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Interim Results from Study 107 Highlight the Potential Role of Telaprevir-Based Regimens in HCV Patients Who Failed Prior Treatment

- ***57% of prior treatment null responder patients achieved an SVR with a 48-week telaprevir-based regimen***
- ***90% of prior treatment relapsers and 55% of prior treatment partial responders achieved an SVR with 24-week or 48-week telaprevir-based regimens***
- ***Results provide further support for the ongoing Phase 3 registration study, REALIZE, in treatment-failure patients***

CAMBRIDGE, Mass., Oct 28, 2009 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that treatment with telaprevir-based regimens in Study 107 resulted in high sustained viral response (SVR) rates in treatment-failure patients infected with hepatitis C virus (HCV). Interim results from 94 patients in Study 107 showed that 90% of prior treatment relapsers (n=29) and 55% of prior treatment partial responders (n=29) achieved an SVR after treatment with 24-week or 48-week telaprevir-based regimens, and 57% of prior treatment null responders (n=28) achieved an SVR after treatment with a 48-week telaprevir-based regimen. At the time of this interim analysis, eight patients (7%, n=117) had discontinued all therapy due to adverse events, which included 4 patients who discontinued due to rash and 1 patient who discontinued due to anemia.

Study 107 is an ongoing, open-label Phase 2 rollover study of patients who had received pegylated interferon (peg-IFN) and ribavirin (RBV) in the control arms of the Phase 2 PROVE studies and who did not achieve an SVR. Telaprevir is an investigational HCV protease inhibitor being developed by Vertex Pharmaceuticals in collaboration with Tibotec and Mitsubishi Tanabe Pharma.

"Results that include SVR rates of 57% in the difficult-to-treat null responder population are important for prior treatment-failure patients who have limited treatment options," said Peter Mueller, Ph.D., Vertex's Executive Vice President, Global Research and Development and Chief Scientific Officer. "These results, as well as data from other telaprevir studies, continue to support the potential role of telaprevir as a new treatment option for hepatitis C patients."

Patient Type	TVR regimen	SVR	Viral Relapse Rates
Prior Null Responders*	T12 + PR 48 (n=28)	57% (16/28)	20% (4/20)
Prior Partial Responders**	T12 + PR24 (n=25) or T12 + PR48 (n=3) or Unassigned (n=1)	55% (16/29)	22% (5/23)
Prior Relapsers^{+}	T12 + PR24 (n= 25) or T12 + PR48 (n=3) or Unassigned (n=1)	90% (26/29)	4% (1/28)
Prior Viral Breakthrough$^{\pm}$	T12 + PR24 (n=7) or T12 + PR48 (n=1)	75% (6/8)	0 (0/6)

** Defined as patients who achieved a viral load decline of less than 1 $\log_{10}$ at week 4 or 2 $\log_{10}$ or less at week 12 during prior therapy*
***Defined as patients who had a greater than 2 $\log_{10}$ viral decline at week 12 but had detectable HCV RNA at week 24*

$^{+}$Defined as patients who had undetectable HCV RNA at the end of treatment with peg-IFN and RBV but subsequently relapsed

$^{\pm}$Defined as patients who had undetectable HCV RNA but relapsed before the end of treatment
Undetectable HCV RNA is defined as <25IU/mL undetectable by Roche COBAS Taqman HCV test
This analysis includes 1 prior partial responder and 1 prior relapser who discontinued all treatment prior to reaching week 12 of dosing and subsequently achieved an SVR - designated as "unassigned"

Interim Analysis of Study 107

Discontinuation of all therapy due to adverse events occurred in eight patients. A complete safety analysis is still being performed and will be reported when the full data are presented at a medical conference expected in 2010.

"These results, which suggest that 48-week telaprevir-based regimens may help to optimize outcomes for treatment-failure patients, support the rationale for many of the key design elements of the ongoing Phase 3 trial, REALIZE, being conducted by our partner, Tibotec," said Dr. Robert Kauffman, Vertex's Senior Vice President, Clinical Development and Chief Medical Officer. "In Study 107 we observed patterns of response across the treatment-failure population, and made amendments during the course of the study in an effort to optimize treatment. The SVR rates reported here are part of our effort to identify the most appropriate regimen for this difficult-to-treat population and to show that 48-week regimens may further enhance SVR rates for certain treatment-failure patients."

Study 107 Design and Interim Results

Study 107 is an ongoing, open-label Phase 2 rollover study of telaprevir in combination with peg-IFN and RBV in patients who had previously enrolled in the control arms of either of the PROVE 1, PROVE 2 or PROVE 3 studies, and did not achieve an SVR. Patients in Study 107 were well-characterized as null responders, partial responders, relapsers or breakthroughs, based on their antiviral response to prior peg-IFN and RBV treatment as a result of their participation in the control arms of prior PROVE clinical trials. This interim analysis, which includes all but two patients who are still on treatment in the study, was conducted to support planned regulatory agency interactions in Europe. Final analysis from Study 107 is planned for presentation at a medical conference in 2010.

