Gemphire Announces Positive Results for Gemcabene in Proof-of-Concept NASH Preclinical Study

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Company Plans to Launch a Clinical Development Program in NAFLD/NASH in 2017 based on Gemcabene's Lipid-Lowering and Inflammation Mechanism of Action

LIVONIA, Mich., Jan. 09, 2017 (GLOBE NEWSWIRE) -- Gemphire Therapeutics Inc. (NASDAQ:GEMP) today announced expanded utility for gemcabene with compelling preclinical data results demonstrating proof of concept efficacy in a well-established animal model of nonalcoholic steatohepatitis (NASH). Gemphire plans to initiate clinical development of gemcabene in NASH/nonalcoholic fatty liver disease (NAFLD) with a Phase 2 trial (AZURE-1) in 2017. There are currently no treatments for NAFLD and NASH approved by the FDA. Gemcabene has the potential to add complementary mechanisms that lower both triglycerides (fat) and inflammation, which we believe prevents the progression of fibrosis, particularly for diabetic and obese people, which represent a large portion of the NASH patients.

The study investigated the effects of gemcabene on the widely used STAM^{TM} mouse model of NASH/fibrosis. In this model, diabetic mice that are fed a high fat diet rapidly develop fatty liver disease. When the mice were administered gemcabene, a hepatoprotective effect preventing liver disease progression was observed, 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. Additionally, hepatic gene expression and plasma markers indicative of inflammation (e.g., CRP and CCR2/CCR5), and lipid modulation (e.g., ApoC-III) were significantly reduced as were other markers. The complete NASH preclinical results will be submitted for publication in 2017.

"We are delighted to share the results of our preclinical study demonstrating exciting proof of concept data for gemcabene in NASH, including its anti-fibrotic and anti-inflammatory features," said Mina Sooch, President and Chief Executive Officer of Gemphire. "Moving forward with Phase 2 clinical development in NAFLD/NASH is now a key priority for us and is in line with our strategy to expand the breadth of indications for gemcabene beyond the 14 million dyslipidemia patients in need of cholesterol and triglyceride reduction that we are also pursuing. Given the high prevalence of NAFLD and those that progress to NASH in the developed world, estimated at 6 million in the US, and only projected to increase further given the current trends in diabetes and obesity, we see it as a sizable additional opportunity to the cardiovascular disease market."

"There is an urgent need for a safe pharmacologic therapy that can regress or prevent progression of liver damage and fibrosis in NAFLD/NASH patients," said Dr. Charles L. Bisgaier, Co-founder and Chief Scientific Officer of Gemphire. "Historical preclinical and clinical studies have shown significant lowering in plasma of LDL-C, triglycerides and CRP when given oral, once-daily gemcabene. Gemcabene's dual mechanism of action includes inhibition of hepatic *de novo* cholesterol and triglyceride synthesis. Gemcabene also reduces hepatic apoC-III and CRP gene expression. We believe these properties of gemcabene are well-suited to address the key functional aspects of NAFLD/NASH and to target the underlying disease pathology."

Gemphire intends to conduct a Phase 2 trial (AZURE-1) to evaluate gemcabene's therapeutic effects in NAFLD/NASH patients. Our current accepted IND for dyslipidemia indications is with the Metabolism and Endocrine Division of the FDA, and we will be submitting a new IND for the NAFLD/NASH indication to the GI Division.

"We are excited by our plan to launch our clinical development program for gemcabene in NAFLD/NASH in 2017," said Dr. Lee Golden, Chief Medical Officer at Gemphire. "We already have a comprehensive data set on gemcabene from 895 subjects across 18 trials. We believe these results should enable us to initiate a Phase 2 proof of concept trial in NAFLD/NASH. In prior studies Gemcabene has been shown to be well tolerated and has not shown any liver toxicities at doses between 150 to 900 mg up to 12 weeks as monotherapy or when combined with statins and other drugs. Like many

other diseases we believe that NAFLD/NASH patients will require therapies targeting several mechanisms of action. In clinical trials to date, gemcabene has also shown a low likelihood for drug-drug interactions, particularly with statins that are widely used in in diabetic and dyslipidemic patients, which we believe adds to gemcabene's potential value in this disease."

About NAFLD/NASH

Nonalcoholic steatohepatitis (NASH) is a severe disease of the liver caused by inflammation and a buildup of fat in the organ. In the United States, NASH affects up to approximately 2-5% of the population. An additional 10-30% of Americans have fat in their liver, but no inflammation or liver damage, a condition called NAFLD or "fatty liver." The underlying cause of NASH is unclear, but it most often occurs in persons who are middle-aged and overweight or obese. Many patients with NASH have elevated serum lipids, diabetes or pre-diabetes. Progression of NAFLD/NASH can lead to liver cirrhosis, fibrosis, hepatocellular carcinoma, liver failure and liver-related death. Liver transplantation is currently the only treatment for advanced cirrhosis with liver failure. At this time, there are no approved treatments by the FDA for NAFLD/NASH.

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, 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 demonstrated proof of concept efficacy in a NASH STAM™ model. 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 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 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 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.

Contact:
Andrew McDonald, Ph.D.
LifeSci Advisors, LLC
(646) 597-6987

Jeff Mathiesen, CFO
Gemphire Therapeutics Inc.
(734) 245-1700

Gemphire Therapeutics