Albireo Reports Second Quarter 2018 Financial Results

August 7, 2018

A4250 Phase 3 trial in PFIC underway

A4250 granted rare pediatric disease designation, eligible to apply for priority review voucher

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

BOSTON, Aug. 07, 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 second quarter ended June 30, 2018.

“The first half of 2018 was defined for Albireo by initiation of our Phase 3 program for A4250 in progressive familial intrahepatic cholestasis (PFIC),” said Ron Cooper, President and Chief Executive Officer of Albireo. “Notably, A4250 also received rare pediatric disease designation from the FDA, making Albireo eligible to apply for a priority review voucher upon submission of a new drug application for A4250. At the same time, we secured U.S. and E.U. method of use patents for elobixibat in the treatment of NASH, shared important findings on cholestatic liver disease at two scientific meetings, continued to strengthen our understanding of the cholestatic liver disease population and made key board and management appointments for our company.”

Recent Highlights

Advanced A4250 Program

- Enrolled the first patient in the PEDFIC-1 study, held successful E.U. and U.S. investigators meetings and continued activating trial sites. Twelve sites were initiated in seven countries as of the end of July. PEDFIC-1 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 (sBA) levels and pruritus. Data generated from this study are expected to support potential drug approval applications for A4250 in the U.S. and European Union Topline data currently are expected in late 2019 or early 2020.

- Reported data on a pharmacodynamic marker measured in the completed Phase 2 clinical trial of A4250 in children with cholestatic liver disease and pruritus at the European Association for the Study of the Liver (EASL) The International Liver Congress™. Treatment with A4250 decreased lysophospholipase autotaxin (ATX) levels in most patients, and there was a statistically significant correlation with a reduction in serum bile acids (sBA) ($r=0.60$; $p=0.003$). Pruritus intensity at baseline was significantly correlated with baseline levels of ATX ($r=0.67$; $p=0.001$).

- Presented data from two studies on cholestatic liver disease at the European Society for Pediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN) Annual Meeting.

- Data from a qualitative study supporting the development of Albireo’s proprietary Patient Reported Outcome (PRO) and Observer Reported Outcome (ObsRO) tools that are being used to measure pruritus in the PEDFIC-1 study illustrated the significant impact that pediatric cholestatic liver diseases, such as PFIC, have on quality of life.

- Research from a systematic literature review of partial external biliary diversion (PEBD) surgery in patients with PFIC found that changes in sBA and bilirubin appear useful as biomarkers in predicting both early and long-term outcomes. sBA responder rate is the primary endpoint in Europe and a key secondary endpoint in the U.S. in the PEDFIC-1 study.

- Participated in a PRIority MEdicines (PRIME) program kick-off meeting at the European Medicines Agency (EMA) that included the rapporteur. PRIME provides enhanced early dialogue with EMA to facilitate accelerated assessment of therapies considered to be priority medicines.

- Received rare pediatric disease designation from the FDA for A4250 in PFIC, affirming Albireo’s eligibility to apply for a priority review voucher upon submission of a new drug application for A4250. A4250 has received orphan drug designation for PFIC in the U.S. and E.U.

- Continued progress in commercialization planning for A4250 with completed qualitative, quantitative and pricing comparison market research on PFIC and other cholestatic liver diseases.

Elobixibat

- Positive Phase 3 trial results from Albireo licensee EA Pharma Co, Ltd. were presented at Digestive Disease Week (DDW) during a Distinguished Abstract Plenary Session. Data from this study demonstrated that elobixibat, a first-in-class IBAT inhibitor, resolved constipation in the short term, and improved bowel function and patient quality of life with long-term use in patients with chronic constipation in Japan. Elobixibat was well tolerated, with the most common side effects being mild gastrointestinal disorders. The data were published in *The Lancet Gastroenterology & Hepatology*.

- Granted a patent for a method of using elobixibat to treat NASH in both the U.S. and Europe.

Corporate

- Elected Anne Klibanski, M.D., and Stephanie Okey, M.S., to Albireo's Board of Directors

- Added Patrick Horn, M.D. as Chief Medical Officer and Jason G. Duncan, J.D., as General Counsel and Secretary to the leadership team.

