Albireo Reports Q1 Financial Results and Business Update

May 6, 2021

– Bylvay™ (odevixibat) commercial readiness on track for anticipated H2 21 launch in U.S. and EU–

– Completed global commercial agreements for two key top ten markets –

– ASSERT & BOLD Phase 3 studies enrolling and on track –

– Advanced novel MOA ASBTi A3907 into Phase 1 study –

– Recent CTO appointment strengthens leadership team as the Company readies for potential global Bylvay launch and progresses multiple clinical development programs –

– Company to host a conference call and webcast today at 10:00 a.m. ET –

BOSTON, May 06, 2021 (GLOBE NEWSWIRE) -- Albireo Pharma, Inc. (Nasdaq: ALBO), a clinical-stage rare pediatric liver disease company developing novel bile acid modulators, today provided a business update and reported financial results for the first quarter ended March 31, 2021.

“It is estimated that there are approximately one hundred thousand pediatric cholestatic patients in the world without an approved drug therapy. We are focused on global commercial readiness and pleased with the progress we have made towards ensuring we reach families in need with the potential first-in-class oral drug option for the treatment of PFIC patients,” said Ron Cooper, President and Chief Executive Officer of Albireo. “Beyond PFIC, we are rapidly advancing our Phase 3 programs in biliary atresia and Alagille syndrome while moving A3907, a new bile acid modulator with novel MOA, into the clinic.”

Recent and Upcoming Highlights

Bylvay (odevixibat)

- The New Drug Application (NDA) for Bylvay is currently being reviewed by the U.S. Food and Drug Administration (FDA) under Priority Review with a Prescription Drug User Fee Act (PDUFA) goal date of July 20, 2021. The Company was informed that there are no plans for an FDA advisory committee meeting and continues to engage in ongoing discussions. Upon approval, the Company expects issuance of a rare pediatric disease Priority Review Voucher and commercial launch.

- In Europe, the Marketing Authorization Application (MAA) for Bylvay is the only submission for an ileal bile acid transport inhibitor (IBATi) granted accelerated assessment by the EMA. Bylvay was granted Orphan Designation as well as access to the PRIority MEdicines (PRIME) scheme for the treatment of PFIC. The Company continues on the accelerated assessment process and anticipates approval and launch in the second half of 2021.

- Bylvay has been provisionally accepted by both the FDA and EMA as the brand name for odevixibat.

- Global commercial readiness continues. Hiring, onboarding and training of specialized U.S. and international teams is underway. In the U.S., sales representatives have conducted onboarding and training activities, while also surveying U.S. HCPs on their current management of PFIC patients in preparation for launch. The team is actively engaging with Travere Therapeutics to operationalize our combined efforts for the U.S. launch. In Europe, Germany is an early launch country that has the largest market potential, so the Company has prioritized and completed hiring of the full in-country commercial organization.

- Countries such as Turkey and Saudi Arabia have an increased prevalence of PFIC, making them top 10 commercial market opportunities. Albireo completed two ex-U.S. commercial distributorships with GEN İlaç in Turkey, and Genpharm Services for Saudi Arabia and other Gulf countries, which follows the agreement with Medison Pharma, Ltd. ("Medison”) for Bylvay in Israel. Each company is a rare disease market leader in its respective region and these agreements advance the Company’s global strategy for the commercialization of Bylvay.

- The BOLD Study, the first and only pivotal Phase 3 trial of an IBAT inhibitor in biliary atresia, the largest pediatric cholestatic liver disease, is on track with 48 global site activations, including 18 U.S. and 30 ex-U.S. global sites.

- The Company continues to enroll and dose patients in the ASSERT Study, a global Phase 3 pivotal trial of Bylvay in

patients with Alagille Syndrome (ALGS).

Early-Stage Pipeline

- Initiated Phase 1 study with A3907, the first oral systemic apical sodium-dependent bile acid transporter (ASBT) inhibitor in clinical studies, being developed for adult cholestatic liver diseases such as primary sclerosing cholangitis (PSC) and primary biliary cholangitis (PBC). Phase 1 study is a first-in-human, double-blind, single and multiple ascending dose study in healthy adult subjects to investigate the safety, tolerability, pharmacokinetics and pharmacodynamics of an A3907 oral formulation.

