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New England Journal of Medicine Publishes Landmark Clinical Studies of the Investigational Hepatitis C Virus Protease Inhibitor Telaprevir

-Addition of telaprevir to standard HCV therapies significantly improved rates of sustained viral response in half the time of current treatments--SVR rates in Phase 2b studies of up to 69% with 24 weeks of telaprevir-based treatment--Phase 3 program ongoing in more than 2,200 patients-

CAMBRIDGE, Mass., Apr 29, 2009 (BUSINESS WIRE) -- Two clinical studies published in this week's *New England Journal of Medicine* demonstrate that treatment with the investigational oral hepatitis C virus (HCV) protease inhibitor telaprevir dosed in combination with pegylated-interferon (peg-IFN) and ribavirin (RBV) as part of a 24-week treatment regimen resulted in a significant improvement in the rate of sustained viral response (SVR), considered a cure of the viral infection, in treatment-naïve genotype 1 HCV patients, as compared with the SVR rate for standard therapy dosed for 48 weeks. The data are from two Phase 2b (mid-stage) clinical trials of telaprevir known as PROVE 1 and PROVE 2. In these trials, patients who received a 24-week telaprevir-based treatment regimen achieved SVR rates of up to 69 percent, as compared to SVR rates of up to 46 percent in patients in the control arms of these trials who received peg-IFN and RBV for a standard duration of 48 weeks. Telaprevir is being developed by Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) in collaboration with Tibotec and Mitsubishi Tanabe Pharma. Telaprevir is currently in Phase 3 (late-stage) clinical development.

HCV is the most common blood-borne infection in the U.S., four times more common than HIV infection, and is the leading cause of liver transplantations and liver cancer in the U.S.

"Currently available therapies for patients infected with HCV can be difficult to tolerate and less than half the patients who start the yearlong treatment regimen achieve the ultimate goal of having an undetectable level of virus in their bodies," said John McHutchison, M.D., Lead Investigator for the PROVE 1 trial and Associate Director of the Duke Clinical Research Institute. "In these Phase 2 clinical trials, up to 69 percent of patients in the 24-week telaprevir-based treatment arm had undetectable virus levels after 24 weeks, and even though telaprevir does produce side effects of its own, its addition to standard therapy allowed us to shorten the duration of treatment. This 24-week regimen was half the duration of currently approved therapies and, if confirmed to be this effective in larger Phase 3 studies, could one day become a very important treatment option for hepatitis C patients."

"In the PROVE 1 and PROVE 2 trials, telaprevir significantly improved the proportion of patients who were cured of their disease and also shortened the duration of HCV therapy from 48 to 24 weeks for the majority of treatment-naïve patients - an exciting achievement and a potentially meaningful advance in the treatment of this disease," said Robert Kauffman, M.D., Ph.D., Senior Vice President of Clinical Development for Vertex. "Based on data from these trials, as well as from the PROVE 3 trial in patients who failed prior HCV therapy, telaprevir is being evaluated in a comprehensive Phase 3 registration program in more than 2,200 treatment-naïve and treatment-failure patients. Assuming successful completion of this program, we expect to file an application for approval of telaprevir with the U.S. FDA in the second half of 2010."

PROVE 1 and PROVE 2 Study Results

The primary endpoint of the PROVE 1 and PROVE 2 trials was the proportion of patients who had no detectable hepatitis C virus in their blood (undetectable plasma HCV RNA) 24 weeks after the completion of therapy, also known as a sustained viral response (SVR). Patients who achieve an SVR are considered to be cured of their HCV infection.

