Galectin Therapeutics Submits Seamless Adaptively-Designed Phase 2b/3 NASH-RX Protocol in NASH Cirrhosis

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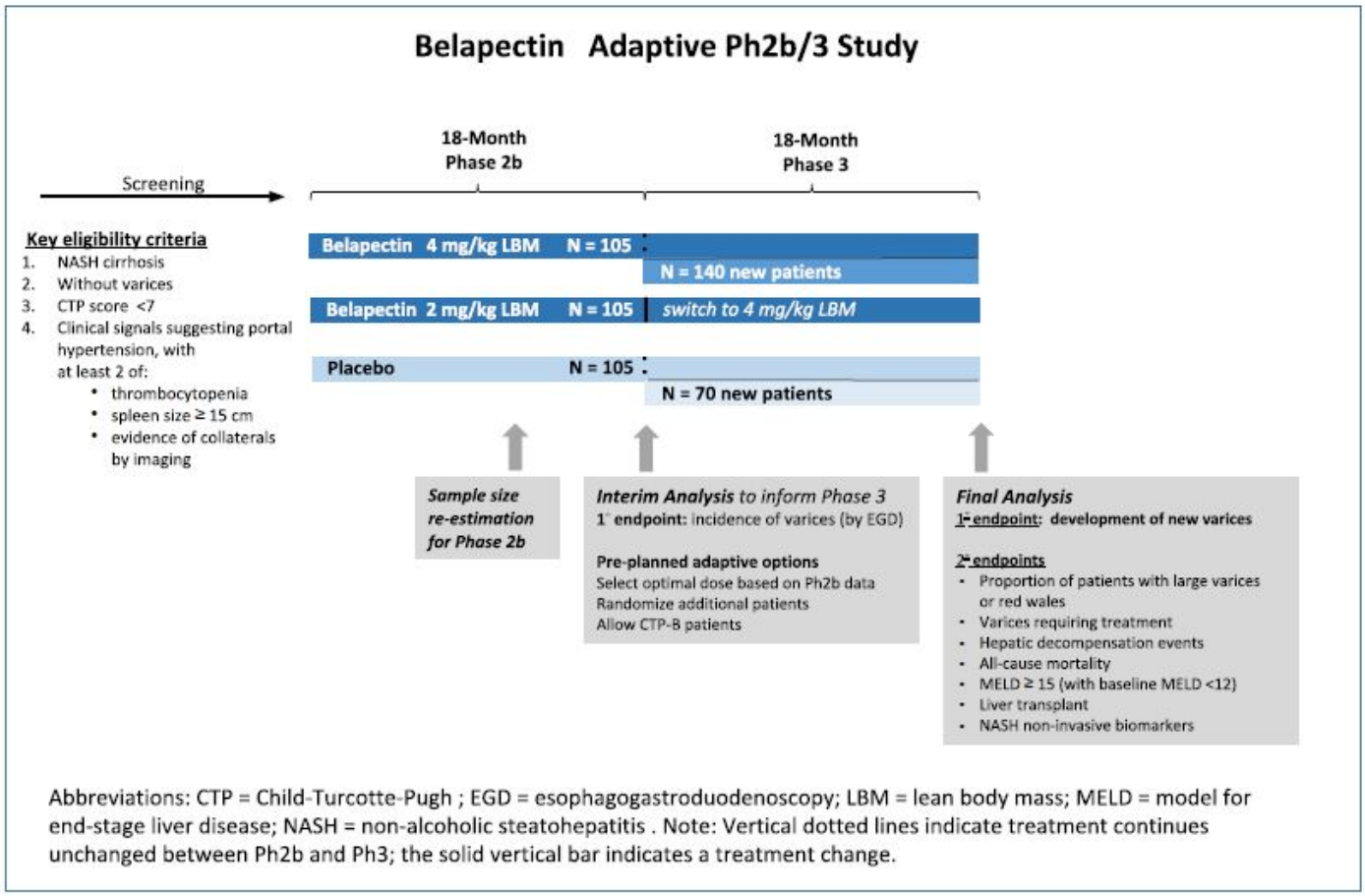
The first patient currently expected to be enrolled in the second quarter of 2020

NORCROSS, Ga., April 30, 2020 (GLOBE NEWSWIRE) -- Galectin Therapeutics, Inc. (NASDAQ: GALT), the leading developer of therapeutics that target galectin proteins, today announced that it has submitted to the U.S. Food and Drug Administration (FDA) its protocol for a seamless adaptively-designed Phase 2b/3 clinical study, the NASH-RX trial, evaluating the safety and efficacy of its galectin-3 inhibitor, belapectin (GR-MD-02), for the prevention of esophageal varices in patients with non-alcoholic steatohepatitis (NASH) cirrhosis.



