

## **Gemphire Announces Fiscal Year 2016 Financial Results and Provides Corporate Update**

March 15, 2017 4:01 PM ET

### **Announces Date for 2017 Annual Meeting of Stockholders**

*Conference Call and Live Webcast, Today at 4:30 Eastern Time*

LIVONIA, Mich., March 15, 2017 (GLOBE NEWSWIRE) -- Gemphire Therapeutics Inc. (NASDAQ:GEMP), a clinical-stage biopharmaceutical company focused on developing and commercializing therapies for cardiometabolic disorders, including dyslipidemia and NASH, today announced financial results for the fourth quarter and full fiscal year ended December 31, 2016 and provided a corporate update.

“Since our IPO in 2016, we continue to advance our clinical programs to validate the therapeutic potential of gemcabene as a first-in-class, once-daily, oral drug candidate for the treatment of dyslipidemia and NASH,” said Mina Sooch, President and CEO of Gemphire. “Our experienced team that has been involved in the development of Lipitor® and other cardiometabolic drugs has successfully launched a comprehensive clinical program evaluating gemcabene in the broad dyslipidemia indications of HeFH/ASCVD and SHTG, as well as in the orphan HoFH population. With the close of the recent financing, we are expanding our Phase 2 clinical program with a fourth planned trial evaluating gemcabene in NASH.”

“Our target market opportunity is over 20 million cardiovascular and NASH patients in the US that need additional cost-effective, complementary new therapies to reduce the risk of cardiovascular and liver diseases,” continued Ms. Sooch. “It is an exciting time to develop a late stage asset in the cardiovascular and NASH space given the recent positive industry developments with Amgen’s positive PCSK9 inhibitor outcomes study further strengthening the LDL-C hypothesis relevant for our ROYAL program and Intercept’s encouraging FDA guidance on acceptable surrogate endpoints for Phase 3 trials, providing a more efficient development path for the NASH indication with no approved therapies. We look forward to a transformational year in 2017 with data readouts expected from all three of our Phase 2b trials in dyslipidemia patients.”

### **Fourth Quarter and Recent Corporate Highlights**

- In October 2016, Gemphire appointed Lee Golden, M.D., an interventional cardiologist with extensive biotech and large-cap pharma experience, as our Chief Medical Officer.
- In November 2016, a key US provisional application was converted to a PCT application directed to the use of gemcabene for treating patients with mixed dyslipidemia or NASH. Any patents issued from this application, absent extension, would expire in 2036. Further, in October 2016, a European patent was granted and validated in 21 European countries including the UK, Germany, France, and the Netherlands for the use of gemcabene for decreasing the risk for developing pancreatitis for SHTG patients with triglycerides 500 mg/dL or greater. These patents will expire in 2031 absent any extension.
- In November 2016, we initiated enrollment for the ROYAL-1 study, a randomized, placebo-controlled, double-blind Phase 2b trial designed to evaluate gemcabene in the treatment of patients with hypercholesterolemia, including those with HeFH and ASCVD, not adequately controlled for LDL-C on high-intensity or moderate-intensity stable statin therapy. Enrollment has completed ahead of schedule. This indication, consistent with the PCSK9 inhibitor labels for Repatha® and Praluent®, represents 11 million addressable patients in the US.
- In December 2016, we initiated screening for the INDIGO-1 study, a randomized, placebo-controlled, double-blind Phase 2b trial evaluating gemcabene to lower triglycerides in SHTG patients. This indication represents 3 million addressable patients in the US.
- In January 2017, we announced positive preclinical proof of concept data on gemcabene in the treatment of NASH and plans to launch a clinical development program in NASH in 2017. This indication represents 6 million addressable patients in the US.

- In January 2017, we announced positive interim data on the LDL-C primary endpoint from the ongoing open label COBALT-1 trial investigating gemcabene in HoFH patients. No SAEs have been reported and the safety profile remains consistent with previous studies. Gemcabene has FDA orphan designation for HoFH in the US.
- In March 2017, we closed on a \$12.5 million private placement that provides funding for the AZURE-1 Phase 2 trial investigating gemcabene in NASH patients, manufacturing activities, and general corporate purposes. This extends the Company's cash runway until at least late 2018.