Final results from the PROVE 1 and PROVE 2 trials showed that the 24-week treatment arm, which consisted of 12 weeks of telaprevir dosed in combination with peg-IFN and RBV followed by an additional 12 weeks of peg-IFN and RBV alone, resulted in a significant improvement in SVR rates, an increased rate of rapid virologic response (RVR, defined as undetectable levels of HCV RNA by the end of week 4) and low rates of viral relapse (defined in patients as undetectable HCV RNA at the end of treatment but detectable viral levels during the post-treatment follow-up period), as compared with the SVR, RVR and relapse rates observed in patients in the control arms who received peg-IFN and RBV for 48 weeks. Final data are outlined in the table below:

Final PROVE 1 & PROVE 2 Results	**SVR Rate**	**RVR Rate**	**Relapse Rate***
PROVE 1			

	61%	81%	2%
24-Week Telaprevir-Based Regimen PROVE 1			
	41%	11%	23%
Control Arm PROVE 2			
	69%	69%	14%
24-Week Telaprevir-Based Regimen PROVE 2			
	46%	13%	22%
Control Arm			

* The relapse rate in the 24-week telaprevir-based treatment arm of PROVE 1 reflects only those patients who achieved RVR and remained undetectable through week 20. For patients in the 24-week telaprevir-based treatment arm of PROVE 2 who achieved RVR and remained undetectable through week 12 the relapse rate was 7%.

Of the small sub-group of African American patients enrolled in PROVE 1, 44 percent achieved an SVR in the telaprevir arms, while 11 percent achieved an SVR in the control group. SVR rates in African Americans are typically lower than in other ethnic groups. African Americans are also disproportionately infected with HCV as compared to other ethnic groups.

Together, these data suggest that a 24-week telaprevir-based treatment regimen may be sufficient for treatment-naïve patients who achieve an RVR. These findings are being confirmed in Vertex's ADVANCE Phase 3 clinical trial in treatment-naïve patients, focusing on 24-week response-guided regimens that consist of either 8 or 12 weeks of telaprevir in combination with peg-IFN and RBV. Treatment-naïve patients in the ADVANCE Phase 3 trial who achieve an RVR and who stay undetectable through week 12 of treatment will receive 24 weeks of treatment. Patients who do not meet the RVR criteria but are undetectable at week 24 will continue on peg-IFN and RBV for a total duration of 48 weeks. This Phase 3 trial is designed to maximize the number of patients who can achieve SVR while offering a large proportion of treatment-naïve patients the benefit of a 24-week treatment duration.

Telaprevir Safety & Tolerability Across PROVE 1 and PROVE 2

More than 400 patients received a telaprevir-containing regimen as part of the PROVE 1 and PROVE 2 clinical trials, and the adverse event profile was generally consistent across these trials. Telaprevir was evaluated in combination with Peginterferon alfa-2a and ribavirin. In these placebo-controlled studies, the most common adverse events reported more frequently in the telaprevir treatment arms compared to the placebo arms were gastrointestinal events, skin events (rash, pruritus) and anemia. Other adverse events reported were similar in type and frequency to those seen with currently approved peg-IFN and RBV treatment. The most common adverse event leading to discontinuation in the telaprevir arms was rash in approximately 7 percent of patients across both PROVE 1 and PROVE 2. Investigators have reported that rash adverse events were reversible upon discontinuation of treatment, and a rash management plan was implemented as part of subsequent telaprevir clinical trials, including ongoing Phase 3 trials.

PROVE 1 Study Design

PROVE 1 was a Phase 2b, randomized, double-blind, placebo-controlled trial that enrolled and treated 250 treatment-naïve genotype 1 HCV patients at 37 clinical trial sites in the U.S. Of the patients enrolled in PROVE 1, the mean age was 48.1 years, 63 percent were men and 77 percent were white. Patients in PROVE 1 received 750mg of telaprevir (or placebo) orally every eight hours, based on treatment arm, and a once-weekly 180ug injection of Peginterferon alfa-2a, as well as a 1,000mg or 1,200mg weight-based daily oral dose of ribavirin. PROVE 1 consisted of four treatment arms: (1) a 24-week telaprevir-based arm consisting of 12 weeks of telaprevir in combination with peg-IFN and RBV, followed by an additional 12 weeks of peg-IFN and RBV alone, (2) a 48-week telaprevir-based arm consisting of 12 weeks of telaprevir in combination with peg-IFN and RBV, followed by an additional 36 weeks of peg-IFN and RBV alone, (3) a 12-week telaprevir-based arm consisting of 12 weeks of telaprevir in combination with peg-IFN and RBV, and (4) a control arm consisting of 12 weeks of placebo in combination with peg-IFN and RBV, followed by 36 weeks of peg-IFN and RBV alone.

