Albireo Reports First Quarter 2019 Financial Results

May 9, 2019

Odevixibat Phase 3 PEDFIC 1 PFIC trial continues to enroll as planned
Initiation of additional cohort of PFIC patients in PEDFIC 2 extension trial announced
Odevixibat pivotal trial in biliary atresia on track to begin H2 2019
IND cleared to initiate elobixibat Phase 2 trial in NASH Q2 2019
Management to host conference call and webcast today at 10 a.m. EDT

BOSTON, May 09, 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 first quarter ended March 31, 2019.

“For Albireo, 2019 is focused on advancing toward potential approval and launch of odevixibat in its first indication, while further developing odevixibat into a pediatric cholestasis medicine that could benefit people across multiple rare diseases,” said Ron Cooper, President and Chief Executive Officer of Albireo. “In the first quarter, we continued to make significant progress on both fronts. While we anticipate PEDFIC 1 topline results at the end of this year or early 2020, we are pleased to expand the PEDFIC program to generate data on additional patient types. We also are extremely excited to be preparing for a pivotal trial with odevixibat in biliary atresia, building on promising results from our Phase 2 trial. In addition, our NASH program is gaining momentum, as we prepare to begin a Phase 2 trial in NAFLD/NASH with elobixibat this quarter.”

Recent Highlights

Odevixibat (A4250)

- Achieved 43 trial sites activated in the U.S., Europe and other territories for the PEDFIC 1 Phase 3 study in PFIC as of May 1. PEDFIC 1 is enrolling PFIC subtypes 1 and 2. Expect topline trial results in late 2019 or early 2020, and plan to refine guidance on full enrollment of the study.

- Data in a wider range of PFIC patient types will be generated to support odevixibat in a planned additional patient cohort of PEDFIC 2, the long-term, open-label extension study of PEDFIC 1. The additional cohort will include PFIC patients who are not eligible for PEDFIC 1, including additional PFIC types, adults and neonates. We anticipate initiation of this additional cohort in the second half of this year.

- Held scientific advice meeting with the European Medicines Agency (EMA), following meeting with the Food and Drug Administration (FDA), on design of odevixibat pivotal trial in biliary atresia. Plan to initiate trial in H2 2019.

- Granted orphan drug designation from the FDA for odevixibat for treatment of biliary atresia, estimated to be one of the larger rare pediatric liver diseases, with extremely high unmet need.

- Presented data from our Phase 2 trial in pediatric cholestasis that support the potential of odevixibat in biliary atresia and Alagille syndrome at the European Association for the Study of the Liver (EASL) annual conference held in April in Vienna, Austria. The Alagille data were selected for inclusion in “The Best of ILC,” which EASL prepared to highlight the most noteworthy contributions to the scientific program at the conference this year.

- Also at EASL, the academic consortium NAPPED (NAtural course and Prognosis of PFIC and Effect of biliary Diversion), which has the world’s largest PFIC database, presented data on the natural history of PFIC. Notably, the NAPPED data showed that surgical biliary diversion improved survival in PFIC2 patients and that lowering serum bile acids to 118 µmol/L or a reduction of 70% was associated with favorable native liver survival outcomes. Albireo is one of the sponsors of NAPPED through an unrestricted grant.

- Odevixibat published in *WHO Drug Information* as the recommended international name for A4250.

Elobixibat

- Submitted an IND and was cleared by FDA to begin a Phase 2 multicenter, placebo-controlled trial of elobixibat in NAFLD/NASH.

Corporate

- Presented at several conferences, including: the 39th Annual Cowen Health Care conference in Boston, the Roth conferences in California and New York, the H.C. Wainwright conference in London and the Needham Healthcare conference in New York.

- Strengthened the Company's commercial team with the appointment of Pamela Stephenson as Chief Commercial Officer.

First-Quarter Financial Highlights

- Revenues were $570 thousand in the first quarter of 2019, compared to $11.2 million in the first quarter of 2018.

- R&D expense was $8.3 million for the first quarter of 2019, up 35.4% from $6.2 million in the first quarter of 2018.

- G&A expense was $5.3 million for the first quarter of 2019, up 28.2% compared to $4.1 million in the first quarter of 2018.

- Net Loss in the first quarter of 2019 was $16.7 million, or $(1.39) per share, compared to $1.6 million, or $(0.15) per share in the first quarter of 2018.

- The Company had cash and cash equivalents at March 31, 2019, of $150.3 million.

Financial Guidance
For the full year 2019, we anticipate total expenses, including R&D and G&A expenses, to be in the range of $75-$80 million. We anticipate our current cash balance to be sufficient to meet our operating needs into 2021.

Conference Call
As previously announced, Albireo will host a conference call and webcast today, May 9, 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 13688060. 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 in the Events & Presentations section of the Media & Investors page of Albireo's website for 1 year 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 (PFIC). Albireo's clinical pipeline also includes two Phase 2 product candidates. Albireo's elobixibat, approved in Japan for the treatment of chronic constipation, 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, Massachusetts, 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 planned 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 planned 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)

	March 31, 2019	December 31, 2018
ASSETS		
Current assets:		
Cash and cash equivalents	$ 150,339	$ 163,885
Prepaid expenses and other assets	1,194	850
Other receivables	2,803	2,915
Total current assets	154,336	167,650
Property and equipment, net	173	187
Goodwill	17,260	17,260
Other noncurrent assets	1,209	369
Total assets	$ 172,978	$ 185,466
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Trade payables	$ 2,987	$ 4,352
Accrued expenses	6,385	8,165
Other liabilities	569	308
Total current liabilities	9,941	12,825
Liability related to sale of future royalties	51,433	49,969
Long-term liabilities	213	35
Total liabilities	61,587	62,829
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at March 31, 2019 and December 31, 2018; 12,038,836 and 11,969,928 issued and outstanding at March 31, 2019 December 31, 2018	120	120
Additional paid in capital	217,807	214,694
Accumulated other comprehensive income	6,591	4,293
Accumulated deficit	(113,127)	(96,470)
Total stockholders' equity	111,391	122,637
Total liabilities and stockholders' equity	$ 172,978	$ 185,466

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

	Three Months Ended March 31,	
	2019	2018
Revenue	$ 570	$ 11,202
Operating expenses:		
Research and development	8,329	6,151
General and administrative	5,293	4,128
Other operating (income) expense, net	2,296	1,504
Total operating expenses	15,918	11,783

Operating loss	(15,348)	(581)
Interest income (expense), net	(1,309)	(1,016)
Non-operating income (expense), net	—	(22)
Net loss before income taxes	(16,657)	(1,619)
Income tax	—	—
Net loss	$ (16,657)	$ (1,619)
Net loss per share attributable to holders of common stock:		
Net loss per share - basic and diluted	$ (1.39)	$ (0.15)
Weighted-average shares outstanding:		
Weighted average shares outstanding - basic and diluted	12,001,125	10,896,575

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Source: Albireo Pharma, Inc.