- Presented at multiple investor conferences, including Jefferies 2018 Global Healthcare Conference and Deutsche Bank 43rd Annual Health Care Conference.

- Selected for inclusion in the Russell 2000® Index as part of the annual reconstitution of the Russell stock indexes.

Financial Update for the Three and Six-Month Periods Ended June 30, 2018

Three Months Ended June 30, 2018 vs. 2017

- Revenues were $730 thousand in the second quarter of 2018.
- R&D expense was $6.4 million up 113% compared to $3.0 million in the second quarter of 2017.
- G&A expense was $4.2 million, up 13.5%, compared to $3.7 million in the second quarter of 2017.
- Net Loss was $14.6 million, or $(1.22) per share, compared to $6.2 million, or $(0.86) per share in the second quarter of 2017.
- The Company had cash and cash equivalents at June 30, 2018, of approximately $183.2 million, compared to $53.2 million at December 31, 2017.

Six Months Ended June 30, 2018 vs. 2017

- Revenues were $11.9 million in the six months ending June 30, 2018.
- R&D expense was $12.6 million up 117% compared to $5.8 million in the six months ending June 30, 2017.
- G&A expense was $8.4 million, up 21.7%, compared to $6.9 million in the same six months of 2017.
- Net Loss was $16.2 million, or $(1.42) per share, compared to $12.8 million, or $(1.91) per share in the same six months of 2017.

Conference Call
As previously announced, Albireo will host a conference call and webcast today, August 7, 2018, at 8:30 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 13680985. 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 on the Events & Presentations section of the Media & Investors page of Albireo's website for at least two 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. 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, elobixibat 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 pricing of A4250 if approved; any future payment that HealthCare Royalty Partners or EA Pharma may make to Albireo or any other action or decision that EA Pharma may make concerning elobixibat or its business relationship with Albireo; 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; the significant control that EA Pharma has over the commercialization of elobixibat in Japan; 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 share and per share data)
(unaudited)

	June 30, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 183,228	$ 53,231
Prepaid expenses and other assets	610	1,054
Other receivables	2,406	726
Total current assets	186,244	55,011
Property and equipment, net	210	178
Goodwill	17,260	17,260
Other noncurrent assets	422	775
Total assets	$ 204,136	$ 73,224
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Trade payables	$ 3,020	$ 1,350
Accrued expenses	4,353	6,105
Other liabilities	541	474
Total current liabilities	7,914	7,929
Liability related to sale of future royalties	46,736	—
Long-term liabilities	39	42
Total liabilities	54,689	7,971
Stockholders' Equity:		
Common stock, $0.01 par value per share — 30,000,000 authorized at June 30, 2018 and December 31, 2017; 11,957,491 and 8,902,784 issued and outstanding at June 30, 2018 and December 31, 2017, respectively	120	89
Additional paid in capital	211,140	114,522
Accumulated other comprehensive income	4,768	1,001
Accumulated deficit	(66,581)	(50,359)
Total stockholders' equity	149,447	65,253
Total liabilities and stockholders' equity	$ 204,136	$ 73,224

See accompanying notes to Condensed Consolidated Financial Statements.

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,	
	2018	**2017**	**2018**	**2017**
Revenue	$ 730	$ 1	$ 11,932	$ 2
Operating expenses:				
Research and development	6,411	2,962	12,562	5,774
General and administrative	4,238	3,713	8,366	6,925
Other (income) expense, net	(191)	(65)	1,313	9
Total operating expenses	10,458	6,610	22,241	12,708
Operating loss	(9,728)	(6,609)	(10,309)	(12,706)
Interest income (expense), net	(1,666)	(152)	(2,682)	(401)
Non-operating income (expense), net	(3,209)	585	(3,231)	260
Net loss before income taxes	(14,603)	(6,176)	(16,222)	(12,847)
Income tax	—	—	—	—
Net loss	$ (14,603)	$ (6,176)	$ (16,222)	$ (12,847)
Net loss per share - basic and diluted	$ (1.22)	$ (0.86)	$ (1.42)	$ (1.91)
Weighted average shares outstanding - basic and diluted	11,938,357	7,171,610	11,417,463	6,734,555

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