

## **Gemphire Therapeutics Provides Clinical Update**

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***Enrollment in the ROYAL-1 Clinical Trial Investigating Gemcabene in Hypercholesterolemia Is Ahead of Plan and Expected to be Completed This Month – Data Expected in the Third Quarter of 2017***

***Interim Data from the COBALT-1 Trial for Homozygous Familial Hypercholesterolemia (HoFH) Patients Expected the Week of January 30, 2017***

***INDIGO-1 Trial Commenced for Severe Hypertriglyceridemia (SHTG) Patients***

LIVONIA, Mich., Jan. 05, 2017 (GLOBE NEWSWIRE) -- Gemphire Therapeutics Inc. (NASDAQ:GEMP), a clinical-stage biopharmaceutical company focused on developing and commercializing therapies for the treatment of dyslipidemia, a serious medical condition that increases the risk of life threatening cardiovascular diseases, and nonalcoholic fatty liver disease/nonalcoholic steatohepatitis (NAFLD/NASH), today provided the following update on the Company's three clinical trials for its product candidate, gemcabene:

- Enrollment is greater than 90% complete toward the target enrollment of 104 patients in Gemphire's ROYAL-1 Phase 2b trial designed to investigate gemcabene in the treatment of patients with hypercholesterolemia not adequately controlled on high-intensity or moderate-intensity stable statin therapy. Due to the faster than expected enrollment, Gemphire now expects to complete enrollment in January and release top line data from ROYAL-1 trial in the third quarter of 2017. The Company believes the rapid enrollment may be a reflection of the large unmet need for additional LDL-C lowering in cardiovascular patients not at goal even on intense statin therapy and/or ezetimibe.
- Gemphire expects to announce interim results for its open-label COBALT-1 trial investigating gemcabene in patients with clinically diagnosed HoFH during the week of January 30, 2017.
- Gemphire commenced its Phase2b INDIGO-1 trial in SHTG patients in December 2016 with a successful Investigators' Meeting representing over 30 clinical sites, followed by the first SHTG patient pre-screened. The Company expects to report top-line results from INDIGO-1 in the fourth quarter of 2017.

"Early completion of enrollment in the ROYAL-1 trial in hypercholesterolemia patients will be an important accomplishment in the clinical development of gemcabene. We are very encouraged by the high level of interest shown by both patients and physicians in gemcabene, our novel, once-daily, oral therapy, and are pleased the trial is expected to be fully enrolled in only two months," said Mina Sooch, President and Chief Executive Officer of Gemphire. "The patient groups that have been enrolled in ROYAL-1 represent a large market of approximately 10 million patients in the U.S. alone that are currently unable to effectively manage their dyslipidemia despite current statin and ezetimibe therapies."

ROYAL-1 is a randomized, placebo-controlled, double-blind Phase 2b trial, with target enrollment of 104 adult patients at over 20 clinical sites in the United States. The trial was designed to enroll a broad patient population, including patients with heterozygous familial hypercholesterolemia (HeFH) and atherosclerotic cardiovascular disease (ASCVD), who still have baseline LDL-C or "bad cholesterol" values  $\geq 100$  mg/dL while on stable background lipid-lowering therapies.

Patients meeting eligibility requirements have been randomized in a 1:1 ratio to 600 mg of gemcabene or placebo for a 12 week treatment period. The primary endpoint is the percent change from baseline of LDL-C at 12 weeks. Secondary endpoints include the change from baseline in non-HDL-C, total cholesterol, triglycerides, ApoB, and hsCRP at the 12-week time point.

"The ROYAL-1 trial builds on the findings of a prior trial with gemcabene as an add-on therapy to stable statin. These data recently published in the Journal of Clinical Lipidology demonstrated gemcabene significantly lowered LDL-C up to

30% and lowered C-Reactive Protein (CRP), a marker of inflammation, by over 50%,” said Lee Golden, M.D., Chief Medical Officer at Gemphire. “If ROYAL-1 demonstrates similar results, then gemcabene could provide important benefits for patients and physicians treating patients with elevated LDL-C despite moderate and high intensity statin therapy.” The full publication in Journal of Clinical Lipidology, the official journal of the National Lipid Association, can be accessed online at: [http://www.lipidjournal.com/article/S1933-2874\(16\)30275-6/fulltext](http://www.lipidjournal.com/article/S1933-2874(16)30275-6/fulltext).

Additional information on the ROYAL-1 trial design, including eligibility criteria and site locations, can be found at <http://www.clinicaltrials.gov/>, using the NCT Identifier [NCT02634151](https://www.clinicaltrials.gov/ct2/show/study/NCT02634151).

Members of Gemphire’s management team will be presenting to investors and analysts in San Francisco, CA during the week of January 9, 2017. The slides presented at these meetings will be available immediately prior to and for 90 days following these meeting on the Investors and Media page of Gemphire’s website at <http://ir.gemphire.com>.

### **About Gemcabene**

Gemphire’s product candidate, gemcabene (CI-1027), is a novel, once-daily, oral therapy that may be suitable for patients who are unable to achieve normal levels of low-density lipoproteins (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 observed to result in a reduction of plasma VLDL-C, LDL-C, and triglycerides, as well as markedly lowering C-reactive protein. Gemcabene is liver-directed and reduces apoC-III mRNA and plasma levels and may also inhibit acetyl-CoA carboxylase (ACC) which has applications in NASH/NAFLD. 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 Heterozygous Familial Hypercholesterolemia (HeFH)**

The HeFH patient population is generally comprised of individuals who have one defective gene that leads to elevated LDL-C levels between 190 mg/dL and 500 mg/dL. These patients are prone to premature cardiovascular events. The incidence of patients with HeFH is estimated to be one in 200 and one in 500, and, accordingly, Gemphire estimates there are approximately 0.5 to 1.5 million patients with HeFH in the United States and 15 to 30 million in the rest of the world.

Current approved treatments for HeFH include statins, ezetimibe, bile acid sequestrants and the recently approved injectable PCSK9 inhibitors. Despite the availability of various treatments, many patients are still unable to achieve recommended LDL-C levels. In addition, patients, physicians and payors may prefer more convenient, cost-effective, oral drugs.

### **About Atherosclerotic Cardiovascular Disease (ASCVD)**

ASCVD represents patients who have experienced or are at risk of a cardiovascular event and are unable to meet their LDL-C lowering goal of less than 70 mg/dL with maximally tolerated statin therapy. This population also includes many patients who, in addition to not being able to meet their LDL-C lowering goal, have elevated triglyceride levels greater than 150 mg/dL and less than 500 mg/dL, categorized as mixed dyslipidemia. Gemphire estimate that approximately 10 million patients in the United States and 200 million patients in the rest of the world have a need for additional therapies to effectively and safely bring them closer to their LDL-C and triglyceride lowering goals.

Currently approved treatments for both primary hypercholesterolemia and ASCVD include statins, ezetimibe, bile acid sequestrants, niacin, fibrates and recently approved PCSK9 inhibitors. While these drugs have demonstrated efficacy in lipid-lowering in this population, they do not sufficiently address the patients with mixed dyslipidemia who need to lower both LDL-C and triglycerides.

### **About Gemphire**

Gemphire is a clinical-stage biopharmaceutical company focused on developing and commercializing therapies for the treatment of dyslipidemia, a serious medical condition that increases the risk of life threatening cardiovascular diseases, and NAFLD/NASH (nonalcoholic fatty liver disease). Gemphire has initiated 3 clinical trials for HoFH, HeFH/ASCVD,

and SHTG under [NCT02722408](#), [NCT02634151](#), and [NCT02944383](#), respectively. 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 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 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.

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