Albireo Reports Second Quarter 2019 Financial Results

August 8, 2019

— Odevixibat Phase 3 PEDFIC 1 topline results expected mid-2020—
— Initiated elobixibat Phase 2 trial in NAFLD/NASH—
— Management to host conference call and webcast today at 10 a.m. EDT —

BOSTON, Aug. 08, 2019 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage orphan pediatric liver disease company developing novel bile acid modulators, today announced financial results and corporate highlights for the second quarter ended June 30, 2019.

“The momentum is building for Albireo, as we advance programs on multiple fronts and ramp up commercial preparations,” said Ron Cooper, President and Chief Executive Officer of Albireo. “While the PEDFIC program moves toward full enrollment, and we plan for potential approval and launch in 2021, we continue to strengthen the body of evidence supporting the potential of odevixibat, and we are preparing to initiate a pivotal study in biliary atresia. At the same time, we are very excited to have initiated an elobixibat Phase 2 trial in NAFLD/NASH, and we believe elobixibat has the potential to find a significant place in the emerging NASH treatment landscape.”

Recent Highlights

Odevixibat (A4250)

- Achieved full trial site activation in the PEDFIC 1 Phase 3 study in progressive familial intrahepatic cholestasis (PFIC), with 44 active sites globally, and enrollment progressing steadily. Patients in screening or entering screening are potentially sufficient to complete the trial based on the current screening success rate. PEDFIC 1 is enrolling both PFIC1 and PFIC2 patients both with and without diversion surgery. The trial is studying both high- and low-dose odevixibat using the planned commercial formulation. Patients randomized to odevixibat are treated with once-daily oral capsules or sprinkles, which do not require refrigeration. Albireo expects topline results in mid-2020, and continues to project potential first regulatory approval and launch in 2021.

- Activated first site and prepared for patient enrollment in expanded second cohort in PEDFIC 2, the long-term, open-label extension study of PEDFIC 1. Cohort 2 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.

- Presented data on odevixibat and rare cholestatic liver disease that affirm the potential of Albireo’s development programs at the 52ndEuropean Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN) Annual Meeting in Glasgow, Scotland. Results from the Phase 2 study in pediatric cholestasis were presented on the effect of odevixibat on reducing serum bile acids, and improving pruritus and sleep measures in a subset of patients with Alagille syndrome, as well as the effect of odevixibat on reducing serum bile acids and improving pruritus in a small subset of patients with biliary atresia. A case study compared the effect of odevixibat on serum bile acids and pruritus in a patient who subsequently underwent biliary diversion surgery. Data on the development of the proprietary patient- and observer-reported outcome (PRO and ObsRO) tools used in the PEDFIC program also were presented.

- Launched *PFIC Voices*, the first-ever PFIC disease awareness initiative, in collaboration with parents, patients, the patient advocacy community and physicians (pficvoices.com). PFIC Voices is a global initiative to build awareness and understanding of PFIC by sharing perspectives about the impact of PFIC, and the critical need for education, treatment and support.

- Prepared to begin an odevixibat pivotal trial in biliary atresia, estimated to be one of the most common rare pediatric liver diseases. Productive discussions are underway with regulators, and the Company plans to initiate the trial in 2020.

Elobixibat

Initiated a Phase 2 multicenter, placebo-controlled clinical trial of elobixibat in non-alcoholic fatty liver disease (NAFLD)/nonalcoholic steatohepatitis (NASH) designed to enroll 46 patients with biopsy-confirmed NASH, or a diagnosis of suspected NAFLD or NASH based on metabolic syndrome definitions. The Company expects to report topline data in mid-2020.

Corporate

- Presented at several conferences, including: the Jefferies 2019 Global Healthcare Conference in New York and the William Blair 39th Annual Growth Stock Conference in Chicago.

Second-Quarter Financial Highlights

- Revenues were $1.3 million in the second quarter of 2019, compared to $0.7 million in the second quarter of 2018.
- R&D expense was $11.0 million for the second quarter of 2019, up 72% from $6.4 million in the second quarter of 2018.
- G&A expense was $5.5 million for the second quarter of 2019, up 29%, compared to $4.2 million in the second quarter of 2018.
- Net Loss in the second quarter of 2019 was $16.6 million, or $(1.35) per share, compared to $14.6 million, or $(1.22) per share in the second quarter of 2018.
- The Company had cash and cash equivalents at June 30, 2019, of $157.7 million.

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 $75-$80 million. Albireo anticipates its current cash balance to be sufficient to meet its operating needs into 2021.

Conference Call
As previously announced, Albireo will host a conference call and webcast today, August 8, 2019, at 10 a.m. EDT. To access the live conference call by phone, dial 877-407-0792 (domestic) or 201-689-8263 (international), and provide the access code 13691544. 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 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, odevixibat (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. 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, 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; the size of the PFIC population, the biliary atresia population, the NASH population or any other disease population for indications that may be targeted by Albireo; 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 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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Source: Albireo Pharma, Inc.

Albireo Pharma, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(unaudited)

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 157,722	$ 163,885
Prepaid expenses and other current assets	5,310	3,765
Total current assets	163,032	167,650
Property and equipment, net	544	187
Goodwill	17,260	17,260
Other assets	1,115	369
Total assets	$ 181,951	$ 185,466
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payables	$ 3,837	$ 4,352
Accrued expenses	7,555	8,165
Other current liabilities	393	308
Total current liabilities	11,785	12,825
Liability related to sale of future royalties	52,224	49,969
Other long-term liabilities	255	35
Total liabilities	64,264	62,829
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at June 30, 2019 and December 31, 2018; 12,685,326 and 11,969,928 issued and outstanding at June 30, 2019 and December 31, 2018	126	120
Additional paid in capital	240,734	214,694
Accumulated other comprehensive income	6,582	4,293
Accumulated deficit	(129,755)	(96,470)
Total stockholders' equity	117,687	122,637
Total liabilities and stockholders' equity	$ 181,951	$ 185,466

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenue	$ 1,250	$ 730	$ 1,820	$ 11,932
Operating expenses:				
Research and development	11,034	6,411	19,363	12,562
General and administrative	5,485	4,238	10,778	8,366
Other operating expense, net	8	487	2,304	1,991
Total operating expenses	16,527	11,136	32,445	22,919
Operating loss	(15,277)	(10,406)	(30,625)	(10,987)
Interest expense, net	(1,351)	(1,666)	(2,660)	(2,682)
Non-operating expense, net	—	(2,531)	—	(2,553)
Net loss	$ (16,628)	$ (14,603)	$ (33,285)	$ (16,222)
Net loss per share attributable to holders of common stock:				
Net loss per common share - basic and diluted	$ (1.35)	$ (1.22)	$ (2.73)	$ (1.42)

Weighted-average common shares used to compute basic and diluted net loss per common share	12,355,969	11,938,357	12,178,376	11,417,463

Source: Albireo Pharma, Inc.