Albireo Reports Year-End 2018 Financial Results and Provides Pipeline Update

March 7, 2019

- A4250 Phase 3 PFIC pivotal program on target for end of '19/early '20 topline readout -
- Plan to initiate second A4250 pivotal program in biliary atresia -
- Plan to initiate Phase 2 study with elobixibat in NASH -
- Management to host conference call and webcast today at 8:30 a.m. EST -

BOSTON, March 07, 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 year ended December 31, 2018, and provided a pipeline update.

"In 2018, Albireo successfully delivered according to plan," said Ron Cooper, President and Chief Executive Officer of Albireo. "The year was highlighted by the start of the A4250 PEDFIC 1 Phase 3 study in PFIC and the approval of elobixibat in Japan for chronic constipation, which made elobixibat the first IBAT inhibitor approved in the world and validated our bile acid scientific platform.

"As positive as last year was, we anticipate 2019 will be truly transformative, with the potential for Phase 3 results in PFIC, initiation of a second rare pediatric cholestatic liver disease pivotal program, and a move into nonalcoholic steatohepatitis (NASH) with an elobixibat Phase 2 trial initiation. Our team is energized for the year ahead."

Recent Highlights

- Achieved trial site activation target for the A4250 PEDFIC 1 Phase 3 pivotal study. We currently have 37 trial sites active in the U.S., Europe and ROW as of February 28, 2019, and we continue to expect topline trial results end of 2019 or early 2020.

- Received multiple key regulatory designations that support our development program across indications, including fast track designation from the U.S. Food and Drug Administration (FDA) for A4250 in the treatment of pruritus in PFIC, orphan drug designation from the FDA for A4250 in Alagille syndrome, and orphan designation from both the FDA and the European Commission for A4250 in biliary atresia.

- Announced the presentation of important findings in PFIC and other cholestatic liver diseases at NASPGHAN and AASLD, illustrating the unmet need in PFIC and the potential impact of A4250. Results presented at NASPGHAN demonstrate that lower serum bile acids and bilirubin after partial external biliary diversion (PEBD) surgery are associated with decreased aggregate need for liver transplant in PFIC patients. Findings presented by the NAPPED Consortium at AASLD The Liver Meeting demonstrate the association of native liver survival rates and serum bile acid levels.

- Presented at the William Blair Biotech Focus Day event "Bay Area Innovation" and the Jefferies 2018 London Healthcare Conference.

- Strengthened senior management team with the appointment of Simon Harford as Chief Financial Officer and Treasurer, Patrick Horn as Chief Medical Officer and Jason Duncan as General Counsel.

Pipeline Update

- Planning for PEDFIC 1 topline results end of 2019 or beginning of 2020.

- Planning a major step toward realizing the larger opportunity for A4250. Preparing to initiate a pivotal trial in biliary atresia with A4250 in the second half of 2019. Biliary atresia may be the largest of the rare pediatric liver diseases of primary initial interest for A4250. Have received orphan designations for A4250 in biliary atresia in U.S. and EU, and engaged regulators on trial design. We continue to see potential for A4250 beyond PFIC and biliary atresia, and are evaluating other indications.

- Planning a significant development effort in NASH. Preparing to initiate a Phase 2 trial with elobixibat in Q2 and continue to progress preclinical novel bile acid modulators for NASH, all of which are potential candidates for partnering in the future.

Financial Update for the Year Ended December 31, 2018

- Revenues were $12.7 million for the year ended December 31, 2018
- R&D expense was $31.7 million, up 144.3% compared to $13.0 million for the year ended December 31, 2017.
- G&A expense was $18.1 million, up 18.5% compared to $15.2 million in the same period of 2017.
- Net Loss was $46.1 million, or $(3.94) per share, compared to $24.4 million, or $(3.12) per share for the year ended December 31, 2017.
- The Company had cash and cash equivalents at December 31, 2018 of $163.9 million, compared to $53.2 million at December 31, 2017.

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 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, March 7, 2019, 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 13685932. 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, 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 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 an orphan drug 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.

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Albireo Pharma, Inc.

Consolidated Balance Sheets
(in thousands, except share and per share data)

	December 31, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 163,885	$ 53,231
Prepaid expenses and other assets	850	1,054
Other receivables	2,915	726
Total current assets	167,650	55,011
Property and equipment, net	187	178
Goodwill	17,260	17,260
Other noncurrent assets	369	775
Total assets	$ 185,466	$ 73,224
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Trade payables	$ 4,352	$ 1,350
Accrued expenses	8,165	6,105
Other liabilities	308	474
Total current liabilities	12,825	7,929
Liability related to sale of future royalties	49,969	—
Long-term liabilities	35	42
Total liabilities	62,829	7,971
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at December 31, 2018 and December 31, 2017; 11,969,928 and 8,902,784 issued and outstanding at December 31, 2018 and December 31, 2017	120	89
Additional paid in capital	214,694	114,522
Accumulated other comprehensive income	4,293	1,001
Accumulated deficit	(96,470)	(50,359)
Total stockholders' equity	122,637	65,253
Total liabilities and stockholders' equity	$ 185,466	$ 73,224

Albireo Pharma, Inc.

Consolidated Statements of Operations
(in thousands, except share and per share data)

	Year Ended December 31, 2018	2017
Revenue	$ 12,740	$ 1
Operating expenses:		
Research and development	31,732	12,991
General and administrative	18,061	15,246
Other operating (income) expense, net	837	(3,659)
Total operating expenses	50,630	24,578
Operating loss	(37,890)	(24,577)
Interest income (expense), net	(4,838)	40
Other non-operating income (expense), net	(3,363)	335
Net loss before income taxes	(46,091)	(24,202)
Income tax	20	212
Net loss	$ (46,111)	$ (24,414)
Net loss per share attributable to holders of common stock:		
Net loss per share - basic and diluted	$ (3.94)	$ (3.12)
Weighted average shares outstanding - basic and diluted	11,702,785	7,819,302

Source: Albireo Pharma, Inc.