Albireo Reports Third Quarter 2018 Financial Results

November 13, 2018

Odevixibat selected as International Nonproprietary Name (INN) for A4250

A4250 granted Fast Track and additional Orphan Drug designations

A4250 Phase 3 trial progressing as planned, new natural history data presented at AASLD

Management to host conference call and webcast today at 8:30 a.m. EST

BOSTON, Nov. 13, 2018 (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 third quarter ended September 30, 2018.

“With our Phase 3 clinical trial of A4250 for progressive familial intrahepatic cholestasis (PFIC) progressing according to our plan, we focused in Q3 on driving Phase 3 enrollment, deepening our understanding of cholestatic liver diseases and achieving key regulatory milestones on the way to bringing the first approved pharmacological treatment for PFIC and other cholestatic liver diseases to patients,” said Ron Cooper, President and Chief Executive Officer of Albireo.

Recent Highlights

A4250

- *Odevixibat* selected as the proposed International Nonproprietary Name (INN) for A4250. Published by the World Health Organization and currently in a 4-month objection period. In addition, the Food and Drug Administration (FDA) has conditionally accepted Albireo’s proposed proprietary brand name for *odevixibat* (A4250).

- Received Fast Track designation from the FDA for A4250 in the treatment of pruritus associated with PFIC. Fast Track designation is intended to facilitate the development and expedite the review of drugs for serious and life-threatening conditions.

- Received Orphan Drug Designation from the FDA for the treatment of Alagille syndrome, a rare and life-threatening disease that affects the liver and has no approved pharmacologic treatment option. Orphan Drug Designation provides 7 years of market exclusivity upon regulatory approval.

- Agreed with the FDA on a CMC plan for registration.

- Achieved 26 sites activated in 11 countries as of the end of October in the PEDFIC1 Phase 3 pivotal study for A4250, including the first site in the United States. PEDFIC1 is a randomized, double-blind, placebo-controlled Phase 3 clinical trial of A4250 in patients ages 6 months to 18 years with PFIC (subtype 1 or 2) who have elevated serum bile acid levels and pruritus. Data from this study are expected to support drug approval applications for A4250 in the U.S. and E.U. Top-line data are expected in late 2019 or early 2020.

- At the AASLD Meeting (American Association for the Study of Liver Diseases®, new data presented by the NAPPED (NAtural course and Prognosis of PFIC and Effect of biliary Diversion) Consortium supported the impact of serum bile acid reduction and native liver survival rates across PFIC types. These findings build on the initial results reported earlier this year at the European Association for the Study of Liver (EASL) and the European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN), which demonstrated that surgical biliary diversion significantly improves native liver survival in children with PFIC2, providing further clinical support for the rationale for the IBAT inhibition mechanism of A4250 in PFIC.

  Also at the AASLD, the Childhood Liver Disease Research Network (ChiLDReN) presented data on the Steroids in Biliary Atresia Randomized Trial (START), showing the prognostic value of serum bile acids after Kasai portoenterostomy in biliary atresia.

- Presented results from two studies on cholestatic liver diseases at the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition (NASPGHAN) 2018 Annual Meeting. Findings illustrated the significant impact

that pediatric cholestatic liver diseases, such as PFIC, have on quality of life, and support the utility of Albireo's proprietary Patient Reported Outcome (PRO) and Observer Reported Outcome (ObsRO) tools to measure pruritus in the PEDFIC1 study. A systematic literature review also demonstrated that changes in serum bile acids and bilirubin appear useful as biomarkers in predicting both early and long-term outcomes in patients with PFIC. Serum bile acid responder rate is the primary endpoint in the E.U. and a key secondary endpoint in the U.S. in the PEDFIC1 study.

Corporate

- Appointed Simon Harford as Chief Financial Officer, and added other key roles to build our capabilities in preclinical, biostatistics, regulatory, finance and accounting.

- Participated in: Wedbush PacGrow Healthcare Conference, H.C. Wainwright Global Investment Conference, Cantor Fitzgerald Global Healthcare Conference and the Roth NASH Conference.

