



Enanta Pharmaceuticals Announces Results from Four All-Oral, Interferon-Free, Phase 3 Studies for the Treatment of Genotype 1 Hepatitis C Virus Infection

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- Results confirm previously reported data in genotype 1 (GT1) patient populations demonstrating high sustained virologic response rates at 12 weeks post-treatment (SVR₁₂)
- Results demonstrate SVR₁₂ of 97 to 100% in GT1b patients
- SVR₁₂ of 92 to 96% was shown in the difficult-to-treat GT1 cirrhotic patient population
- AbbVie announced it expects U.S. launch in 2014

WATERTOWN, Mass.--(BUSINESS WIRE)--Jan. 31, 2014-- Enanta Pharmaceuticals, Inc. (NASDAQ:ENTA) today announced results from the PEARL-II, PEARL-III, PEARL-IV and TURQUOISE-II studies. These studies are the remaining four phase 3 studies of the six phase 3 registrational studies being conducted by AbbVie for the treatment of genotype 1 (GT1) hepatitis C virus (HCV) infection using a regimen containing Enanta's lead protease inhibitor ABT-450. ABT-450 is part of AbbVie's investigational three direct-acting antiviral regimen consisting of boosted protease inhibitor ABT-450/ritonavir, NS5A inhibitor ABT-267, and non-nucleoside polymerase inhibitor ABT-333. These studies were conducted with and without ribavirin. The combination of the three different mechanisms of action in this regimen interrupts the HCV replication process with the goal of optimizing SVR rates across different patient populations.

Results from these studies demonstrate high sustained virologic response rates 12 weeks post treatment (SVR₁₂) and tolerability in these GT1 patients and low rates of discontinuation due to adverse events.

A summary of AbbVie's Phase 3 clinical trial results for the 3D regimen consisting of ABT-450/ritonavir, ABT-267 and ABT-333 follows:

Study	Patient Type (number)	Treatment Regimen	Treatment Duration	SVR ₁₂
PEARL-II	GT1b treatment-experienced (n=179)	• 3D regimen (n=91)	12 week	100%
		• 3D regimen with ribavirin (n=88)	12 weeks	97%
PEARL-III	GT1b, treatment-naïve (n=419)	• 3D regimen (n=209)	12 weeks	99%
		• 3D regimen with ribavirin (n=210)	12 weeks	99%
PEARL-IV	GT1a, treatment-naïve (n=305)	• 3D regimen (n=205)	12 weeks	90%
		• 3D regimen with ribavirin (n=100)	12 weeks	97%
TURQUOISE-II	GT1 treatment-naïve and treatment-experienced with compensated cirrhosis (n=380)	• 3D regimen with ribavirin (n=208)	12 weeks	92%
		• 3D regimen with ribavirin (n=172)	24 weeks	96%

SAPPHIRE-I	GT1 treatment-naïve (n=631)	<ul style="list-style-type: none"> • 3D regimen with ribavirin (n=473) 	12 weeks	96%
SAPPHIRE-II	GT1 treatment-experienced (n=394)	<ul style="list-style-type: none"> • 3D regimen with ribavirin (n=297) 	12 weeks	96%

Overall, across the four studies, the three direct-acting antiviral regimen was well tolerated with few adverse event-related discontinuations. The most commonly reported adverse events in PEARL-II and PEARL-III were fatigue and headache. In PEARL-IV and TURQUOISE-II, the most commonly reported adverse events were fatigue, headache and nausea.

“We are pleased that SVR rates continue to be high in both treatment-naïve and treatment-experienced GT1 HCV patients with and without ribavirin, as well as in the difficult-to-treat compensated cirrhotic patients. In addition, these trials demonstrate the exceptional tolerability of the regimen, with less than one percent (0.8%) of patients discontinuing therapy due to adverse events,” said Jay R. Luly, Ph.D. President and CEO. “We are also pleased that AbbVie has announced it is on track to begin major regulatory submissions early in the second quarter of 2014.”

