FDA Grants Fast Track Designation to Enanta's FXR Agonist Candidate, EDP-305, for the treatment of NASH with Liver Fibrosis

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WATERTOWN, Mass.--(BUSINESS WIRE)--Jan. 4, 2017-- Enanta Pharmaceuticals, Inc. (NASDAQ:ENTA), a research and development-focused biotechnology company dedicated to creating small molecule drugs for viral infections and liver diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Enanta's drug candidate EDP-305, an FXR agonist, Fast Track designation for the treatment of patients with non-alcoholic steatohepatitis (NASH) with liver fibrosis.

Fast track is a process designed to facilitate the development and expedite the review of drugs that treat serious conditions and fill an unmet medical need. A drug that receives Fast Track designation is also eligible for more frequent meetings and communications with the FDA to discuss the drug's development plan.[1]

"We are extremely pleased to receive this Fast Track designation from the FDA and look forward to working with the agency to bring this investigational treatment to patients as soon as possible," stated Jay R. Luly, Ph.D.

EDP-305 is currently in Phase 1 clinical development. Enanta's double-blind, placebo-controlled Phase 1a/b study is designed to evaluate the safety, tolerability and pharmacokinetics of single ascending doses (SAD) and multiple ascending doses (MAD) of EDP-305 in healthy adults, and in adults with presumptive non-alcoholic fatty liver disease (NAFLD) (obese, with or without pre-diabetes or type 2 diabetes). The study will enroll approximately 90 subjects and is planned to evaluate up to 5 dose cohorts, with EDP-305 administered orally, once daily.

The current study includes subjects with presumptive NAFLD in order to obtain initial safety data and additional data regarding the relationship between EDP-305 plasma concentration levels and certain pharmacological effects in the context of fatty liver disease. This relationship will be explored by using biomarkers that are relevant to the disease and to the activity of EDP-305, such as evaluation of lipids, glucose, insulin resistance and specific markers of FXR activity.

About EDP-305, a Farnesoid X Receptor (FXR) Agonist
EDP-305 is a potent FXR agonist and Enanta's lead product candidate being developed for the treatment of NASH and PBC. EDP-305 represents a new class of FXR agonists that has been designed to take advantage of increased binding interactions with the receptor. Further, this non-bile acid class contains steroid and non-steroid components, and does not contain the carboxylic acid group normally present in other classes of FXR agonists and natural bile acids that can lead to the formation of taurine and glycine conjugates. EDP-305 is currently in Phase 1 clinical development.

About NAFLD, NASH, and FXR
Non-alcoholic fatty liver disease (NAFLD) is the accumulation of excessive fat in the form of triglycerides in patients' liver cells (steatosis) that is not caused by alcohol. NAFLD is widely considered to be the liver expression of metabolic disease associated with type 2 diabetes, insulin resistance, obesity, and hyperlipidemia. A subgroup of NAFLD patients also develops liver cell injury and inflammation. This condition is called non-alcoholic steatohepatitis (NASH). Patients with NASH can develop fibrosis (the first stage of scarring of the liver) and ultimately cirrhosis of the liver, potentially leading to hepatocellular carcinoma or requiring a liver transplant. Farnesoid X receptor (FXR) is a nuclear receptor and 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 Enanta
Enanta Pharmaceuticals is a research and development-focused biotechnology company that uses its robust chemistry-driven approach and drug discovery capabilities to create small molecule drugs for viral infections and liver diseases. Enanta's research and development efforts are currently focused on four disease targets: Hepatitis C Virus (HCV), Non-alcoholic Steatohepatitis (NASH), Respiratory Syncytial Virus (RSV) and Hepatitis B Virus (HBV).

Enanta has discovered novel protease inhibitors that are members of the direct-acting-antiviral (DAA) inhibitor classes designed for use against the hepatitis C virus (HCV). These protease inhibitors, developed through Enanta's collaboration with AbbVie, include paritaprevir, which is contained in AbbVie's marketed DAA regimens for HCV, and glecaprevir (ABT-493), Enanta's second protease inhibitor product, which has been developed in Phase 3 studies as part of an investigational, pan-genotypic, once-daily, ribavirin-free, fixed-dose combination (G/P) with pibrentasvir (ABT-530), AbbVie's second NS5A inhibitor. AbbVie has announced it has filed an NDA for G/P with the FDA and is on track to submit a marketing authorization application for G/P in the European Union in early 2017. Enanta has also discovered EDP-305, an FXR agonist product candidate for NASH, currently in Phase 1 clinical development, as well as a cyclophilin inhibitor, EDP-494, a novel host-targeting mechanism for HCV, which is also in Phase 1 clinical development. In addition, Enanta has early lead candidates for HBV and RSV in preclinical development. Please visit www.enanta.com for more information on Enanta's programs and pipeline.

Forward Looking Statements Disclaimer
This press release contains forward-looking statements, including statements with respect to the prospects for Enanta's further development of EDP-305. 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 new disease areas such as NASH; the impact of development, regulatory and marketing efforts of others with respect to competitive treatments for NASH; regulatory and reimbursement actions affecting any competitive treatment 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-K for the fiscal year ended September 30, 2016 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.

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[1] http://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm

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Source: Enanta Pharmaceuticals, Inc.

Investor Contact
Enanta Pharmaceuticals, Inc.
Carol Miceli, 617-607-0710
cmiceli@enanta.com
or
Media Contact
MacDougall Biomedical Communications
Kari Watson, 781-235-3060
kwatson@macbiocom.com