Gemphire Therapeutics Reports Fourth Quarter and Fiscal Year 2017 Financial Results

March 15, 2018 4:01 PM ET

Conference Call & Live Webcast Today at 4:30pm Eastern Time

LIVONIA, Mich., March 15, 2018 (GLOBE NEWSWIRE) -- Gemphire Therapeutics Inc. (NASDAQ:GEMP), a clinical-stage biopharmaceutical company focused on developing and commercializing therapies for cardiometabolic disorders, including dyslipidemia, non-alcoholic fatty liver disease (NAFLD) and nonalcoholic steatohepatitis (NASH), today announced its financial results for the quarter and fiscal year ended December 31, 2017, and provided a corporate update.

“2017 was a productive and successful year for Gemphire, with significant progress in our comprehensive Phase 2 program for gemcabene which is designed to show proof of concept in a broad spectrum of cardiometabolic conditions, including hypercholesterolemia, severe hypertriglyceridemia, and NASH,” said Steven Gullans, Ph.D., interim CEO of Gemphire. “Our Phase 2 clinical trials completed in 2017, COBALT-1 and ROYAL-1, both met their primary endpoints, demonstrating that gemcabene is capable of lowering LDL-C, triglycerides and inflammation. In 2018, we expect to make meaningful progress on our remaining Phase 2 trials, including concluding our INDIGO-1 trial in severe hypertriglyceridemia (SHTG) patients in Q2, and enrolling open-label pediatric and adult programs in NAFLD/NASH. In addition, our goal is to advance gemcabene into Phase 3 in dyslipidemia in the second half of 2018, once agreement with the FDA is reached.”

“We are especially excited to have launched our investigation of gemcabene as a potential treatment for NAFLD/NASH with our recently initiated Phase 2 trials in pediatric NAFLD and in familial partial lipodystrophy (FPL),” added Dr. Gullans. “Our decision to move forward in NAFLD/NASH is based on the strong rationale for gemcabene to target the underlying pathology of these diseases. We believe that the drug’s novel mechanism of action, which includes both cardio- and liver-protective properties, together with its favorable safety profile, will provide it with a distinct competitive advantage.”

Fourth Quarter and Recent Corporate Highlights

- **Completed patient enrollment in Phase 2b INDIGO-1 trial investigating gemcabene in severe hypertriglyceridemia (SHTG) patients.**
  - INDIGO-1 is a 12 week, multicenter, double-blind, placebo-controlled, randomized trial in patients with SHTG (TG ≥ 500mg/dL) with or without background statin therapy.
  - Patients are enrolled into one of three arms: gemcabene 300 mg, gemcabene 600 mg or placebo once daily.
  - The primary endpoint is triglyceride (TG) reduction from baseline after 12 weeks.
  - Company remains on track to report top-line results from the study in the second quarter of 2018.
- **Launched a Clinical Program to Develop Gemcabene as a Treatment for NASH/NAFLD.**
  - Initiated a Phase 2a proof-of-concept (POC) clinical trial investigating gemcabene as a treatment for pediatric NAFLD. Pediatric NAFLD/NASH is a large and untreated market and provides an opportunity to advance gemcabene in an underserved area with millions of affected patients.
  - This open label pediatric trial is expected to enroll approximately 40 adolescent children between the ages of 12 and 17 who are diagnosed with NAFLD and abnormal liver function as assessed by liver transaminases. Top line results are expected in early 2019.
  - As part of its NASH program, Gemphire also initiated a Phase 2a clinical trial investigating gemcabene in Familial Partial Lipodystrophy (FPL). FPL is a rare genetic disorder and orphan disease which can lead to a variety of metabolic abnormalities including NASH.
  - The trial is designed to assess the efficacy and safety of two dosing regimens of gemcabene in up to eight FPL patients with elevated TGs and NAFLD. Top line results are expected in the second half of 2018.
  - The trials in pediatric NAFLD and FPL have been designed to pave the way for confirmatory trials in a

broader NASH adult and pediatric population.

- **Presented poster at The Liver Meeting®, the annual meeting of the Association for the Study of Liver Diseases (AASLD).**
  - Data from a preclinical study support clinical evaluation of gemcabene as a potential treatment for NAFLD/NASH.
- **Presented final results for ROYAL-1 clinical data in hypercholesterolemic patients at the American Heart Association (AHA) scientific sessions.**
  - Gemcabene met the primary endpoint and demonstrated a statistically significant lowering in LDL-C when used as an add-on for patients on maximally tolerated doses of statins.
  - Gemcabene was well tolerated with no evidence of drug-drug interactions (DDIs), liver toxicity, or muscle toxicity as an add-on to the highest doses of statins.
  - Greater efficacy was observed in a cardiometabolic population, patients with mixed dyslipidemia who have a particularly high atherogenic particle burden. In the mixed dyslipidemia group of patients, gemcabene 600 mg demonstrated a placebo adjusted LDL-C reduction of 23% ($p < 0.05$) on top of the highest doses of statins.
  - Consistent with gemcabene's mechanism of action, the sub-set of patients who had mixed dyslipidemia showed placebo adjusted reductions in non-HDL-C of 19%, ApoB of 26%, ApoE of 34% and triglycerides (TGs) of 33%.
  - Gemcabene treatment was associated with a significant median reduction in high-sensitivity C-reactive protein (hsCRP) of 40%, compared to 6% for those treated with placebo. hsCRP is a biomarker for inflammation and there is growing acceptance that reducing hsCRP is associated with reductions in major adverse cardiovascular events (MACE).
- **Raised approximately $23 million in net proceeds from a public offering of common stock in the first quarter of 2018.**

