline data by mid-2020. In

addition, the company expects data from a second study being conducted in Japan with elobixibat 10mg and in combination with a bile acid sequestrant later this year or early 2021.

- Selected lead preclinical product candidate, filed for intellectual property protection and initiated IND-enabling studies.

Fourth Quarter 2019 Financial Results

- Revenues were $6.4 million for the fourth quarter of 2019, compared to $0.6 million for the fourth quarter of 2018. The year-over-year increase was primarily the result of estimated milestone royalty revenue received from EA Pharma for elobixibat for the treatment of chronic constipation.

- R&D expense was $14.2 million for the fourth quarter of 2019, compared to $9.5 million for the fourth quarter of 2018. The year-over-year change was primarily the result of program expenses for odevixibat and elobixibat, as well as personnel costs, as we continue to increase our program activities and headcount.

- G&A expense was $6.2 million for the fourth quarter of 2019, compared to $5.8 million for the fourth quarter of 2018. The year-over-year change was primarily the result of personnel and related expenses, as we continue to increase our headcount.

- Net loss for the fourth quarter of 2019 was $7.5 million, or $(0.57) per share, compared to $15.9 million, or $(1.34) per share for the fourth quarter of 2018.

Financial Results for the Year Ended December 31, 2019

- Revenues were $9.6 million for the year ended December 31, 2019, compared to $12.7 million for the year ended December 31, 2018. The year-over-year decline is primarily due to a milestone payment received in 2018 from EA Pharma due to the approval by the Japanese MHLW of elobixibat for the treatment of chronic constipation offset by royalty revenue from EA Pharma.

- R&D expense was $45.6 million for 2019 up from $31.7 million for the same period in 2018. The year-over-year increase was primarily the result of program expenses for odevixibat, elobixibat and preclinical work, as well as personnel costs, as we continue to increase our program activities and headcount.

- G&A expense was $23.0 million for 2019, compared to $18.1 million for the previous year. The year-over-year increase was primarily the result of personnel and stock-based compensation, as we continue to increase our headcount.

- Net loss for the year ended December 31, 2019, was $62.7 million, or $(5.04) per share, compared to $46.1 million, or $(3.94) per share for the year ended December 31, 2018.

- The company had cash and cash equivalents at December 31, 2019, of $131.8 million, which compares to $163.9 million at December 31, 2018.

Financial Guidance
For the full year 2020, the company anticipates total expenses, including R&D and G&A expenses, to be around $100M. Following a February 2020 common stock offering of $43.2 million, Albireo now anticipates that its current cash balance will be sufficient to meet its operating needs into the second half 2021.

Conference Call
As previously announced, Albireo will host a conference call and webcast today, March 2, 2020, at 10:00 a.m. ET. To access the live conference call by phone, dial 877-407-0792 (domestic) or 201-689-8263 (international), and provide the access code 13697430. A live audio webcast will be accessible from the Media & Investors page of Albireo's website, 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 on the Events & Presentations section of the Media & Investors page of Albireo's website for 3 months following the event.

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.

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.

The odevixibat PFIC program has 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.

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 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, and the long-term open-label extension study, the planned pivotal trials for odevixibat in biliary atresia and Alagille syndrome, or the Phase 2 trial for elobixibat in NAFLD/NASH; the potential approval and commercialization of odevixibat; discussions with the FDA 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 pricing of odevixibat if approved; 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: 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, or the planned pivotal programs in biliary atresia and Alagille syndrome, and the outcomes of such trials; Albireo’s completion of discussions with the FDA regarding the planned pivotal trial for odevixibat in Alagille syndrome; 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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Albireo Pharma, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(unaudited)

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 131,843	$ 163,885
Prepaid expenses and other current assets	9,956	3,765
Total current assets	141,799	167,650
Property and equipment, net	597	187
Goodwill	17,260	17,260
Other assets	5,413	369
Total assets	$ 165,069	$ 185,466
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,785	$ 4,352
Accrued expenses	13,486	8,165
Other current liabilities	653	308
Total current liabilities	18,924	12,825

Liability related to sale of future royalties	48,714	49,969
Other long-term liabilities	4,270	35
Total liabilities	71,908	62,829
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at December 31, 2019 and December 31, 2018; 12,749,443 and 11,969,928 issued and outstanding at December 31, 2019 and December 31, 2018, respectively	127	120
Additional paid-in capital	245,769	214,694
Accumulated other comprehensive income	6,452	4,293
Accumulated deficit	(159,187)	(96,470)
Total stockholders' equity	93,161	122,637
Total liabilities and stockholders' equity	$ 165,069	$ 185,466

Albireo Pharma, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2019	**2018**	**2019**	**2018**
Revenue	$ 6,431	$ 571	$ 9,636	$ 12,740
Operating expenses:				
Research and development	14,216	9,504	45,575	31,732
General and administrative	6,175	5,845	22,963	18,061
Other operating expense (income), net	(4,109)	(540)	2,210	837
Total operating expenses	16,282	14,809	70,748	50,630
Operating loss	(9,851)	(14,238)	(61,112)	(37,890)
Interest expense, net	(1,362)	(789)	(5,296)	(4,838)
Non-operating income (expense), net	3,691	(817)	3,691	(3,363)
Net loss before income taxes	(7,522)	(15,844)	(62,717)	(46,091)
Income tax	—	20	—	20
Net loss	$ (7,522)	$ (15,864)	$ (62,717)	$ (46,111)
Net loss per share attributable to holders of common stock:				
Net loss per common share - basic and diluted	$ (0.57)	$ (1.34)	$ (5.04)	$ (3.94)
Weighted-average shares outstanding:	12,698,492	11,969,928	12,437,742	11,702,785

Source: Albireo Pharma, Inc.