PROVE 2 Study Design

PROVE 2 was a Phase 2b, randomized, partially double-blind, placebo-controlled trial that enrolled and treated 323 treatment-naïve genotype 1 HCV patients at 28 clinical trial sites in France, Germany, the United Kingdom and Austria. Of the patients enrolled in PROVE 2, the mean age was 44.3 years, 59.4 percent were men and 94.1 percent were white. Patients in PROVE 2 received 750mg of telaprevir (or placebo) orally every eight hours, based on treatment arm, a once-weekly 180ug injection of Peginterferon alfa-2a, as well as a 1,000mg or 1,200mg weight-based daily oral dose of ribavirin. PROVE 2 consisted of four treatment arms: (1) a 24-week telaprevir-based arm consisting of 12 weeks of telaprevir in combination with peg-IFN and RBV, followed by an additional 12 weeks of peg-IFN and RBV alone, (2) a 12-week telaprevir-based arm consisting of 12 weeks of

telaprevir in combination with peg-IFN and RBV, (3) a 12-week telaprevir-based arm consisting of 12 weeks of telaprevir in combination with peg-IFN (no RBV), and (4) a control arm consisting of 12 weeks of placebo in combination with peg-IFN and RBV, followed by 36 weeks of peg-IFN and RBV alone.

About Telaprevir and Vertex's HCV Development Portfolio

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

Vertex is also developing VCH-222, an oral inhibitor of the HCV NS5B polymerase. HCV polymerase inhibitors represent an additional class of drug candidates that are aimed at inhibiting viral replication.

About Hepatitis C

Hepatitis C is an infectious disease caused by the hepatitis C virus. There are an estimated 170 million people chronically infected with HCV globally, and 3 to 4 million new infections occur each year. The United States Centers for Disease Control (CDC) estimate that approximately 4.1 million people in the U.S. have been infected with HCV, and 3.2 million of these people have chronic infection, making it the most common chronic blood-borne infection in the country. Approximately 50 percent - or 2 million people - in the US living with HCV remain undiagnosed and untreated.

The burden of liver disease associated with HCV infection is increasing, and current therapies typically provide sustained benefit in less than half of patients with genotype 1 HCV, the most common strain of the virus. As many as 250,000 patients in the United States have received at least one course of treatment with peg-IFN and RBV but have not achieved an SVR. Patients who have failed interferon-based treatment typically have few or no available treatment options, and are at risk for progressive liver disease. In a recent study, the risk of liver failure, cancer or death following unsuccessful HCV treatment was 23% after 4 years, and 43% after 8 years. (1).

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 is a registered trademark of the GlaxoSmithKline group of companies.

(1) 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) data from the Phase 2b clinical trials of telaprevir, the potential to shorten the duration of treatment and the possibility that if the data from PROVE 1 and PROVE 2 is confirmed in Phase 3 studies that a 24-week treatment regimen could one day become a very important treatment option for hepatitis C patients, (ii) the data from PROVE 1 and PROVE 2 being a potentially meaningful advance in the treatment of HCV infection, (iii) the Company's expectations regarding filing an application for approval of telaprevir in the second half of 2010, (iv) the suggestion that a 24-week telaprevir-based treatment regimen may be sufficient for treatment-naïve patients who achieve an RVR, (v) the confirmation being sought in Vertex's ADVANCE Phase 3 trial, which is focused on 24-week response-guided regimens and (vi) the ADVANCE Phase 3 trial being designed to maximize the number of patients who can achieve SVR while offering a large proportion of patients the benefit of a 24-week treatment duration. 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 clinical trials in the Phase 3 clinical program, may not be favorable or confirm the results of the telaprevir Phase 2b clinical program, that regulatory authorities may require supplemental clinical trials in order to support registration of telaprevir in any particular indication, that there may be varying interpretations of data produced by one or more of our 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 in HCV 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.

(VRTX-GEN)

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