November 10, 2012

# Vertex Presents New Phase 3 Data that Showed People with Hepatitis C Treated with Twice-Daily Telaprevir Achieved Viral Cure (SVR12) Rates Similar to Those Treated Three Times Daily

*- New Phase 2 data showed approximately 3 of 4 people co-infected with hepatitis C virus (HCV) and HIV achieved HCV viral cure (SVR24) with telaprevir combination treatment -*

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that new data from a Phase 3 study in people being treated for hepatitis C for the first time showed similar rates of viral cure (SVR12, HCV RNA levels < 25 IU/mL 12 weeks after the end of all treatment) when telaprevir was given twice daily compared to three times daily, the currently approved dosing schedule. Telaprevir is approved for use in combination with pegylated-interferon and ribavirin by the U.S. Food and Drug Administration (FDA) and Health Canada under the brand name INCIVEK® (telaprevir) tablets for people with genotype 1 chronic hepatitis C virus (HCV) with compensated liver disease (some level of damage to the liver but the liver still functions), including cirrhosis (scarring of the liver). In addition, new data from a Phase 2 study demonstrated that approximately three of four people co-infected with HCV and HIV achieved an HCV viral cure (SVR24, HCV RNA levels < 25 IU/mL 24 weeks after the end of all treatment) with telaprevir combination treatment. These and other new data on Vertex's medicines in development for hepatitis C were presented at The Liver Meeting®, the 63rd Annual Meeting of the American Association for the Study of Liver Diseases (AASLD).

"INCIVEK is the most prescribed direct-acting antiviral for the treatment of hepatitis C with more than 50,000 people in the United States treated since its approval," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer at Vertex. "We're encouraged by the new data presented at AASLD that show consistent and high viral cure rates, even when telaprevir is given twice instead of three times daily."

**"OPTIMIZE trial: Non-inferiority of twice-daily telaprevir versus administration every 8 hours in treatment-naïve, genotype 1 HCV infected patients."**
**Poster Presentation #LB-8**
**November 12, 2012, 8:00 a.m. — 5:30 p.m. EST**

Results of a Phase 3 study showed that 74 percent (274/369) of people with HCV who were new to treatment and received twice-daily (BID) telaprevir in combination with pegylated-interferon and ribavirin achieved a viral cure (SVR12), compared to 73 percent (270/371) of people who received telaprevir three times a day (q8h). The study met its primary endpoint of non-inferiority. All study participants received the same total daily dose (2,250 mg) of telaprevir. Fourteen percent (103/740) of people in the study were cirrhotic at study entry, and 52 percent (53/103) of them achieved a viral cure. Adverse events were generally similar between treatment arms and consistent with the safety profile described in the U.S. prescribing information for telaprevir and included rash, anemia and pruritis (itchiness). Vertex plans to submit data supporting this new dosing regimen to the FDA in 2013 for potential inclusion in the U.S. telaprevir label.

**"Telaprevir in combination with peginterferon alfa-2a/ribavirin in HCV/HIV co-infected patients: SVR24 final study results."**
**Oral Presentation #54**
**November 11, 2012, 4:15 p.m. EST**

Final results of a Phase 2 study designed to evaluate the safety and tolerability of telaprevir in combination with pegylated-interferon and ribavirin in people who are co-infected with HCV and HIV also were presented at AASLD. Data showed that 74 percent (28/38) of patients who were treated with telaprevir combination treatment achieved an HCV viral cure (SVR24) compared to 45 percent (10/22) of those who were treated with pegylated-interferon and ribavirin alone. Changes in CD4 counts were similar between the treatment groups and no HIV viral load breakthroughs were observed in either treatment group during the study. The safety and tolerability of telaprevir observed in this study was comparable to what has been observed in HCV mono-infected patients. Adverse events that occurred more frequently (≥10 percent difference) among people treated with telaprevir compared to pegylated-interferon and ribavirin alone included pruritis (itchiness), headache, nausea, rash and dizziness.

"There are many people with hepatitis C who cannot or should not wait to be treated given the severity of their disease," said

Kenneth E. Sherman, M.D., Ph.D., Director of the Division of Digestive Diseases at the University of Cincinnati College of Medicine. "People who are co-infected with hepatitis C and HIV are among those who are most in need of effective new medicines, and these new data showed that three out of four people were able to clear the hepatitis C virus with telaprevir combination treatment. We look forward to the results of the ongoing Phase 3 study."

**About INCIVEK**

INCIVEK® (telaprevir) tablets is an oral medicine that acts directly on the hepatitis C virus protease, an enzyme essential for viral replication. INCIVEK has been prescribed to more than 50,000 patients in the United States. Approximately three out of four U.S. patients who are prescribed a direct-acting antiviral for the treatment of genotype 1 chronic hepatitis C (HCV) receive INCIVEK combination therapy.