### **Upcoming 2017 Clinical Milestones**

- Poster presentation at American College of Cardiology (ACC) Annual Scientific Session in March 2017 on gemcabene lipid-lowering effects and insulin sensitization in obese patients.
- Top-line results from COBALT-1 Phase 2b trial are expected in June 2017.
- Top-line results from ROYAL-1 Phase 2b trial are expected in the third quarter of 2017.
- Top-line results from INDIGO-1 Phase 2b trial are expected in the fourth quarter of 2017.
- Plan to initiate AZURE-1 Phase 2 trial in NASH in the second half of 2017.

### **Year End 2016 Financial Update**

General and administrative expense for the three months and full year ended December 31, 2016 were \$2.4 million and \$6.0 million, respectively, compared to \$1.0 million and \$3.2 million for the three months and full year ended December 31, 2015, respectively. The increase over the prior year periods reflects the added infrastructure and personnel costs to support the clinical trials as well as costs associated with operating as a public company.

Research and development expense for the three months and full year ended December 31, 2016 were \$4.8 million and \$8.7 million, respectively. This compared to R&D expense of \$1.5 million and \$4.0 million for the three months and year ended December 31, 2015, respectively. The increase reflects costs of three separate trials ongoing in the fourth quarter of 2016, and the acceleration of some costs tied to the faster than anticipated enrollment rate of the ROYAL-1 trial.

Net loss attributable to common stockholders for the three months and full year ended December 31, 2016 were \$7.2 million and \$15.0 million, respectively, compared to \$3.8 million and \$13.0 million for the three months and full year ended December 31, 2015, respectively.

Cash and cash equivalents at December 31, 2016 totaled \$24.0 million compared to \$3.6 million at December 31, 2015. There was no debt outstanding at December 31, 2016. Cash used in operations in 2016 was \$11.0 million compared to \$5.4 million in 2015. The increase in cash year over year was primarily a result of the cash raised in our IPO in August 2016, offset by our operating expenses during the year.

On March 15, 2017, the Company closed its previously announced \$12.5 million private placement.

For 2017, management expects operating expenses and cash used in operating activities to be approximately double 2016 levels, primarily in research and development as we fund our ongoing clinical trials, and to be more heavily weighted in the first two quarters of 2017, aligned with the activity levels of ongoing trials. In addition, non-cash compensation expenses are currently approximately \$0.9 million per quarter, which management expects to trend at or above those levels through 2017. Based on the Company's current operating plans, management believes existing cash, including the net proceeds from the private placement, is sufficient to fund operations through completion of all three dyslipidemia trials reading out in 2017 as well as for the AZURE-1 Phase 2 clinical trial anticipated to complete in the second half of 2018.

### **Corporate Update**

In October 2016, Gemphire announced the appointment of Lee Golden, M.D. to the position of Chief Medical Officer. Dr. Golden has more than 15 years of industry experience in the cardiovascular space. He began his industry career at Pfizer as a Medical Director on the global Lipitor® team, where his responsibilities included overseeing multinational

cardiovascular trials.

In November 2016, a key US provisional application was converted to a PCT application directed to the use of gemcabene for treating patients with mixed dyslipidemia or NASH. Any patent issued from this application, absent extension, would expire in 2036. Further, in October 2016, a European patent, directed to the use of gemcabene for decreasing the risk for developing pancreatitis for SHTG patients with triglycerides 500 mg/dL or greater, was granted and validated in 21 European countries. These patents will expire in 2031 absent any extension. The European countries include Austria, Belgium, Bulgaria, Czech Republic, Denmark, Finland, France, Greece, Hungary, Ireland, Italy, Luxembourg, the Netherlands, Poland, Portugal, Romania, Spain, Sweden, Switzerland, the UK (patent number EP2658536), and Germany (patent number 602011031537.4).

In November 2016, Gemphire announced enrollment in ROYAL-1, a randomized, placebo-controlled, double-blind Phase 2b trial designed to evaluate gemcabene at a dose of 600 mg in the treatment of patients with hypercholesterolemia not adequately controlled on high-intensity or moderate-intensity stable statin therapy. The trial is enrolling a broad patient population, including patients with HeFH and ASCVD, who have baseline LDL-C (“bad cholesterol”) values  $\geq 100$  mg/dL despite background high and moderate intensity statin therapy. The rationale for evaluating gemcabene in these high risk patients is based on data from a prior Phase 2 study ([Trial 1027-018](#)). Enrollment of 105 patients in ROYAL-1 completed in February 2017, ahead of schedule, with plans to release top-line data in the third quarter of 2017.