- The Company was issued a U.S. composition of matter and method of use patent for novel compound A3907. The patent covers the chemical composition of A3907 and use in multiple adult liver and viral diseases and is one of a series of patents that will expire between 2039 and 2040, not including patent term extension. The Company continues to pursue additional patents for A3907.

- Pre-clinical studies and modeling for A2342, the first potent oral systemic sodium-taurocholate co-transporting peptide (NTCP) inhibitor continue in viral and cholestatic liver diseases.

Corporate

- Recently appointed Joan Connolly as Chief Technology Officer and key member of the Albireo Enterprise Team. In this new role, Connolly will be responsible for overseeing drug substance and product development, clinical supply distribution, commercial supply chain and quality. Leadership addition strengthens the Company for commercial readiness, the potential global Bylvay launch and the ongoing clinical development programs.

First Quarter 2021 Financial Results

- Revenues were $2.0 million for the first quarter of 2021, compared to $1.5 million for the first quarter of 2020. The higher revenue was due to the estimated royalty revenue received from EA Pharma for elobixibat for the treatment of chronic constipation. The royalty revenue is passed on to HealthCare Royalty Partners.

- R&D expenses were $19.9 million for the first quarter of 2021, compared to $16.1 million for the first quarter of 2020. The higher expenses were principally due to personnel expenses as we continue to increase our headcount and program activities. The increase in program activities were primarily related to Bylvay for regulatory submissions in PFIC, the additional indications for biliary atresia and Alagille syndrome, as well as A3907 and partially offset by elobixibat and preclinical programs.

- G&A expenses were $15.3 million for the first quarter of 2021, compared to $8.2 million for the first quarter of 2020. The increase is attributable to personnel and related expenses as the Company continues to increase headcount and commercialization readiness activities related to PFIC.

- Net loss for the first quarter of 2021 was $43.7 million, or $(2.29) per share, compared to $31.5 million, or $(2.23) per share for the first quarter of 2020.

- The Company had cash and cash equivalents at March 31, 2021, of $217.1 million, which compares to $251.3 million at December 31, 2020. The Company has sufficient capital resources to fund the planned launch and development programs. Cash runway into 2023 and plans to monetize a Priority Review Voucher, if received upon approval. The 2021 operating cash burn is anticipated to be in the range of $130-$135 million. 2021 revenue from Bylvay is anticipated to be in the low single digit U.S. $ millions.

Conference Call

To access the live conference call by phone, please dial 888-599-8686 (domestic) or 323-994-2082 (international) and provide the access code 8407763. Live audio webcast will be accessible from the Media & Investors page of Albireo's website 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 Bylvay (odevixibat)

Bylvay is an investigational product candidate being developed to treat rare pediatric cholestatic liver diseases, including PFIC, biliary atresia and ALGS. A potent, once-daily, non-systemic ileal bile acid transport inhibitor (IBATi), Bylvay acts locally in the small intestine. Bylvay does not require refrigeration and can be taken as a capsule for older children, or opened and sprinkled onto food, which are factors of key importance for adherence in a pediatric patient population. The FDA has granted Priority Review and set a PDUFA goal date of July 20, 2021. In Europe, the EMA validated MAA. Bylvay is the only IBATi granted accelerated assessment by the EMA.

Bylvay also been granted Orphan Designation, as well as access to the PRIority MEdicines (PRIME) scheme for the treatment of PFIC. The EMA's Pediatric Committee has agreed to Albireo's Bylvay Pediatric Investigation Plans for PFIC and biliary atresia. In addition to PFIC, Bylvay has Orphan Drug Designations for the treatment of Alagille syndrome, biliary atresia and primary biliary cholangitis. With FDA and EMA regulatory submissions complete, Bylvay has the potential to become the first approved drug treatment for patients with PFIC in the U.S and Europe. The Company anticipates potential regulatory approvals, issuance of a rare pediatric disease priority review voucher and launch in the second half of 2021.

