Enanta Pharmaceuticals Initiates Phase 1 Clinical Study of EDP-305, its Lead FXR Agonist for the Treatment of Non-alcoholic Steatohepatitis (NASH)

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- *First subjects dosed in study that will evaluate EDP-305 in healthy subjects and subjects with presumptive non-alcoholic fatty liver disease (NAFLD)*

WATERTOWN, Mass.--(BUSINESS WIRE)--Sep. 28, 2016-- 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 it has initiated a Phase 1 clinical study and has begun dosing healthy adults with EDP-305, Enanta's lead farnesoid X receptor (FXR) agonist under development to treat patients with NASH.

The 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 NAFLD (obese, with or without pre-diabetes or type 2 diabetes mellitus). The study will enroll approximately 90 subjects and is designed to evaluate up to 5 dose cohorts, with EDP-305 administered orally, once daily.

The current study will include 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 bio-markers 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.

"This expanded Phase 1 study design was driven by extensive preclinical data that demonstrate that EDP-305 is a highly selective FXR agonist that shows potent activity in a variety of *in vitro* assays and *in vivo* NASH and fibrosis models," commented Jay R. Luly, Ph.D. "As part of a more complete characterization of EDP-305, we expect to share data regarding fibrosis and other preclinical data in November at The Liver Meeting® in Boston."

About NAFLD, NASH, and FXR
Non-alcoholic fatty liver disease (NAFLD) is the accumulation in patients of excessive fat in the form of triglycerides in 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 has liver cell injury and inflammation in addition to excessive fat (steatohepatitis). Progression of this condition leads to non-alcoholic steatohepatitis (NASH). Patients with NASH can develop fibrosis 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 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. 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 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), Hepatitis B Virus (HBV), Non-alcoholic Steatohepatitis (NASH) and Respiratory Syncytial Virus (RSV).

Enanta has discovered novel protease inhibitors and NS5A inhibitors that are members of the direct-acting-antiviral (DAA) inhibitor classes designed for use against the hepatitis C virus (HCV). Enanta's protease inhibitors, developed through its collaboration with AbbVie, include paritaprevir, which is contained in AbbVie's marketed DAA regimens for HCV, and ABT-493, Enanta's second protease inhibitor, which AbbVie is developing in Phase 3 studies in combination with ABT-530, AbbVie's NS5A inhibitor. Enanta has also discovered a cyclophilin inhibitor, EDP-494, a novel host-targeting mechanism for HCV, which is now in a clinical proof of concept study in HCV patients, and EDP-305, a non-bile acid FXR agonist for NASH, currently in Phase 1 clinical development. Please visit www.enanta.com for more information on our programs and pipeline.

Forward Looking Statements Disclaimer
This press release contains forward-looking statements, including statements with respect to the prospects for clinical development of one of Enanta's early lead compounds for the treatment of 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 disease areas such as NASH that have no current therapeutic treatment; potential competition from the development efforts of others in this disease area; Enanta's lack of 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, 2015 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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