These six phase 3 trials included 2,308 patients from more than 25 countries around the world. This is the only registrational program to include a dedicated study of an all-oral regimen in patients with compensated cirrhosis. In May 2013, AbbVie’s three direct-acting antiviral regimen with and without ribavirin for GT1 HCV was designated as a Breakthrough Therapy by the U.S. Food and Drug Administration (FDA). AbbVie has stated that it intends to disclose detailed study results at future scientific congresses and in publications.

About Study M13-389 (PEARL-II)

PEARL-II is a global, multi-center, randomized, open-label, controlled study to evaluate the efficacy and safety of 12 weeks of treatment with the three direct-acting antiviral regimen with and without ribavirin in non-cirrhotic, GT1b HCV-infected, treatment-experienced adult patients.

The study population consisted of 179 GT1b treatment-experienced patients with no evidence of liver cirrhosis: 91 patients were randomized to the regimen without ribavirin for 12 weeks, and 88 patients were randomized to the regimen plus ribavirin for 12 weeks. In the ribavirin-free arm, 100 percent (n=91/91) of patients achieved SVR₁₂, while 97 percent (n=85/88) achieved SVR₁₂ in the ribavirin-containing arm.

The most commonly reported adverse events were fatigue and headache. Discontinuations due to adverse events were reported in none of the patients in the ribavirin-free arm and two (2 percent) patients in the ribavirin-containing arm. There were no patients in either arm of the study that experienced virologic relapse or breakthrough.

About Study M13-961 (PEARL-III)

PEARL-III is a global, multi-center, randomized, double-blind, controlled study to evaluate the efficacy and safety of 12 weeks of treatment with the three direct-acting antiviral regimen with and without ribavirin in non-cirrhotic, GT1b HCV-infected, treatment-naïve adult patients.

The study population consisted of 419 GT1b treatment-naïve patients with no evidence of liver cirrhosis; 209 patients were randomized to the regimen without ribavirin for 12 weeks, and 210 patients were randomized to the regimen plus ribavirin for 12 weeks. Following 12 weeks of treatment, 99 percent receiving the regimen without ribavirin (n=207/209) and 99 percent receiving the regimen plus ribavirin (n=209/210) achieved SVR₁₂.

The most commonly reported adverse events were fatigue and headache. No patient discontinued study drug due to adverse events. Virologic relapse or breakthrough was noted in none of the patients receiving the regimen without ribavirin and 0.5 percent of patients receiving the regimen plus ribavirin.

About Study M14-002 (PEARL-IV)

PEARL-IV is a global, multi-center, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of 12 weeks of treatment with the three direct-acting antiviral regimen with and without ribavirin in non-cirrhotic, GT1a HCV-infected, treatment-naïve adult patients.

The study population consisted of 305 GT1a treatment-naïve patients with no evidence of liver cirrhosis; 205 patients were randomized to the regimen without ribavirin for 12 weeks, and 100 patients were randomized to the regimen with ribavirin for 12 weeks. Following 12 weeks of treatment, 90 percent of patients receiving the regimen without ribavirin (n=185/205) and 97 percent receiving the regimen plus ribavirin (n=97/100) achieved SVR₁₂.

The most commonly reported adverse events were fatigue, headache and nausea. Discontinuations due to adverse events were reported in two (1 percent) patients receiving the regimen without ribavirin and no patients in the ribavirin-containing arm. Virologic relapse or breakthrough was noted in 8 percent of patients receiving the regimen without ribavirin and 2 percent of patients receiving the regimen with ribavirin.

About Study M13-099 (TURQUOISE-II)

TURQUOISE-II is the first phase III study completed exclusively in GT1 cirrhotic patients investigating an all-oral interferon-free regimen. It is a global, multi-center, randomized, open-label study evaluating the efficacy and safety of 12 or 24 weeks of treatment with the three direct-acting antiviral regimen with ribavirin in cirrhotic, GT1a and GT1b HCV-infected, treatment-naïve and treatment-experienced adult patients.