Financial Update for the Three and Nine Month Periods Ended September 30, 2018

Three Months Ended September 30, 2018 vs. 2017

- Revenues were $237 thousand in the third quarter of 2018.
- R&D expense was $9.7 million, up 200% from $3.2 million in the third quarter of 2017.
- G&A expense was $3.9 million, up 3.8% compared to $3.7 million in the third quarter of 2017.
- Net Loss was $14.0 million, or $(1.17) per share, compared to $6.5 million, or $(0.73) per share in the third quarter of 2017.
- The Company had cash and cash equivalents at September 30, 2018 of $173.6 million, compared to $53.2 million at December 31, 2017.

Nine Months Ended September 30, 2018 vs. 2017

- Revenues were $12.2 million in the nine months ending September 30, 2018.
- R&D expense was $22.2 million, up 147% compared to $9.0 million in the nine months ending September 30, 2017.
- G&A expense was $12.2 million, up 14.9% compared to $10.6 million in the same period of 2017.
- Net Loss was $30.2 million, or $(2.60) per share, compared to $19.4 million, or $(2.60) per share in the same period of 2017.

Financial Guidance
For the full year 2018, we anticipate total expenses, including R&D and G&A expenses, to be at the high end of the range of $45-$50 million. In terms of cash, we expect 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, November 13, 2018, at 8:30 a.m. EST. To access the live conference call by phone, dial 877-407-0792 (domestic) or 201-689-8263 (international) and provide the access code 13683207. 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 users 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 at least 2 weeks 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, 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. 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 A4250 or any other Albireo product candidate or program, including regarding the Phase 3 clinical program for A4250 in patients with PFIC; the target indication(s) for development, the size, design, population, location, conduct, objective, 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 A4250; the size of the PFIC population or any other disease population for indications that may be targeted by Albireo; the potential benefits or competitive position of A4250, or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential benefits of a rare pediatric disease designation, the potential benefits of a fast track designation, the pricing of A4250 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 A4250 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 A4250; 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 A4250 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 A4250, 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.

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

	September 30, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 173,602	$ 53,231
Prepaid expenses and other assets	490	1,054
Other receivables	2,304	726
Total current assets	176,396	55,011
Property and equipment, net	199	178
Goodwill	17,260	17,260
Other noncurrent assets	396	775
Total assets	$ 194,251	$ 73,224
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Trade payables	$ 3,481	$ 1,350
Accrued expenses	5,278	6,105
Other liabilities	327	474
Total current liabilities	9,086	7,929
Liability related to sale of future royalties	48,461	—
Long-term liabilities	37	42
Total liabilities	57,584	7,971
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at September 30, 2018 and December 31, 2017; 11,969,928 and 8,902,784 issued and outstanding at September 30, 2018 and December 31, 2017, respectively	120	89
Additional paid in capital	213,005	114,522
Accumulated other comprehensive income	4,150	1,001
Accumulated deficit	(80,608)	(50,359)
Total stockholders' equity	136,667	65,253
Total liabilities and stockholders' equity	$ 194,251	$ 73,224

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue	$ 237	$ —	$ 12,169	$ 2
Operating expenses:				
Research and development	9,666	3,226	22,228	9,000

General and administrative	3,850	3,709	12,216	10,634
Other operating (income) expense, net	(614)	(401)	1,377	(392)
Total operating expenses	12,902	6,534	35,821	19,242
Operating loss	(12,665)	(6,534)	(23,652)	(19,240)
Interest income (expense), net	(1,367)	23	(4,049)	(378)
Other income (expense), net	7	—	(2,546)	260
Net loss before income taxes	(14,025)	(6,511)	(30,247)	(19,358)
Income tax		—		—
Net loss	$ (14,025)	$ (6,511)	$ (30,247)	$ (19,358)
Net loss per share attributable to holders of common stock:				
Net loss per share - basic and diluted	$ (1.17)	$ (0.73)	$ (2.60)	$ (2.60)
Weighted average shares outstanding - basic and diluted	11,969,791	8,878,430	11,612,760	7,452,709

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