Gemphire Therapeutics Enrolls First Patient in the COBALT-1 Trial Investigating Gemcabene in Homozygous Familial Hypercholesterolemia

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Phase 2b Study to Determine Safety and Tolerability of Gemcabene in HoFH

Topline Data Readout Currently Anticipated in First Half -2017

LIVONIA, Mich., Sept. 26, 2016 (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 disease, and NAFLD/NASH (nonalcoholic fatty liver disease), today announced enrollment of its first patient in COBALT-1, a Phase 2b trial designed to investigate gemcabene in the treatment of homozygous familial hypercholesterolemia (HoFH). The purpose of this study is to assess the efficacy, safety, and tolerability of multiple rising doses of gemcabene in patients with HoFH who are on stable, lipid-lowering therapy, including statins, ezetimibe and Repatha.

“We are pleased to begin patient enrollment in the COBALT-1 trial for HoFH,” said Mina Sooch, President and Chief Executive Officer of Gemphire. “Many patients with HoFH have high levels of LDL-C despite the use of statins and other approved medications and remain at high risk for cardiovascular disease. We believe that gemcabene, which is being developed as a convenient, once a day, oral cost-effective medication, has the potential to be a complementary add on therapy in this patient population. Gemcabene has been well tolerated in 895 subjects treated across 18 clinical trials both as monotherapy and in combination with statins and other cardiovascular agents.”

The open-label Phase 2b trial, “*Efficacy and Safety of Gemcabene in Patients with Homozygous Familial Hypercholesterolemia on Stable, Lipid-Lowering Therapy (COBALT-1)*” will enroll up to eight adult patients at clinical sites in the United States, Canada, and Israel. Patients meeting eligibility requirements will be treated with an initial dose of 300 mg gemcabene, which will be increased to 600 mg at 4 weeks and then 900 mg at 8 weeks. All patients will continue to receive their lipid-lowering background therapy. The primary endpoint will be the percent change from baseline of LDL-C at 4, 8, and 12 weeks. Secondary endpoints include the change from baseline in non-HDL-C, total cholesterol, triglycerides, ApoB, and hsCRP at the same time points.

Gemphire currently anticipates the 12-week study to complete enrollment and all patient follow-up visits in the first half of 2017, with top-line data readout expected in June 2017.

Additional information on the trial design, including eligibility criteria and site locations, can be found at www.clinicaltrials.gov, using the NCT Identifier NCT02722408.

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 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 Homozygous Familial Hypercholesterolemia (HoFH)

HoFH is a rare genetic disease that is usually caused by a mutation in both alleles of the LDL receptor gene. The LDL

receptor is responsible for removing LDL from the blood. As a result, HoFH patients exhibit severely high LDL-C levels, are at very high risk of experiencing premature cardiovascular events, such as a heart attack or stroke, and develop premature and progressive atherosclerosis. LDL-C levels in HoFH patients are typically in the range of 500 mg/dL to 1,000 mg/dL, compared to a normal target range of 70 mg/dL to 100 mg/dL. Unless treated, most patients with HoFH do not survive adulthood beyond 30 years of age. There are approximately 300 to 2,000 HoFH patients in the United States and 6,000 to 45,000 patients in the rest of the world based on an estimated prevalence rate of one in 160,000 to one in one million.

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 disease, and NAFLD/NASH (nonalcoholic fatty liver disease). Please visit 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," 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 June 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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