Upcoming 2018 Clinical Milestones

- Top-line results from the INDIGO-1 Phase 2b trial in SHTG are targeted for the second quarter of 2018 following completion of enrollment in the first quarter of 2018.
- Reaching agreement with FDA on the design of a Phase 3 program in familial hypercholesterolemia (FH) to enable initiation of Phase 3 study of gemcabene in FH by the end of 2018. In addition we plan to resolve our partial clinical hold with the FDA by completing and submitting our two-year rodent carcinogenicity study.
- Advance two Phase 2a clinical trials in NAFLD/NASH with Proof-of-Concept data in adult FLP reading out by the end of 2018, and enrollment advancing in pediatric NAFLD to enable data readout in Q1 2019.

Fourth Quarter and Fiscal 2017 Financial Update

General and administrative expense for the fourth quarter and fiscal year ended December 31, 2017 was $1.5 million and $10.4 million, respectively, compared to $2.4 million and $6.0 million for the fourth quarter and fiscal year ended December 31, 2016, respectively. The increase for the year was primarily attributable to an increase in staffing and professional services associated largely with supporting our clinical trials and becoming a public company in August 2016 together with separation costs incurred in 2017 for our former chief executive officer.

Research and development expense for the fourth quarter and fiscal year ended December 31, 2017 was $5.1 million and $22.7 million, respectively, compared to $4.8 million and $8.7 million for the fourth quarter and fiscal year ended December 31, 2016, respectively. The increase for the year was primarily attributable to increased staffing and fees paid to external service providers for clinical trial development, regulatory consulting, preclinical studies and manufacturing activities to support the clinical advancement of gemcabene.

Net loss attributable to common stockholders for the fourth quarter and fiscal year ended December 31, 2017 was $6.7 million, or ($0.63) per share, and $33.4 million, or ($3.23) per share, respectively, compared to $7.2 million, or ($0.78) per share, and $15.0 million, or ($2.57) per share, for the fourth quarter and fiscal year ended December 31, 2016, respectively.

At December 31, 2017, the company had cash and cash equivalents of approximately $18.5 million. Subsequent to December 31, 2017, the Company raised approximately $23.0 million in net proceeds from a public offering of common stock. Based on the Company's current operating plans, management believes the current cash on hand will be sufficient to fund operations through completion of the INDIGO-1 Phase2b study in 2018, the initiation of the Phase 3 program in dyslipidemia in the second half of 2018 and the completion of the first of two NASH/NAFLD Phase 2a studies in 2018 and the second in the first half of 2019.

Conference Call and Webcast

The call and webcast will take place on Thursday, March 15, at 4:30 pm Eastern Time to participate, please dial (844) 494-0188 (domestic) or +1 (425) 278-9114 (international) and reference conference ID 2090268. The live webcast can be accessed via the following link: https://edge.media-server.com/m6/p/bxoocvxs. A webcast replay will be available on the News & Events section of the Gemphire website for all interested parties following the call and will be archived and available for 90 days.

Gemcabene's mechanism of action and safety profile are highly differentiated from other clinical candidates

Gemphire's product candidate gemcabene is a first-in-class, once-daily, oral therapy that may be suitable for patients who are unable to achieve normal levels of LDL-C or triglycerides with currently approved therapies, primarily statins. Gemcabene's mechanism of action (MOA) is designed to enhance the clearance of very low-density lipoproteins (VLDLs) in the plasma and inhibition of the production of cholesterol and triglycerides in the liver. The combined effect of these mechanisms has been clinically observed to result in a reduction of plasma non-HDL-C, VLDL-C, LDL-C, apolipoprotein B and triglycerides. In addition, gemcabene has been shown to markedly lower C-reactive protein in humans and improve insulin sensitization. Gemcabene's MOA is liver-directed involving downregulation of hepatic apolipoprotein C-III (apoC-III) mRNA expression and decrease of plasma apoC-III levels. Gemcabene has also been shown to reduce liver sulfatase-2 mRNA levels, known to be elevated in diabetic and obese patients. Elevated sulfatase-2 is thought to reduce the effectiveness of the liver VLDL-remnant receptor (also known as Syndecan-1), that normally plays a role in removing triglyceride containing particles from the plasma. Gemcabene also reduces acetyl-CoA carboxylase (ACC1), CCR2/CCR5 receptor and TNF-α mRNA levels, markers thought to be involved in the progression of NASH/NAFLD. Gemcabene has demonstrated POC efficacy for NASH in the rodent STAM™ model developed at SMC Laboratories in Tokyo, Japan. Gemcabene has been tested as monotherapy and in combination with statins and other drugs in nearly 1,100 subjects across 23 Phase 1 and Phase 2 clinical trials. Given this profile of efficacy across multiple pathological pathways, as well as evidence of safety and tolerability, particularly when used as an add-on to many other therapeutic drugs, gemcabene has attributes that support studies in humans for NASH.

