Albireo Reports Third Quarter 2019 Financial Results and Provides Business Update

November 6, 2019

—Phase 3 PFIC trial randomization more than three-quarters complete. —
— Topline results expected mid-2020.—
— Pivotal trial in biliary atresia expected to be initiated in 2020. —
— Management to host conference call and webcast today at 10 a.m. ET. —

BOSTON, Nov. 06, 2019 (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 third quarter ended September 30, 2019.

“Randomization of patients in our Phase 3 trial of odevixibat in PFIC is nearing completion, and we remain on track to report topline results in mid-2020,” said Ron Cooper, President and Chief Executive Officer of Albireo. “Odevixibat is being developed for a range of cholestatic liver diseases, and Albireo is finalizing discussions with regulators for a pivotal program in biliary atresia, one of the most common rare pediatric liver diseases. We expect to initiate the world’s first pivotal trial in this indication during 2020.”

Recent Highlights

Odevixibat

- Over three-quarters of the patients needed to complete the PEDFIC 1 Phase 3 study in progressive familial intrahepatic cholestasis (PFIC) had been randomized as of end of October 2019. Additionally, the patients in screening or entering screening should be sufficient to meet the trial’s 60-patient target based on the screening success rate to date. PEDFIC 1 is enrolling both PFIC type 1 and type 2 patients that are 6 months to 18 years of age. The trial is studying both high- and low-dose odevixibat using the company’s planned commercial formulation. Patients randomized to odevixibat are treated with once-daily oral capsules or sprinkles, which do not require refrigeration. Albireo expects to report topline results in mid-2020, and continues to project a potential first regulatory approval and launch in 2021.

- Enrolled the first patients in the second cohort in PEDFIC 2, the long-term, open-label extension study of PEDFIC 1. The expanded cohort is expected to broaden the evidence base for odevixibat by including PFIC patients who do not meet eligibility criteria for PEDFIC 1, but have elevated serum bile acid levels and pruritus. This includes patients with all types of PFIC, and patients younger than 6 months or older than 18 years of age. Cohort 1 of PEDFIC 2 is composed of patients who have completed, and rolled over from, the PEDFIC 1 study. We now have patients who are approaching a year on odevixibat treatment.

- Presented data on odevixibat at the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition Annual Meeting in Chicago. The presentations included data regarding the clinical pharmacology of odevixibat, as well as information about the development of the proprietary patient- and observer-reported outcome (PRO and ObsRO) tools used in the PEDFIC program.

- Completing dialogue with the U.S. Food and Drug Administration (FDA) regarding key parameters for a planned pivotal clinical trial of odevixibat in biliary atresia, one of the most common rare pediatric liver diseases. Albireo plans to initiate this trial in 2020.

Elobixibat

- Randomized the first patients in the company’s Phase 2 multicenter, placebo-controlled clinical trial of elobixibat in non-alcoholic fatty liver disease (NAFLD)/nonalcoholic steatohepatitis (NASH). This trial, which has all 10 initial clinical trial sites active, is designed to randomize 46 patients with biopsy-confirmed NASH, or a diagnosis of suspected NAFLD or NASH based on metabolic syndrome definitions.

Corporate

- Strengthened the company’s leadership team with the appointment of Michelle Graham as Chief Human Resources Officer.

Third-Quarter Financial Results

- Revenues were $1.4 million for the third quarter of 2019, compared to $0.2 million for the third quarter of 2018. The

year-over-year change was primarily the result of estimated royalty revenue received from EA Pharma for elobixibat for the treatment of chronic constipation.

- R&D expense was $12.0 million for the third quarter of 2019, compared to $9.7 million for the third quarter of 2018. The year-over-year change was primarily the result of personnel, and program expenses as we continue to increase our headcount, and program activities, respectively.

- G&A expense was $6.0 million for the third quarter of 2019, compared to $3.9 million for the third 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 third quarter of 2019 was $21.9 million, or $(1.73) per share, compared to $14.0 million, or $(1.17) per share for the third quarter of 2018.

- The company had cash and cash equivalents at September 30, 2019, of $142.7 million, which compares to $163.9 million at December 31, 2018.

Financial Guidance
For the full year 2019, the company anticipates total expenses, including R&D and G&A expenses, to be in the range of $70M-$75M. Albireo continues to anticipate that its current cash balance will be sufficient to meet its operating needs into 2021.

Conference Call
As previously announced, Albireo will host a conference call and webcast today, November 6, 2019, at 10 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 13694376. 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.

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, and the long-term open-label extension study, 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; 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, and the outcomes of such trials; Albireo's completion of discussions with the FDA regarding the planned pivotal trial for odevixibat in biliary atresia; 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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Albireo Pharma, Inc.

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

(unaudited)

	September 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 142,666	$ 163,885
Prepaid expenses and other current assets	5,353	3,765
Total current assets	148,019	167,650
Property and equipment, net	633	187
Goodwill	17,260	17,260
Other assets	5,578	369
Total assets	$ 171,490	$ 185,466
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,185	$ 4,352
Accrued expenses	8,459	8,165
Other current liabilities	683	308
Total current liabilities	12,327	12,825
Liability related to sale of future royalties	53,073	49,969
Other long-term liabilities	4,418	35
Total liabilities	69,818	62,829
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at September 30, 2019 and December 31, 2018; 12,685,326 and 11,969,928 issued and outstanding at September 30, 2019 and December 31, 2018	126	120
Additional paid in capital	242,638	214,694
Accumulated other comprehensive income	10,573	4,293
Accumulated deficit	(151,665)	(96,470)
Total stockholders' equity	101,672	122,637
Total liabilities and stockholders' equity	$ 171,490	$ 185,466

Albireo Pharma, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except share and per share data)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenue	$ 1,385	$ 237	$ 3,205	$ 12,169
Operating expenses:				
Research and development	11,996	9,666	31,359	22,228
General and administrative	6,010	3,850	16,788	12,216
Other operating expense (income), net	4,015	(614)	6,319	1,377
Total operating expenses	22,021	12,902	54,466	35,821

Operating loss	(20,636)	(12,665)	(51,261)	(23,652)
Interest expense, net	(1,274)	(1,367)	(3,934)	(4,049)
Non-operating income (expense), net	—	7	—	(2,546)
Net loss	$ (21,910)	$ (14,025)	$ (55,195)	$ (30,247)
Net loss per common share - basic and diluted	$ (1.73)	$ (1.17)	$ (4.47)	$ (2.60)
Weighted-average common shares used to compute basic and diluted net loss per common share	12,685,000	11,969,791	12,349,870	11,612,760

Source: Albireo Pharma, Inc.