The study population consisted of 380 GT1a and GT1b, treatment-naïve and treatment-experienced patients with compensated cirrhosis; 208 patients were randomized to the regimen plus ribavirin for 12 weeks, and 172 patients were randomized to the regimen plus ribavirin for 24 weeks. Following 12 weeks of treatment, 92 percent of patients (n=191/208) achieved SVR₁₂. Following 24 weeks of treatment, 96 percent of patients (n=165/172) achieved SVR₁₂.

The most commonly reported adverse events were fatigue, headache and nausea. Discontinuations due to adverse events were reported in four (2 percent) patients receiving the regimen with ribavirin for 12 weeks and four (2 percent) patients in the 24-week arm. Virologic relapse or breakthrough was noted in 6 percent of patients in the 12-week arm and 2 percent in the 24-week arm.

Additional information about AbbVie's phase 3 studies can be found on www.clinicaltrials.gov.

Protease Inhibitor Collaboration with AbbVie (formerly the research-based pharmaceutical business of Abbott Laboratories)

In December 2006, Enanta and Abbott announced a worldwide agreement to collaborate on the discovery, development and commercialization of HCV NS3 and NS3/4A protease inhibitors and HCV protease inhibitor-containing drug combinations. ABT-450 is a protease inhibitor identified as a lead compound through the collaboration. Under the agreement, AbbVie is responsible for all development and commercialization activities for ABT-450. Enanta received \$57 million in connection with signing the collaboration agreement, has received \$55 million in subsequent clinical milestone payments, and is eligible to receive an additional \$195 million in payments for regulatory milestones, as well as double-digit royalties worldwide on any revenue allocable to the collaboration's protease inhibitors. Also, for any additional collaborative HCV protease inhibitor product candidate developed under the agreement, Enanta holds an option to modify the U.S. portion of its rights to receive milestone payments and worldwide royalties. With this option, Enanta can fund 40 percent of U.S. development costs and U.S. commercialization efforts (sales and promotion costs) for the additional protease inhibitor in exchange for 40 percent of any U.S. profits ultimately achieved after regulatory approval, instead of receiving payments for U.S. commercial regulatory approval milestones and royalties on U.S. sales of that protease inhibitor.

About Hepatitis C Virus (HCV)

Hepatitis C is a liver disease affecting over 170 million people worldwide. The virus is typically spread through direct contact with the blood of an infected person. Hepatitis C increases a person's risk of developing chronic liver disease, cirrhosis, liver cancer and death. Patients with compensated cirrhosis have a liver that is heavily scarred but that can still perform many important bodily functions with few or no symptoms. There is an acute need for new HCV therapies that are safer and more effective for many variants of the virus.

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 in the infectious disease field. Enanta is discovering, and in some cases developing, novel inhibitors designed for use against the hepatitis C virus (HCV). These inhibitors include members of the direct acting antiviral (DAA) inhibitor classes – protease (partnered with AbbVie), NS5A (partnered with Novartis) and nucleotide polymerase – as well as a host-targeted antiviral (HTA) inhibitor class targeted against cyclophilin. Additionally, Enanta has created a new class of antibiotics, called Bicyclolides, for the treatment of multi-drug resistant bacteria, with a focus on developing an intravenous and oral treatment for hospital and community MRSA (methicillin-resistant *Staphylococcus aureus*) infections.

Forward Looking Statements Disclaimer

This press release contains forward-looking statements, including with respect to the clinical data and the planned regulatory submissions for the three direct-acting antiviral HCV treatment regimen containing ABT-450. Statements that are not historical facts are based on our management's current expectations, estimates, forecasts and projections about our business and the industry in which we operate and our 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 that may affect actual results include final results of ongoing clinical trials of the three direct-acting antiviral ABT-450-containing regimen, the development, regulatory and marketing efforts of AbbVie (our collaborator on ABT-450), clinical development of competitive product candidates, regulatory submissions by AbbVie and its competitors in HCV and regulatory actions affecting the three direct-acting antiviral ABT-450-containing regimen and competitive regimens. 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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