Gemphire Announces Top-Line Data from Familial Partial Lipodystrophy (FPLD) Phase 2a Proof-of-Concept NAFLD/NASH Clinical Trial

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Gemcabene safely lowered serum triglycerides (TGs), the primary endpoint, in a subset of FPLD patients

LIVONIA, Mich., June 26, 2019 (GLOBE NEWSWIRE) -- Gemphire Therapeutics Inc. (NASDAQ: GEMP) today announced top-line results based upon the Company's preliminary review of the limited top-line dataset from the investigator-led Phase 2a study of adult FPLD patients evaluating efficacy, safety, and tolerability of oral gemcabene.

Five FPLD patients were enrolled in this open-label study with two patients having lamin A (LMNA) gene mutations and three patients with unknown causes of the condition. Average baseline serum triglyceride levels were 587.3 mg/dL and average MRI-PDFF liver fat fraction was 14.1%. All patients received a 300 mg/day dose of gemcabene for the first 12 weeks, with randomization to either the same dose (n=3) or a higher dose of 600 mg/day (n=2) for the subsequent 12 weeks.

Gemcabene treatment resulted in a median change in serum triglycerides (TG) of -19.6% for the five patients at twelve weeks (the primary endpoint). The range of TG responses was +40.4 % to -52.9%, with three patients showing decreases. Secondary endpoints included measurement of liver fat fraction by MRI-PDFF which showed reduction in 2 of the 3 responding patients. Four patients completed treatment and a fifth one discontinued at 22 weeks (with data carried forward as 24 weeks). Gemcabene appeared to be generally safe and well-tolerated in these five patients. There was one serious adverse event of benign paroxysmal positional vertigo, considered unrelated to gemcabene.

"This limited, five patient, proof-of-concept study provided evidence that gemcabene has the potential to lower TG levels in certain FPLD patients at 12 weeks," commented Dr. Elif Oral, the Principal Investigator of the study. "Furthermore, gemcabene appeared to be safe and well-tolerated in this patient population."

FPLD is a rare genetic disorder and orphan disease characterized by an abnormal distribution of fat (adipose) tissue, which can lead to a variety of metabolic abnormalities including the development of a fatty liver. Typical FPLD patients have a marked loss of subcutaneous fat from the arms, legs and trunk accompanied by variable amounts of excess fat deposition in the non-lipodystrophic areas such as the face, chin, back, and intraabdominal regions. First line therapy is focused on effective management of metabolic complications in patients with lipodystrophy and includes dietary fat restriction and other lifestyle changes. However, despite lifestyle changes and conventional hypoglycemic and hypolipidemic therapies, some FPLD patients continue to experience extreme hypertriglyceridemia, hepatic steatosis, inflammation and poorly controlled diabetes. Hypertriglyceridemia is a common condition of FPLD (serum TGs of 250 mg/dL to several thousands of mg/dL), increasing risk of pancreatitis, hepatic steatosis and premature cardiovascular disease. The genetic causes of FPLD are complex and can include defects in lamin A and C proteins.

"We are encouraged by the results of this study in this difficult to treat patient population," commented Dr. Steve Gullans, CEO of Gemphire. "Patient differences in responses to gemcabene in serum TGs and liver fat may be related to differing genetic profiles of the patients. Interestingly, clinical studies of adult NASH patients involving other therapeutic treatments have also shown heterogeneous patient responses. Further study will be needed to understand this phenomenon. We will continue to analyze all the data as it becomes available from our FPL trial to generate a more complete understanding of patient responses to gemcabene in the FPL population."

In the Company's INDIGO-1 trial, in patients with severe hypertriglyceridemia (TGs > 500 mg/dL), gemcabene lowered median serum TGs by -47%. In the COBALT-1 familial hypercholesterolemia trial, gemcabene lowered LDL-C by a mean of -25% (300 mg dose) to -30% (600 mg dose) in a population with an average baseline LDL-C of 351 mg/dL and already on background LDL-lowering therapies. Gemcabene has been tested as monotherapy and in combination with statins and other drugs in more than 1,100 subjects (defined as healthy volunteers and patients), across 25 Phase 1 and Phase 2 clinical trials and has demonstrated promising evidence of efficacy, safety and tolerability.

As previously announced, Gemphire continues to work with Ladenburg Thalmann & Co., Inc. to pursue strategic alternatives. In addition, the Company is continuing to make progress with the preclinical studies requested by the FDA to address the partial clinical hold on gemcabene.

About Gemphire
Gemphire is a clinical-stage biopharmaceutical company 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. Please visit www.gemphire.com for more information.

Forward Looking Statements
Any statements in this press release that are not statements of historical fact, including statements about Gemphire's future expectations, milestones, goals, plans and prospects, such as statements about the review of strategic alternatives to maximize stockholder value, the clinical development of Gemphire's product candidate, gemcabene, expectations regarding clinical trials, expectations regarding the results of a further analysis of the data as

it becomes available, timing and expectations for pre-clinical studies, regulatory submissions and meetings, future expectations and plans and prospects for gemcabene, and other statements containing the words “believes,” “anticipates,” “estimates,” “expects,” “intends,” “plans,” “predicts,” “projects,” “promising,” “targets,” “may,” “potential,” “will,” “would,” “could,” “should,” “continue,” “scheduled,” “goal” 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 ability of the Company to successfully and timely negotiate and consummate a possible transaction on terms that are favorable to the Company; Gemphire’s ability to analyze the results and understand the reasons for the patient responses; that MRI-PDFF scans or other follow-up tests of patients show undesirable side effects; uncertainties inherent in the clinical drug development process and the regulatory approval process, including the risk that gemcabene may have properties that could delay or prevent regulatory approval; Gemphire’s substantial dependence on its product candidate, gemcabene; 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; Gemphire’s ability to obtain additional financing; and other factors discussed in the "Risk Factors" section of Gemphire’s most recent annual report, subsequent quarterly reports 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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