



Enanta Pharmaceuticals Initiates Phase 1 Clinical Study of EDP-297, its Highly Potent and Targeted Follow-On Farnesoid X Receptor Agonist for the Treatment of Non-Alcoholic Steatohepatitis

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-- Initial clinical data including safety, tolerability and pharmacokineticsexpected in 2Q 2021 --

WATERTOWN, Mass.--(BUSINESS WIRE)-- Enanta Pharmaceuticals, Inc., (NASDAQ: ENTA), a clinical stage biotechnology company dedicated to creating small molecule drugs for viral infections and liver diseases, today announced that it has dosed the first subjects in its Phase 1 clinical trial of EDP-297, a highly potent and targeted follow-on farnesoid X receptor (FXR) agonist, being developed for the treatment of non-alcoholic steatohepatitis (NASH).

"We are excited to advance our efforts in NASH and progress EDP-297, our follow-on FXR agonist, into clinical development," commented Jay R. Luly, Ph.D., President and Chief Executive Officer of Enanta Pharmaceuticals. "In preclinical studies, EDP-297 demonstrated a compelling product profile, with a potency greater than that published on any FXR agonist in clinical development and high target-tissue distribution in the liver and intestine. Based on these data, we believe we may be able to effectively dose EDP-297 at lower doses and with reduced drug levels in non-targeted tissues, potentially improving tolerability by reducing pruritis. We look forward to reporting clinical data in the second quarter of 2021."

The Phase 1, randomized, double-blind, placebo-controlled, first-in-human study is designed to assess the safety, tolerability, and pharmacokinetics, including the effect of food intake, of orally administered EDP-297 in approximately 74 healthy adult subjects. Two phases are planned: a single ascending dose phase enrolling six cohorts, including a two-part food effect cohort, and a multiple ascending dose phase enrolling three cohorts.

In two recent poster presentations at the European Association for the Study of the Liver (EASL) Digital International Liver Congress™ 2020, treatment with EDP-297 demonstrated significantly reduced fibrosis progression and improved liver function in a rat model of NASH. Additionally, in 3D NASH microtissues, EDP-297 modulated multiple pathways associated with the pathogenesis of NASH, including decreased expression of genes encoding multiple lipogenic and inflammatory proteins, and significantly reduced expression of inflammatory and fibrotic genes and normalized circulating markers of liver injury.

About NASH and FXR

NASH is a serious form of non-alcoholic fatty liver disease (NAFLD) which is common in the United States and around the world and is closely associated with diabetes and obesity. Characterized by an excessive build-up of fat in the liver causing stress and damage to liver cells, NASH can lead to inflammation and fibrosis, causing permanent damage, including cirrhosis and impaired liver function, as well as cancer and eventually death. NASH is the leading cause of liver transplants in the United States and Europe and currently has no FDA-approved treatment.¹ A farnesoid X receptor is a main regulator of bile acid levels in the liver and small intestine. It responds to bile acids by regulating gene transcription of key enzymes and transporters, many of which play important roles in lipid metabolism, insulin resistance, inflammation and fibrosis.

About EDP-297, a FXR Agonist

EDP-297 is a potent FXR agonist and Enanta's follow-on FXR agonist candidate being developed for the treatment of NASH. EDP-297 represents a class of FXR agonists that has been designed to take advantage of increased binding interactions with the receptor. Preclinical findings of EDP-297 demonstrate potent anti-fibrotic, anti-inflammatory and hepatoprotective effects. EDP-297 has demonstrated preclinical potency greater than that published on any FXR agonist in clinical development today. Further, in preclinical models EDP-297 has been shown to be targeted to tissues important for efficacy, namely liver and intestine, versus plasma and skin.

About Enanta

Enanta is using its robust, chemistry-driven approach and drug discovery capabilities to become a leader in the discovery and development of small molecule drugs for the treatment of viral infections and liver diseases. Enanta's research and development efforts have produced clinical candidates for the following disease targets: respiratory syncytial virus (RSV), non-alcoholic steatohepatitis (NASH) and hepatitis B virus (HBV). Enanta is also conducting research in human metapneumovirus (hMPV) and SARS-CoV-2 (COVID-19).

Enanta's research and development activities are funded by royalties from hepatitis C virus (HCV) products developed under its collaboration with AbbVie. Glecaprevir, a protease inhibitor discovered by Enanta, is sold by AbbVie in numerous countries as part of its leading treatment for chronic HCV infection under the tradenames MAVYRET® (U.S.) and MAVIRET® (ex-U.S.) (glecaprevir/pibrentasvir). Please visit www.enanta.com for more information.

Forward Looking Statements Disclaimer

This press release contains forward-looking statements, including statements with respect to the prospects for further development of EDP-297 for NASH. Statements that are not historical facts are based on management's current expectations, estimates, forecasts and projections about Enanta's business and the industry in which it operates and management's beliefs and assumptions. The statements contained in this release are not guarantees of future performance and involve certain risks, uncertainties and assumptions, which are difficult to predict. Therefore, actual outcomes and results may differ materially from what is expressed in such forward-looking statements. Important factors and risks that may affect actual results include: the development risks of early stage discovery efforts in the disease areas in Enanta's research and development pipeline, such as NASH; the impact of development, regulatory and marketing efforts of others with respect to competitive treatments for NASH; Enanta's limited clinical

development experience; Enanta's need to attract and retain senior management and key scientific personnel; Enanta's need to obtain and maintain patent protection for its product candidates and avoid potential infringement of the intellectual property rights of others; and other risk factors described or referred to in "Risk Factors" in Enanta's most recent Form 10-Q for the quarter ended June 30, 2020 and other periodic reports filed more recently with the Securities and Exchange Commission. Enanta cautions investors not to place undue reliance on the forward-looking statements contained in this release. These statements speak only as of the date of this release, and Enanta undertakes no obligation to update or revise these statements, except as may be required by law.

¹ <https://www.worldhepatitisalliance.org/latest-news/infohep/3548835/nash-fastest-growing-reason-liver-transplant-united-states>



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