The interim results reported here reflect data from patients who were considered to have received an appropriate treatment regimen based on their prior response to peg-IFN and RBV and reflecting the amendments made during the conduct of Study 107. When Study 107 began, all patients were to receive 12 weeks of telaprevir in combination with peg-IFN and RBV followed by an additional 12 weeks of peg-IFN and RBV (T12/PR24). Stopping rules required any patient who did not achieve undetectable HCV RNA by week 4 to stop all treatment. In 2008, Study 107 was amended and underwent several changes. The most important change to the protocol was to the week 4 stopping rules for virologic failure, as it became evident that treatment-failure patients had a somewhat slower viral response to treatment than did naïve patients. Following this amendment, patients who had detectable HCV RNA at week 4 were permitted to continue therapy. Additionally, while the initial study protocol specified 24 weeks of total treatment for all patients, a longer total duration of treatment (48 weeks) was determined to be warranted in prior null responders to provide a higher likelihood of achieving an SVR.

From a total of 117 patients who enrolled in Study 107, data from 94 patients were included in this interim analysis. The 23 patients not included in this interim analysis were null responders, who, prior to protocol amendments, were designated to receive only 24 weeks of therapy, and a portion of whom met the strict stopping rule criteria at week 4. The 48-week telaprevir-based regimen is being studied in the ongoing Phase 3 trial, known as REALIZE, in treatment-failure patients. Final data will be presented at a medical conference in 2010.

About Telaprevir

Telaprevir (VX-950) is an investigational oral inhibitor of HCV protease, an enzyme essential for viral replication, and is one of the most advanced investigational antiviral agents in development that specifically targets HCV. Telaprevir is being evaluated as part of a global Phase 3 registration program in more than 2,200 treatment-naïve and treatment-failure patients.

Vertex is collaborating with Tibotec and Mitsubishi to develop telaprevir. Vertex retains commercial rights to telaprevir in North America. Tibotec has rights to commercialize telaprevir in Europe, South America, Australia, the Middle East and other countries. Mitsubishi Tanabe Pharma has rights to commercialize telaprevir in Japan and certain Far East countries.

About Hepatitis C

Hepatitis C is a liver disease caused by the hepatitis C virus, which is found in the blood of people with the disease. HCV, a serious public health concern affecting 3.2 million individuals in the United States, is spread through direct contact with the blood of infected people.[1] Though many people with HCV infection may not experience symptoms, others may have symptoms such as jaundice, abdominal pain, fatigue and fever.[1] Chronic HCV significantly increases a person's risk for developing long-term infection, chronic liver disease, cirrhosis or death.[1]

Current therapies for HCV typically provide sustained benefit in about half of patients with genotype 1 HCV, the most common strain of the virus.[2] If treatment is not successful patients remain at risk for progressive liver disease.[1] In a recent study, the risk of liver failure, cancer or death following unsuccessful HCV treatment was assessed at 23% after 4 years, and 43% after 8 years.[3]

About Vertex

Vertex Pharmaceuticals Incorporated is a global biotechnology company committed to the discovery and development of breakthrough small molecule drugs for serious diseases. The Company's strategy is to commercialize its products both independently and in collaboration with major pharmaceutical companies. Vertex's product pipeline is focused on viral diseases, cystic fibrosis, inflammation, autoimmune diseases, cancer, and pain. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

Lexiva(R) is a registered trademark of the GlaxoSmithKline group of companies.

[1]Centers for Disease Control and Prevention. Hepatitis C Fact Sheet: CDC Viral Hepatitis. Available at: http://www.cdc.gov/hepatitis/HCV/PDFs/HepCGeneralFactSheet.pdf. Accessed, September 24, 2009.

[2] Strader DB, Wright T, Thomas DL, Seeff LB, AASLD practice guideline: diagnosis, management and treatment of hepatitis C. Hepatology: 2004(39):1147-1171.

[3]Veldt et al, "Sustained virologic response and clinical outcomes in patients with chronic hepatitis C and advanced fibrosis," Annals of Internal Medicine, 20 November 2007; 147: 677-684.

Special Note Regarding Forward Looking Statements

This press release contains forward-looking statements, including statements regarding (i) interim results from Study 107 highlighting the potential role of telaprevir-based regimens in HCV patients who failed prior treatment; (ii) interim results from Study 107 providing further support for the ongoing Phase 3 registration study, REALIZE, in treatment-failure patients; (iii) the results from this study being important for prior treatment-failure patients, who have limited treatment options; (iv) the results continuing to support the potential role of telaprevir as a new treatment option for hepatitis C patients; (v) the results, which suggest that 48-week telaprevir-based regimens may help to optimize outcomes for treatment-failure patients, supporting the rationale for many of the key design elements of the ongoing Phase 3 trial, REALIZE, being conducted by our partner, Tibotec; (vi) SVR rates reported here being part of our effort to identify the most appropriate regimen for this difficult-to-treat population and showing that 48-week regimens may further enhance SVR rates for certain treatment-failure patients; and (vii) a final analysis from Study 107 being planned for presentation at a medical conference in 2010. While the Company believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the outcomes for each of its clinical trials of telaprevir (including the ongoing Phase 3 clinical trials) may not be favorable or may be less favorable than the outcomes obtained from earlier studies including the 107 Study, that there may be varying interpretations of data produced by one or more of the Company's clinical trials, that regulatory authorities will require more extensive data for a telaprevir NDA filing than currently expected, that future competitive or other market factors may adversely affect the commercial potential for the Company's product candidates and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the Company's website at www.vrtx.com. The Company disclaims any obligation to update the information contained in this press release as new information becomes available.

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