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Vertex Announces New Data that Showed High Viral Cure Rates with a Total of 12 and 24 Weeks of Telaprevir Combination Treatment Among People with Genotype 1 Hepatitis C Who Have the IL28B CC Genotype

- Interim analysis of the Phase 3b CONCISE study showed SVR12 rates of 87 percent with 12 total weeks of treatment and 97 percent with 24 total weeks of treatment among people who achieved RVR and completed 12 weeks of treatment -

AMSTERDAM--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced new data from an interim analysis of the exploratory global Phase 3b CONCISE study evaluating the potential to shorten total treatment with telaprevir combination therapy to 12 weeks in certain people with genotype 1 chronic hepatitis C virus (HCV) infection who have the IL28B CC genotype. In the CONCISE trial, telaprevir was administered twice daily in combination with pegylated-interferon and ribavirin. Of the 239 people in the study, 159 people completed 12 weeks of telaprevir combination treatment and had undetectable hepatitis C virus at week four of treatment (rapid viral response, or RVR) and were eligible to be randomized. One hundred seven[1] people were randomized to receive no further treatment and 52 people were randomized to receive an additional 12 weeks of treatment with pegylated-interferon and ribavirin alone, for a total of 24 weeks of treatment. In the 12-week total treatment group, of the 85 people with data available at the time of the interim analysis, 87 percent (74/85) had undetectable hepatitis C virus 12 weeks after the end of treatment (SVR12). In the 24-week treatment group, of the 30 people with data available at the time of the interim analysis, 97 percent (29/30) achieved SVR12.

This study includes people with genotype 1 chronic HCV who were new to treatment or who had relapsed after at least one prior course of treatment with pegylated-interferon and ribavirin alone. Approximately one-third of people with hepatitis C have the ‘CC' genotype, which has been associated with higher sustained viral response (SVR, or viral cure) rates and faster response to interferon-based treatment. The safety profile of telaprevir combination therapy observed in the CONCISE study through the time of the interim analysis was similar to that seen in previously reported clinical trials. The interim results of this study will be presented at the 48th Annual Meeting of the European Association for the Study of the Liver (EASL) in Amsterdam, Netherlands, April 24 to 28, 2013 (poster #881).

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 HCV infection with compensated liver disease (some level of damage to the liver but the liver still functions), including cirrhosis (scarring of the liver). INCIVEK's approved dosing schedule is two 375mg tablets three times daily, and it is given for 12 weeks in combination with pegylated-interferon and ribavirin. After the first 12 weeks, all patients stop receiving INCIVEK and continue treatment with pegylated-interferon and ribavirin alone for an additional 12 weeks or 36 weeks.

About CONCISE

CONCISE is a randomized, placebo-controlled, global, multi-center Phase 3b study designed to evaluate the safety and efficacy of a 12-week regimen of telaprevir tablets in combination with pegylated-interferon and ribavirin in people with genotype 1 chronic HCV infection who have the IL28B CC genotype. In this study, telaprevir was dosed as three 375mg tablets twice daily. The study includes 239 people with hepatitis C who are new to treatment as well as those who relapsed after at least one prior course of treatment with pegylated-interferon and ribavirin alone. The primary endpoint of the study is the proportion of randomized people who achieve a sustained viral response (HCV RNA < lower limit of quantification) 12 weeks after the last planned dose of study drug (SVR12). All study participants were assigned to receive telaprevir in combination with pegylated-interferon and ribavirin for 12 weeks. People who continued all study drugs for 12 weeks and achieved a rapid viral response to treatment (measured as undetectable HCV RNA at week 4) were randomized 2:1 to receive no further treatment or an additional 12 weeks of pegylated-interferon and ribavirin alone. People who did not achieve a rapid viral response or who did not continue all study drugs for 12 weeks were assigned a total pegylated-interferon and ribavirin treatment duration of 24 or 48 weeks based on virologic response.

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 60,000 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) are prescribed 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 treatment with 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® (telaprevir) should always be used in combination with peginterferon alfa and ribavirin. INCIVEK combination treatment may cause serious side effects including skin rash and serious skin reactions, anemia (low red blood cell count) that can be severe, and birth defects or death of an unborn baby.

Skin rashes are common with INCIVEK combination treatment. Sometimes these skin rashes and other skin reactions can become serious, require treatment in a hospital, and may lead to death. Patients should call their healthcare provider right away if they develop any skin changes during treatment with INCIVEK. Their healthcare provider will decide if they need treatment or if they need to stop INCIVEK or any of their other medicines. Patients should not stop taking INCIVEK combination treatment without talking with their healthcare provider first.

Patients' healthcare providers will do blood tests regularly to check for anemia. If anemia is severe, the healthcare providers may tell them to stop taking INCIVEK.

INCIVEK combined with peginterferon alfa and 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. Females who can become pregnant and females whose male partner takes these medicines must have a negative pregnancy test before starting treatment, every month during treatment, and for 6 months after treatment ends. Patients must use two forms of effective birth control during treatment and for 6 months after all treatment has ended. These two forms of birth control should not contain hormones, as these 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.

The most common side effects of INCIVEK combination treatment include itching, nausea, diarrhea, vomiting, anal or rectal problems (including hemorrhoids, discomfort , burning or itching around or near the anus), 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 provider about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information including Boxed Warning, and the Medication Guide for INCIVEK 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.[2] Chronic hepatitis C can lead to serious and life-threatening liver problems, including liver damage, cirrhosis, liver failure or liver cancer.[2] Though many people with hepatitis C may not experience symptoms, others may have symptoms such as fatigue, fever, jaundice and abdominal pain.[2] Unlike HIV and hepatitis B virus, chronic hepatitis C can be cured.[3] If treatment is not successful and a person does not achieve a viral cure, they remain at an increased risk for progressive liver disease.[4,5]

More than 170 million people worldwide are chronically infected with hepatitis C.[6] In the United States, up to 5 million people have chronic hepatitis C and 75 percent of them are unaware of their infection.[7,8] Hepatitis C is four times more prevalent in the United States compared to HIV.[8] 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.[9] Hepatitis C is the leading cause of liver transplantations in the United States and is reported to contribute to 15,000 deaths annually.[10,11] 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.[12]

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.

(VRTX-GEN)

Footnote:

[1] One person had genotype 6 HCV infection and was excluded from the efficacy analysis but included in the safety analysis.

References:

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