



## Enanta Announces New Data on FXR Agonist EDP-305 for Non-Alcoholic Steatohepatitis (NASH) at The Liver Meeting® 2016

November 11, 2016

- **Data support the ongoing development of EDP-305 for the treatment of NASH**

WATERTOWN, Mass.--(BUSINESS WIRE)--Nov. 11, 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 new preclinical data from Enanta's wholly-owned program in non-alcoholic steatohepatitis (NASH) at the Liver Meeting®, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD) taking place November 11-15, 2016 in Boston.

New data is being presented on Enanta's Farnesoid X Receptor (FXR) agonist EDP-305, demonstrating its potency, selectivity and effects on fibrosis progression and lipid metabolism in pre-clinical models. Characterization of a new FXR preclinical lead compound with enhanced potency, EP-024297, will also be presented.

Detailed data will be available during each poster session.

### Poster Presentations:

#### EDP-305 and EP-024297 FXR Agonists for Non-Alcoholic Steatohepatitis (NASH)

##### November 13, 8:00 am – 5:30 pm ET

#650 - The Novel Farnesoid X Receptor (FXR) agonist, EDP-305, Reduces Fibrosis Progression in Bile Duct Ligated Rats (C. Farrar, *et al.*)

- Data demonstrated that EDP-305 reduced liver fibrosis in CDAHFD and BDL rodent models of NASH.

#1540 (*Poster of Distinction*) - EDP-305, A Novel and Selective Farnesoid X Receptor Agonist, Exhibits High Potency and Efficacy *In Vitro* and *In Vivo* (Y. Li, *et al.*)

- Data demonstrated that EDP-305, a highly specific FXR agonist with minimal activity against TGR5, has potent effects on FXR-dependent gene expression *in vitro* and *in vivo*.

#1568 - EDP-305, A Novel and Highly Potent Farnesoid X Receptor Agonist, Exerts Favorable Effects on Lipid Metabolism *In Vitro* (Y. Li, *et al.*)

- Data demonstrated that EDP-305 may have the potential to increase LDL clearance via up-regulation of LDLr and may potentially reduce HDL degradation by down-regulating the expression of hepatic lipase.

#1596 - EDP-305, A Novel and Potent Farnesoid X Receptor Agonist, Exhibits Favorable Anti-inflammatory and Anti-fibrotic Activity *In Vitro* (Y. Li, *et al.*)

- Data demonstrated that EDP-305 exhibits the ability to regulate key genes involved in inflammation and fibrosis.

##### November 11, 8:00 am – 5:30 pm ET

#1569 - EP-024297, A Novel and Selective Farnesoid X Receptor Agonist, Exhibits High Potency and Efficacy *In Vitro* and *In Vivo* (M. Chau, *et al.*)

- Data demonstrated that EP-024297 is a sub-nanomolar agonist of FXR, and can regulate FXR-dependent gene expression *in vivo* at dose levels as low as 0.1 mg/kg.

"The additional preclinical data sets being presented at The Liver Meeting further demonstrate that EDP-305 is a highly selective FXR agonist with potent activity in a variety of *in vitro* assays and *in vivo* NASH models and fibrosis models," commented Jay R. Luly, Ph.D. "Our aim is to develop superior compounds to treat this debilitating disease, and we look forward to data from our ongoing clinical development program."

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 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 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. 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 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 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 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 AbbVie has developed in Phase 3 studies in a fixed-dose combination (G/P) with pibrentasvir (ABT-530), AbbVie's second NS5A inhibitor, and is preparing for regulatory approval filings in the U.S., Europe and Japan.

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](http://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 Enanta's further development of EDP-305 other FXR agonists and their potential effects in contrast to potential NASH treatments being developed by others. 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 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 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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