In December 2016, Gemphire commenced screening for INDIGO-1, a randomized, placebo-controlled, double-blind Phase 2b trial in SHTG patients. The rationale for investigating gemcabene in this setting is based on data from a prior Phase 2 study ([Trial 1027-004](#)), which showed an approximately 40% reduction in triglycerides in a broad population and the potential for even higher reductions, approximately 60%, in those patients with baseline triglycerides above 500 mg/dL. The first patient dosed in the INDIGO-1 trial occurred in February 2017 with over 30 sites in the US and Canada. Top-line results from the trial are expected in the fourth quarter of 2017.

In January 2017, Gemphire announced plans to initiate clinical development of gemcabene in NASH, beginning with a Phase 2 trial (AZURE-1). The decision to rapidly advance in NASH was based on multiple completed clinical trials where gemcabene has shown the ability to lower biomarkers for blood lipids such as triglycerides (fat) and inflammation, both of which are relevant in the pathogenesis of NASH along with positive results from the recent preclinical NASH model. In this preclinical study conducted in diabetic mice, a hepatoprotective effect preventing liver disease progression was observed with gemcabene, in contrast to the control group. Specifically, gemcabene treatment resulted in a significant lowering of the liver NAFLD activity score (NAS), a composite measure of fatty liver disease comprised of measures of steatosis, inflammation, and hepatocyte ballooning. Progression of liver fibrosis was also significantly reduced with gemcabene treatment. The data from the preclinical NASH study will be submitted for publication in a peer review journal. We are proposing a 16-week multicenter trial in approximately 81 patients where the primary endpoint will be percent change in hepatic fat fraction as measured by MRI-PDFF. Following the filing of an IND, Gemphire plans to initiate a Phase 2 trial for NASH in the second half of 2017. The Company believes gemcabene’s ability to add complementary mechanisms that lower both triglycerides and inflammation while also reducing the progression of fibrosis in NASH patients, particularly those who are diabetic and obese, represents a differentiated treatment option.

Also in January 2017, the Company announced positive interim data on the LDL-C primary endpoint from the ongoing open label COBALT-1 trial. COBALT-1 is evaluating gemcabene in HoFH patients. The results from two genetically-confirmed HoFH patients through 8 weeks of treatment showed that gemcabene 600 mg decreased mean LDL-C by 28% on top of maximum statin and/or ezetimibe in HoFH patients. No SAEs have been reported, where reported AEs have been mild to moderate and are consistent with previously completed studies. Six patients have been enrolled in COBALT-1 with two patients being evaluated for enrollment for a total of 6 to 8 patients. As other Phase 2 HoFH trials by other sponsors have evaluated between 3 to 8 subjects, we believe the total number of subjects in our trial will be sufficient to support advancement into Phase 3. Top-line results at the 600 mg target commercial dose are expected by end of June 2017.

## Conference Call Details

Management will host an investment community conference call today, March 15, 2017 at 4:30 p.m. Eastern / 1:30 p.m. Pacific to discuss these results and answer questions. Shareholders and other interested parties may participate in the conference call by dialing Domestic: 844-494-0188; International: +1-425-278-9114. Conference ID: 87530687. A webcast will be available for 90 days on the Investors & Media section of the company's website (<http://www.gemphire.com>) under Events & Presentations.

## Annual Meeting of Stockholders

Gemphire today announced that it will host its Annual Meeting of Stockholders on Tuesday, May 23, 2017 at 8:00 AM Eastern Time, at the Company's headquarters, 17199 N. Laurel Park Drive, Livonia, Michigan 48152. The board of directors has established Monday, March 27, 2017 as the record date for determining stockholders entitled to vote at the meeting. Additional details about the meeting will be specified in the Company's proxy statement related to the Annual Meeting.

Because the date of the Annual Meeting is more than 30 days before the anniversary date of last year's Annual Meeting (which took place by written consent in lieu of such Annual Meeting prior to the Company's initial public offering), pursuant to applicable SEC rules and the Company's amended and restated bylaws, the Company has set the deadline for submission of proposals to be included in the Company's proxy materials, as well as director nominations by stockholders or other business to be brought before the Annual Meeting, as the close of business on March 27, 2017.