In Phase 3 clinical studies, 79 percent of people who had not previously been treated for HCV achieved a viral cure following treatment with INCIVEK combination therapy, compared with 46 percent of those who received pegylated-interferon and ribavirin (P/R) alone. Among people who were treated previously but did not achieve a viral cure, in the Phase 3 studies: 86 percent of relapsers achieved a viral cure with INCIVEK combination therapy compared to 22 percent with P/R alone; 59 percent of partial responders achieved a viral cure compared with 15 percent with P/R alone; and 32 percent of null responders achieved a viral cure compared with 5 percent with P/R alone. In addition, many people are eligible to complete treatment with INCIVEK combination therapy in 24 weeks — half the time required for P/R alone.

INCIVEK was approved by the U.S. Food and Drug Administration (FDA) in May 2011 and by Health Canada in August 2011 for use in combination with pegylated-interferon and ribavirin for adults with genotype 1 chronic hepatitis C with compensated liver disease (some level of damage to the liver but the liver still functions), including cirrhosis (scarring of the liver). INCIVEK is approved for people who are new to treatment, and for people who were treated previously with interferon-based treatment but who did not achieve a sustained viral response, or viral cure (relapsers, partial responders and null responders).

Vertex developed telaprevir in collaboration with Janssen and Mitsubishi Tanabe Pharma. Vertex has rights to commercialize telaprevir in North America where it is being marketed under the brand name INCIVEK (in-SEE-veck). Janssen has rights to commercialize telaprevir in Europe, South America, Australia, the Middle East and certain other countries. In September 2011, telaprevir was approved in the European Union and Switzerland. Telaprevir is known as INCIVO® in Europe. Mitsubishi Tanabe Pharma has rights to commercialize telaprevir in Japan and certain Far East countries. In September 2011, telaprevir was approved in Japan and is known as Telavic®.

**IMPORTANT SAFETY INFORMATION**

**Indication**

INCIVEK® (telaprevir) is a prescription medicine used with the medicines peginterferon alfa and ribavirin to treat chronic (lasting a long time) hepatitis C genotype 1 infection in adults with stable liver problems, who have not been treated before or who have failed previous treatment. It is not known if INCIVEK is safe and effective in children under 18 years of age.

**Important Safety Information**

INCIVEK should always be taken in combination with peginterferon alfa and ribavirin. Ribavirin may cause birth defects or death of an unborn baby. Therefore, a patient should not take INCIVEK combination treatment if she is pregnant or may become pregnant, or if he is a man with a sexual partner who is pregnant. Patients must use two forms of effective birth control during treatment and for the 6 months after treatment with these medicines. Hormonal forms of birth control, including birth control pills, vaginal rings, implants or injections, may not work during treatment with INCIVEK.

INCIVEK and other medicines can affect each other and can also cause side effects that can be serious or life threatening. There are certain medicines patients cannot take with INCIVEK combination treatment. Patients should tell their healthcare providers about all the medicines they take, including prescription and non-prescription medicines, vitamins and herbal supplements.

INCIVEK can cause serious side effects including skin reactions, rash and anemia that can be severe. The most common side effects of INCIVEK include itching, nausea, diarrhea, vomiting, anal or rectal problems, taste changes and tiredness. There are other possible side effects of INCIVEK, and side effects associated with peginterferon alfa and ribavirin also apply to INCIVEK combination treatment. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information for INCIVEK including the Medication Guide, available at www.INCIVEK.com.

**About Hepatitis C**

Hepatitis C is a serious liver disease caused by the hepatitis C virus, which is spread through direct contact with the blood of infected people and ultimately affects the liver.[1] Chronic hepatitis C can lead to serious and life-threatening liver problems, including liver damage, cirrhosis, liver failure or liver cancer.[1] Though many people with hepatitis C may not experience symptoms, others may have symptoms such as fatigue, fever, jaundice and abdominal pain.[1]

Unlike HIV and hepatitis B virus, chronic hepatitis C can be cured.[2] If treatment is not successful and a person does not achieve a viral cure, they remain at an increased risk for progressive liver disease.[3,4] More than 170 million people worldwide are chronically infected with hepatitis C.[5] In the United States, up to 5 million people have chronic hepatitis C and 75 percent of them are unaware of their infection.[6,7] Hepatitis C is four times more prevalent in the United States compared to HIV.[7] The majority of people with hepatitis C in the United States were born between 1945 and 1965, accounting for 82 percent of people with the disease.[8] Hepatitis C is the leading cause of liver transplantations in the United States and is reported to contribute to 15,000 deaths annually.[9,10] By 2029, total annual medical costs in the United States for people with hepatitis C are expected to more than double, from $30 billion in 2009 to approximately $85 billion.[11]

**About Vertex**

Vertex creates new possibilities in medicine. Our team discovers, develops and commercializes innovative therapies so people with serious diseases can lead better lives.

Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of hepatitis C, cystic fibrosis, rheumatoid arthritis and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, Mass., we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada. Today, Vertex has more than 2,000 employees around the world, and for three years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences

Vertex's press releases are available at www.vrtx.com.

**Special Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, the statement regarding Vertex's plans to submit data supporting a twice-daily dosing regimen to the FDA in 2013 for potential inclusion in the U.S. telaprevir label. 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 the risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through Vertex's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

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