In patients with NASH cirrhosis and clinical signs of portal hypertension but without esophageal varices at baseline, this trial will assess the effect of belapectin on the incidence of new varices (the primary endpoint) – as well as assessing the effect on the incidence of long-term, clinically significant cirrhosis-related “hard” outcomes (a key secondary efficacy endpoint).The filing anticipates clinical trials will begin in the second quarter of this year. The major features of the innovative, seamless adaptively-designed Phase 2b/3 study design are summarized below and graphically depicted in the figure:

- The design of this trial reflects the unmet medical needs of the target patient population for belapectin treatment: NASH patients with compensated cirrhosis who develop esophageal varices. Bleeding varices are a cause of death in about one-third of cirrhotic patients. There is no approved treatment for preventing varices in these patients. The development of new varices reflects the progression of hepatic cirrhosis and thus portends the development of other cirrhosis complications and outcomes such as significant ascites, hepatic encephalopathy, and ultimately liver failure.

- During the first 18 months of the trial, two belapectin dose levels (2 mg/kg LBM and 4 mg/kg LBM) will be compared to placebo. Then, at the interim analysis (IA), one belapectin dose will be selected based on efficacy and safety, for continued evaluation in Stage 2 (Phase 3). Prior trials have demonstrated the safety of belapectin with doses of up to 8 mg/kg for 52 weeks (Phase 2b Study GT-026).

“The protocol filed today reflects the previous feedback received from the FDA, as well as key contributions from Dr. Naga Chalasani and Dr. Stephen Harrison, NASH opinion leaders who are co-primary investigators for the study, biostatistical experts and numerous other collaborators at Covance, our CRO,” said Harold H. Shlevin, Ph.D., President and Chief Executive Officer of Galectin Therapeutics. “We are very grateful for all of their efforts, and for the previous feedback and suggestions from the FDA. The study design has been modified and further refined such that it provides for a prespecified interim analysis (IA). The IA of efficacy and safety data will be conducted after all planned subjects in Phase 2b component have completed at least 78 weeks (18 months) of treatment and a gastro-esophageal endoscopic assessment. The purpose of the IA is to allow potential seamless adaptive modifications of the study, including: (1) the selection of the optimal dose of belapectin for Phase 3; (2) the re-estimation of the study sample size for Phase 3 portion of the trial; (3) the re-evaluation of the randomization ratio for the Phase 3 portion of the trial; (4) the refinement of the inclusion and exclusion criteria for the Phase 3 portion of the trial, including the CTP status; (5) and/or termination of the study for overwhelming efficacy or futility.

The new trial design also minimizes invasive testing requirements, which we believe will enhance the enrollment and retention of patients. It also provides for a seamless transition of patients from the phase 2b component into the phase 3 stage, as well as provides for the potential addition of new patients. The trial design preserves the surrogate end-point concepts previously discussed with the FDA.

As previously communicated, this is a uniquely challenging time to start a new clinical trial, and we are doing everything in our direct control to prepare for the initiation of the study later this quarter; however, factors beyond our control, specifically related to the COVID-19 pandemic, may delay the trial's initiation. Notwithstanding that, we remain optimistic in moving forward. The unmet medical need for effective treatment for patients with NASH cirrhosis remains an important motivation.

The trial, entitled "A Seamless Adaptive Phase 2b/3, Double-Blind, Randomized, Placebo-controlled Multicenter, International Study Evaluating the Efficacy and Safety of Belapectin (GR-MD-02) for the Prevention of Esophageal Varices in NASH Cirrhosis" is now posted on www.clinicaltrials.gov (NCT04365868). Galectin Therapeutics will share more details about the protocol at the time the clinical trial begins. In addition, the FDA has received Galectin's protocol for the hepatic impairment study which will be conducted in subjects with normal hepatic function and subjects with varying degrees of hepatic impairment (NCT04332432). This filing anticipates that this study will also begin in the second quarter of this year.