About Gemphire

Gemphire is a clinical-stage biopharmaceutical company that is committed to helping patients with cardiometabolic disorders, including dyslipidemia and NASH. The Company is focused on providing new treatment options for cardiometabolic diseases through its complementary, convenient, cost-effective product candidate gemcabene as add-on to the standard of care, especially statins, that will benefit patients, physicians, and payors. Gemphire's Phase 2 clinical program is evaluating the efficacy and safety of gemcabene in hypercholesterolemia, including FH and ASCVD, SHTG and NASH/NAFLD. Two trials supporting hypercholesterolemia have been completed under NCT02722408 and NCT02634151. Gemphire has completed recruitment for a clinical trial for SHTG under NCT02944383, and has initiated separate trials for adult NASH and pediatric NAFLD. Please visit www.gemphire.com for more information.

Forward Looking Statements

Any statements in this press release about Gemphire's future expectations, milestones, goals, plans and prospects, including statements about Gemphire's financial prospects, future operations and sufficiency of funds for future operations, clinical development of Gemphire's product candidate, expectations regarding future clinical trials, regulatory submissions and meetings and future expectations and plans and prospects for Gemphire, expectations for the future competitive environment for gemcabene, expectations regarding operating expenses and cash used in operations, and other statements containing the words "believes," "anticipates," "estimates," "expects," "intends," "plans," "predicts," "projects," "targets," "may," "potential," "will," "would," "could," "should," "continue," "scheduled" and similar

expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: developments in the capital markets, the success and timing of Gemphire's regulatory submissions and pre-clinical and clinical trials; regulatory requirements or developments; changes to Gemphire's clinical trial designs and regulatory pathways; changes in Gemphire's capital resource requirements; the actions of Gemphire's competitors; Gemphire's ability to obtain additional financing; Gemphire's ability to successfully market and distribute its product candidate, if approved; Gemphire's ability to obtain and maintain its intellectual property protection; and other factors discussed in the "Risk Factors" section of Gemphire's annual report and in other filings Gemphire makes with the SEC from time to time. In addition, the forward-looking statements included in this press release represent Gemphire's views as of the date hereof. Gemphire anticipates that subsequent events and developments will cause Gemphire's views to change. However, while Gemphire may elect to update these forward-looking statements at some point in the future, Gemphire specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Gemphire's views as of any date subsequent to the date hereof.

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Gemphire Therapeutics Inc.
Condensed Statements of Comprehensive Loss
(in thousands, except per share amounts)

	Year Ended December 31,		Three Months Ended December 31,	
	2017	2016	2017	2016
Operating expenses:	(unaudited)		(unaudited)	(unaudited)
General and administrative	$ 10,438	$ 5,956	$ 1,487	$ 2,389
Research and development	22,686	8,740	5,080	4,839
Total operating expenses	33,124	14,696	6,567	7,228
Loss from operations	(33,124)	(14,696)	(6,567)	(7,228)
Interest (expense) income	(286)	114	(179)	13
Other (expense) income	(5)	(4)		1
Net loss	$ (33,415)	$ (14,586)	$ (6,746)	$ (7,214)
Adjustment to redemption value on Series A convertible preferred stock	—	(366)	--	--
Net loss attributable to common stockholders	$ (33,415)	$ (14,952)	$ (6,746)	$ (7,214)
Net loss per share:				
Basic and diluted	$ (3.23)	$ (2.57)	$ (0.63)	$ (0.78)
Number of shares used in per share calculations:				
Basic and diluted	10,349,136	5,809,396	10,633,042	9,264,228

Gemphire Therapeutics Inc.
Balance Sheet Data
(in thousands)

	Year Ended December 31, 2017	2016
	(unaudited)	
Cash and cash equivalents	$ 18,473	$ 24,033
Total assets	19,017	24,754
Term loan (long-term portion)	8,683	—
Total liabilities	15,076	4,122
Accumulated deficit	(60,474)	(27,059)
Total stockholders' equity	3,941	20,632

Primary Logo

Gemphire Therapeutics Inc.