The MAA and NDA filings are supported by results from PEDFIC 1 and PEDFIC 2 Phase 3 studies. PEDFIC 1 was the first and largest, global, pivotal Phase 3 study conducted in PFIC, which evaluated the efficacy and tolerability of Bylvay in reducing pruritus and serum bile acids in a randomized, double-blind, placebo-controlled trial. In the PEDFIC 1 study, Bylvay met both primary endpoints and was well tolerated with very low incidence of diarrhea/frequent bowel movements (9.5% of Bylvay treated patients vs. 5.0% of placebo patients). ir.albireopharma.com/news-releases/news-release-details/albireo-phase-3-trial-meets-both-primary-endpoints-odevixibat. PEDFIC 2 is a long-term, open-label Phase 3 extension study. The Company also provides an Expanded Access Program (EAP) for eligible patients with PFIC in the U.S., Europe, Canada and Australia. Bylvay is also currently being evaluated in the BOLD Phase 3 trial in patients with biliary atresia, and the global Phase 3 ASSERT trial for ALGS.

About GEN

GEN is the leading specialty pharmaceutical company in Turkey with more than 20 years of experience. GEN partners with global pharmaceutical companies to bring innovative therapies and rare solutions to the community. GEN works compliant with ethical and scientific principles and strives to set the best standards for quality, safety, and value in the manufacture and access to health care products. With its GMP certificated production facility and R&D center based in Ankara, GEN offers solutions around the globe in the treatment of rare diseases and disorders. In addition to its HQ and offices in Turkey, GEN has offices in Germany, Russia, Kazakhstan, Uzbekistan, and Azerbaijan. For more information please visit GEN website at https://en.genilac.com.tr/. You can also follow GEN on LinkedIn, Twitter, Instagram, and Facebook.

About Genpharm Services
Genpharm Services is a privately held regional pharmaceutical company, focused on Rare Diseases and Specialty therapeutics for the Middle East and North Africa (MENA) region. It was founded in 2012 and has its regional offices in Dubai, UAE. It is led by senior industry executives and a dedicated team of experienced staff. It provides fast-track and early market access, sustainable commercial solutions and strategic advice on medical, regulatory, pricing and launch excellence. Our current strategic partners are Novartis Gene Therapy, Orchard Therapeutics, PTC Therapeutics, Sarepta Therapeutics, Ultragenyx Pharmaceuticals, Biohaven Pharmaceuticals and others. Thanks to our "patient first" approach and our Mantra "Bringing Cures to MENA patients", we are the first and only company to launch successfully Gene therapy in the region. For more information visit https://www.genpharmservices.com/ You can also follow us on LinkedIn and Twitter.