About Belapectin (GR-MD-02)

Belapectin is a complex carbohydrate drug that targets galectin-3, a critical protein in the pathogenesis of fatty liver disease and fibrosis. Galectin-3 plays a major role in diseases that involve scarring of organs including fibrotic disorders of the liver, lung, kidney, heart and vascular system. The drug binds to galectin proteins and disrupts their function. Preclinical data in animals have shown that belapectin has robust treatment effects in reversing liver fibrosis and cirrhosis.

About Fatty Liver Disease with Advanced Fibrosis and Cirrhosis

Non-alcoholic steatohepatitis (NASH) has become a common disease of the liver with the rise in obesity and other metabolic diseases. NASH is estimated to affect up to 28 million people in the U.S. It is characterized by the presence of excess fat in the liver along with inflammation and hepatocytes damage (ballooning) in people who consume little or no alcohol. Over time, patients with NASH can develop excessive fibrosis, or scarring of the liver, and ultimately liver cirrhosis. It is estimated that as many as 1-2 million individuals in the U.S. will develop cirrhosis as a result of NASH, for which liver transplantation is the only curative treatment available. Approximately 8,890 liver transplants are performed annually in the U.S. There are no drug therapies approved for the treatment of liver fibrosis or cirrhosis.

About Galectin Therapeutics

Galectin Therapeutics is dedicated to developing novel therapies to improve the lives of patients with chronic liver disease and cancer. Galectin's lead drug belapectin is a carbohydrate-based drug that inhibits the galectin-3 protein which is directly involved in multiple inflammatory, fibrotic, and malignant diseases, for which it has Fast Track designation by the U.S. Food and Drug Administration. The lead development program is in non-alcoholic steatohepatitis (NASH) with cirrhosis, the most advanced form of NASH-related fibrosis. This is the most common liver disease and one of the largest drug development opportunities available today. Additional development programs are in treatment of combination immunotherapy for advanced melanoma and other malignancies. Advancement of these additional clinical programs is largely dependent on finding a suitable partner. Galectin seeks to leverage extensive scientific and development expertise as well as established relationships with external sources to achieve cost-effective and efficient development. Additional information is available at www.galectintherapeutics.com.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements relate to future events or future financial performance, and use words such as "may," "estimate," "could," "expect" and others. They are based on management's current expectations and are subject to factors and uncertainties that could cause actual results to differ materially from those described in the statements. These statements include those regarding the hope that Galectin's development program for belapectin will lead to the first therapy for the treatment of fatty liver disease with cirrhosis and those regarding the hope that our lead compounds will be successful in cancer immunotherapy and in other therapeutic indications. Factors that could cause actual performance to differ materially from those discussed in the forward-looking statements include, among others, that trial endpoints established in our clinical trials may not be achieved; Galectin may not be successful in developing effective treatments and/or obtaining the requisite approvals for the use of belapectin or any of its other drugs in development; the Company may not be successful in scaling up manufacturing and meeting requirements related to chemistry, manufacturing and control matters; the Company's currently planned clinical trial and any future clinical studies as modified to meet the requirements of the FDA may not produce positive results in a timely fashion, if at all, and could require larger and longer trials, which would be time consuming and costly; plans regarding development, approval and marketing of any of Galectin's drugs are subject to change at any time based on the changing needs of the Company as determined by management and regulatory agencies; regardless of the results of any of its development programs, Galectin may be unsuccessful in developing partnerships with other companies or raising additional capital that would allow it to complete the NASH-RX trial or further develop and/or fund any other studies or trials. Galectin has incurred operating losses since inception, and its ability to successfully develop and market drugs may be impacted by its ability to manage costs and finance continuing operations. Global factors such as coronavirus may limit access to NASH patient populations around the globe and slow trial enrollment and prolong the duration of the trial and significantly impact associated costs. For a discussion of additional factors impacting Galectin's business, see the Company's Annual Report on Form 10-K for the year ended December 31, 2019, and subsequent filings with the SEC. You should not place undue reliance on forward-looking statements. Although subsequent events may cause its views to change, management disclaims any obligation to update forward-looking statements.

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A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/535ab060-4a23-4e52-b9d6-527cf591fd12

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