Written notice for any such proposals, nominations or other business must be received by the Company at its principal executive office (Gemphire Therapeutics Inc., 17199 N. Laurel Park Drive, Livonia, Michigan 48152) by such deadline and must comply with the procedures and requirements of applicable SEC rules and the Company's amended and restated bylaws.

## About Gemcabene

Gemphire's product candidate, gemcabene (CI-1027), 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 is designed to enhance the clearance of very low-density lipoproteins (VLDLs) in the plasma and inhibit the production of cholesterol and triglycerides in the liver. The combined effect for these mechanisms has been clinically observed to result in a reduction of plasma VLDL-C, LDL-C, and triglycerides. In addition, gemcabene has been shown to markedly lower C-reactive protein. Gemcabene is liver-directed and reduces apoC-III mRNA and plasma levels. Gemcabene also reduces acetyl-CoA carboxylase (ACC) and CCR2/CCR5 receptor mRNA levels, which may have applications in non-alcoholic steatohepatitis (NASH)/non-alcoholic fatty liver disease (NAFLD). Gemcabene has demonstrated proof of concept efficacy in the STAM<sup>TM</sup> model for NASH developed at SMC Laboratories in Tokyo, Japan. Gemcabene has been tested as monotherapy and in combination with statins and other drugs in 895 subjects across 18 Phase 1 and Phase 2 clinical trials and has demonstrated promising evidence of efficacy, safety and tolerability.

## About Gemphire

Gemphire is a clinical-stage biopharmaceutical company that is committed to helping patients with cardiometabolic disorders, including dyslipidemia and NASH. We are focused on providing new treatment options for cardiometabolic diseases through our 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 has initiated 3 clinical trials for homozygous familial hypercholesterolemia (HoFH), heterozygous familial hypercholesterolemia (HeFH)/atherosclerotic cardiovascular disease (ASCVD), and severe hypertriglyceridemia (SHTG) under [NCT02722408](#), [NCT02634151](#), and [NCT02944383](#), respectively with a 4<sup>th</sup> planned trial in NASH. Please visit [www.gemphire.com](http://www.gemphire.com) for more information.

## Forward Looking Statements

Any statements in this press release about Gemphire's future expectations, 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 and future expectations and plans and prospects for Gemphire, 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: 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; 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2016, 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.

## Gemphire Therapeutics Inc.

### Balance Sheet Data

(in thousands)

	December 31, 2016	December 31, 2015
	(unaudited)	
Cash and cash equivalents	\$ 24,033	\$ 3,620
Total assets	24,754	4,490
Accounts payable and accrued liabilities	4,121	2,148
Convertible notes	—	6,769
Total liabilities	4,122	8,917
Series A convertible preferred stock	—	7,953
Common stock	17	12
Additional paid-in capital	47,674	—
Accumulated deficit	(27,059 )	(12,392 )
Total stockholders' equity (deficit)	20,632	(12,380 )

## Condensed Statements of Comprehensive Loss

(in thousands, except per share amounts)

	For the Three Months Ended		For the Year Ended	
	December 31,		December 31,	
	2016	2015	2016	2015
	(unaudited)		(unaudited)	
Operating expenses:				
General and administrative	\$ 2,389	\$ 1,047	\$ 5,956	\$ 3,177
Research and development	4,839	1,464	8,740	3,991
Acquired in-process research and development	—	—	—	908
Total operating expenses	7,228	2,511	14,696	8,076
Loss from operations	(7,228 )	(2,511 )	(14,696 )	(8,076 )
Interest and other income (expense)	14	(59 )	110	(953 )
Net loss	\$ (7,214 )	\$ (2,570 )	\$ (14,586 )	\$ (9,029 )
Adjustment to redemption value on Series A convertible preferred stock	—	(150 )	(366 )	(2,968 )
Premium upon substantial modification of convertible notes with certain stockholders	—	(1,047 )	—	(1,047 )
Net loss attributable to common stockholders	\$ (7,214 )	\$ (3,767 )	\$ (14,952 )	\$ (13,044 )
Net loss per share:				
Basic and diluted	\$ (0.78 )	\$ (1.14 )	\$ (2.57 )	\$ (4.54 )
Number of shares used in per share calculations:				
Basic and diluted	9,264	3,297	5,809	2,875

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