

Enanta Pharmaceuticals Initiates Proof-of-Concept Study with Pan-genotypic Cyclophilin Inhibitor EDP-494 in Patients with Genotype 1 or Genotype 3 Chronic Hepatitis C Virus

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- *EDP-494 represents a new host-targeted approach that may play an important role in future treatment regimens for chronic hepatitis C virus (HCV) patients that have failed therapy due to resistance arising from direct-acting antiviral (DAA) therapies currently on the market*
- *New non-DAA mechanism retains potency against important DAA resistance-associated variants (RAVs), regardless of class*

WATERTOWN, Mass.--(BUSINESS WIRE)--Jun. 20, 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 proof of concept study (POC) and has begun dosing with EDP-494, a potent, pan-genotypic cyclophilin inhibitor to treat patients with genotype 1 (GT1) or genotype 3 (GT3) chronic hepatitis C virus.

The double-blind, randomized POC portion of this ongoing phase 1 study will assess the safety, pharmacokinetics and anti-viral activity of two planned different doses of EDP-494 in treatment-naïve HCV patients dosed orally, once daily for 14 days. Safety and pharmacokinetics of single and multiple ascending doses of once daily, orally administered EDP-494 have been established in healthy volunteers and these data support the testing of EDP-494 in a proof-of-concept study in HCV patients.

EDP-494, a Cyclophilin Inhibitor for HCV Infection

Due to resistance arising with direct-acting antiviral HCV therapies currently on the market, Enanta has developed an alternative treatment approach to HCV that targets the human host protein cyclophilin, which is essential for replication of HCV. Since cyclophilin inhibitors act as host-targeted antivirals, the viral mutation resistance that arises from direct-acting antiviral (DAA) treatments would not be expected for this mechanism, and thus cyclophilin inhibitors may have the highest barrier to resistance of any class of HCV treatments.

“As the number of patients treated with the current HCV DAA regimens on the market rapidly increases, the treatment failure population will become an important unmet medical need in patients with hepatitis C and may require new mechanisms of therapy such as cyclophilin inhibition,” commented Professor Edward J. Gane, MD, Professor of Medicine at the University of Auckland, New Zealand and Chief Hepatologist, Transplant Physician and Deputy Director of the New Zealand Liver Transplant Unit at Auckland City Hospital and chief investigator of the EDP-494 study. “This host-targeted approach may prove valuable not only in the toughest to treat patients with DAA resistance, but also in the general HCV patient population, given its pangenotypic mechanism of action and high barrier to resistance.”

Data presented at the International Liver Congress™¹ in Barcelona in April, 2016 demonstrated that EDP-494 in replicon assays is a potent inhibitor of HCV replication and has shown no loss of potency against all major NS5A RAVs, NS5B RAVs (both Nucleotide and non-Nucleotide) as well as NS3 protease inhibitor RAVs.

Following the completion of the phase 1 study, Enanta plans to develop EDP-494 in combination with one or more DAAs for the treatment of any emerging HCV resistance to currently marketed therapies or any other therapies in development that use DAAs. Enanta anticipates that a DAA-cyclophilin inhibitor combination could provide one of the highest barrier to resistance combination treatments for HCV.

About EDP-494

EDP-494 is a cyclophilin inhibitor that targets the human host protein, cyclophilin, which is essential for replication of

chronic hepatitis C virus (HCV). Host-targeted antivirals (HTAs) such as cyclophilin inhibitors are not affected by changes in the virus and, therefore, use of this class of inhibitor may provide a unique solution for a subset of hard-to-treat HCV patients. Enanta has demonstrated in replicon assays that EDP-494 is a potent inhibitor of HCV. A phase 1 clinical study in HCV patients is currently underway.

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 phase 1 clinical development, and EDP-305, an FXR agonist, which Enanta plans to advance into clinical development for NASH later in 2016. 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 potential market for patients with DAA-resistant HCV and the prospects for further clinical development of EDP-494 for the treatment of HCV and its RAVs. 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 clinical trials of EDP-494; regulatory actions affecting approvals of new HCV treatment regimens after recent approvals of new regimens and anticipated submissions and approvals of other regimens currently in late stage clinical development; the pricing, market acceptance and reimbursement rates of competitive HCV treatment regimens on the market and product candidates of other companies under development; 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.

¹ P-250. C. Owens et.al., "EDP-494 is a Potent Pan-Genotypic Cyclophilin Inhibitor for HCV Infection, Including DAA Resistance Associated Variance (DAAs)". The 51st Annual Meeting of the European Association for the Study of the Liver, The International Liver Congress™, April 13-17, 2016 Barcelona, Spain.

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