About Albireo

Albireo Pharma is a clinical-stage biopharmaceutical company focused on the development of novel bile acid modulators to treat rare pediatric and adult liver diseases. Albireo's lead product candidate, Bylvay, is being developed to treat rare pediatric cholestatic liver diseases with Phase 3 trials in PFIC, Alagille syndrome and biliary atresia. For PFIC, the FDA recently granted Priority Review and set a PDUFA goal date of July 20, 2021. In Europe, the EMA validated MAA. Bylvay is the only IBATi granted accelerated assessment by the EMA. The Company has also initiated a Phase 1 clinical trial for A3907 to advance development in adult cholestatic liver disease, with IND-enabling studies moving ahead with A2342 for viral and cholestatic liver disease. Albireo was spun out from AstraZeneca in 2008 and is headquartered in Boston, Massachusetts, with its key operating subsidiary in Gothenburg, Sweden. The Boston Business Journal named Albireo one of the 2020 Best Places to Work in Massachusetts for the second consecutive year. 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, initiation, duration, enrollment, results or timing for availability of results of, development of Bylvay or any other Albireo product candidate or program; including expectations regarding the impact of the COVID-19 pandemic on our business and our ability to adapt our plans and activities as appropriate; the pivotal trial for Bylvay in biliary atresia (BOLD), and the pivotal trial for Bylvay in Alagille syndrome (ASSERT); the target indication(s) for development or approval, the size, design, population, location, conduct, cost, objective, enrollment, duration or endpoints of any clinical trial, or the timing for initiation or completion of or availability or reporting of results from any clinical trial, including the long-term open-label extension study for Bylvay in PFIC, BOLD, ASSERT ; the Phase 1 clinical trial for A3907, the potential approval and commercialization of Bylvay; the potential for Bylvay to become the first approved drug for PFIC patients; discussions with the FDA or EMA regarding our programs; the potential benefits or competitive position of Bylvay or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential effects of Bylvay of the treatment of PFIC patients and its potential to improve the current standard of care; the potential benefits of an orphan drug designation; the potential issuance of a rare pediatric disease priority review voucher; 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," or the negative of these terms or other 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 the NDA for Bylvay for the treatment of pruritus in patients with PFIC will be approved by the FDA and whether the MAA for Bylvay in PFIC will be approved by the EMA; whether the FDA or EMA will complete their respective reviews within the target timelines, including the FDA's PDUFA goal date, as a potential result of the impact of the COVID-19 pandemic or otherwise; the risk that the NDA will not be approved despite the FDA's acceptance of the NDA for review; whether the FDA will require additional information, whether we will be able to provide in a timely manner any additional information that the FDA requests, and whether such additional information will be satisfactory to the FDA; other potential negative impacts of the COVID-19 pandemic, including on manufacturing, supply, conduct or initiation of clinical trials, or other aspects of our business; whether favorable findings from clinical trials of Bylvay to date, including findings in indications other than PFIC, will be predictive of results from other clinical trials of Bylvay; 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 to support approval of Bylvay 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 Bylvay or A3907, including BOLD and ASSERT, 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, company's clinical trials; 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.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(unaudited)

	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 217,081	$ 251,272
Prepaid expenses and other current assets	8,884	10,593
Total current assets	225,965	261,865
Property and equipment, net	440	478
Goodwill	17,260	17,260
Other assets	6,154	6,004
Total assets	$ 249,819	$ 285,607
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,577	$ 5,283
Accrued expenses	13,628	19,051
Current portion of note payable, net of discount	600	—
Other current liabilities	1,406	948
Total current liabilities	22,211	25,282
Liability related to sale of future royalties	67,113	65,894
Note payable, net of discount	9,136	9,621
Other long-term liabilities	3,441	3,579
Total liabilities	101,901	104,376
Stockholders’ Equity:		
Preferred stock, $0.01 par value per share — 50,000,000 authorized at March 31, 2021 and December 31, 2020; 0 and 0 issued and outstanding at March 31, 2021 and December 31, 2020, respectively	—	—
Common stock, $0.01 par value per share — 30,000,000 authorized at March 31, 2021 and December 31, 2020; 19,192,805 and 19,107,040 issued and outstanding at March 31, 2021 and December 31, 2020, respectively	192	191
Additional paid-in capital	459,937	456,472
Accumulated other comprehensive loss	(1,658)	(8,612)
Accumulated deficit	(310,553)	(266,820)
Total stockholders’ equity	147,918	181,231
Total liabilities and stockholders’ equity	$ 249,819	$ 285,607

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

	Three Months Ended March 31,	
	2021	2020
Revenue	$ 1,966	$ 1,549
Operating expenses:		
Research and development	19,943	16,130
General and administrative	15,273	8,153

Other operating expense, net	6,528	6,816
Total operating expenses	41,744	31,099
Operating loss	(39,778)	(29,550)
Interest expense, net	(3,955)	(1,938)
Net loss	$ (43,733)	$ (31,488)
Net loss per common share - basic and diluted	$ (2.29)	$ (2.23)
Weighted-average common shares used to compute basic and diluted net loss per common share	19,131,557	14,132,217

Source: Albireo Pharma, Inc.