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Vertex Advances INCIVEK™ (telaprevir) and Broad Portfolio of Medicines in Development With Goal of Further Expanding and Improving Treatment for People With Hepatitis C

- Subanalysis of Phase 2 data shows 12 weeks of INCIVEK combination treatment resulted in a viral cure (SVR) for 100% of people with hepatitis C who had the IL28B CC genotype; Phase 3 study enrolling -

- Vertex's four direct-acting antivirals allow for the clinical exploration of multiple combination regimens with a non-nucleoside polymerase inhibitor, two distinct nucleotide polymerase inhibitors and INCIVEK -

BARCELONA, Spain--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced data from a retrospective subanalysis of the Phase 2 PROVE 2 study that showed that 100 percent (12/12) of patients with the IL28B CC genotype who were new to treatment achieved a viral cure (sustained viral response, or SVR) with a total of 12 weeks of treatment with INCIVEK™ (telaprevir) tablets, pegylated-interferon and ribavirin. These data support Vertex's ongoing Phase 3b study that is evaluating a total treatment duration as short as 12 weeks in people with the IL28B CC genotype. Data from the PROVE 2 subanalysis and 13 other abstracts on INCIVEK and Vertex's medicines in development for the treatment of hepatitis C will be presented at the 47th Annual Meeting of the European Association for the Study of the Liver (EASL) in Barcelona, Spain, April 18 to 22, 2012.

Vertex also announced today plans to open for enrollment a Phase 2b study to evaluate an all-oral (interferon-free) treatment regimen as short as 12 weeks in people with genotype 1 (1a and 1b) hepatitis C. The study is part of Vertex's commitment to exploring multiple interferon-free combinations with its portfolio of four direct-acting antivirals that, in addition to INCIVEK, which was approved by the U.S. Food and Drug Administration (FDA) in May 2011, include a non-nucleoside polymerase inhibitor and two distinct nucleotide polymerase inhibitors. Vertex continues to evaluate ways to expand and improve treatment with ongoing studies of INCIVEK combination therapy. The company's broad portfolio and development program support its goal of further shortening treatment time, improving convenience and tolerability and increasing viral cure rates for people with hepatitis C.

"INCIVEK has changed the way we think about treating hepatitis C by curing up to 79 percent of people new to treatment and cutting treatment time to 24 weeks for most patients," said Jean-Pierre Bronowicki, M.D., of the Centre Hospitalier Universitaire de Nancy-Brabois, Vandoeuvre-lès-Nancy, France, who presented the PROVE 2 subanalysis data at EASL. "These new data give us hope that some patients may be able to achieve a cure in as short as 12 weeks with INCIVEK combination therapy."

"More than 30,000 patients have been treated with INCIVEK in the United States and Canada in less than a year, making it the most prescribed direct-acting antiviral for chronic hepatitis C," said Christopher Wright, M.D., Ph.D., Vertex's Senior Vice President, Global Medicines Development and Medical Affairs. "Our goal is to offer people living with hepatitis C their best chance for a cure with the shortest and most tolerable treatments. We're pursuing a broad development program to evaluate multiple interferon-free combination regimens with our four direct-acting antivirals while also continuing to study INCIVEK among additional groups of patients who are in particular need of new treatment options."

Rash and anemia are the most serious side effects associated with INCIVEK. The most common side effects reported with INCIVEK combination treatment include fatigue, itching, nausea, diarrhea, vomiting, anal or rectal problems, and taste changes.

Additional Studies of INCIVEK

Vertex has multiple studies of INCIVEK ongoing or planned to evaluate additional groups of people with hepatitis C who may benefit from treatment. An ongoing Phase 3 study is evaluating INCIVEK combination therapy in people co-infected with hepatitis C and human immunodeficiency virus (HIV). Vertex is also evaluating whether treatment with INCIVEK can be effectively reduced to twice daily instead of three times daily. Pending data from these studies, Vertex plans to submit applications for approval of these indications in 2013. Additional studies of INCIVEK are ongoing among African Americans with hepatitis C and among people who have undergone a liver transplant.

Vertex to Begin Enrollment in a Phase 2 Study of its Most Advanced All-Oral Regimen

In the coming weeks, Vertex will begin enrollment in a Phase 2b study evaluating combination regimens of INCIVEK, VX-222 and ribavirin. The study will evaluate total treatment durations as short as 12 weeks in people with genotype 1 (1a and 1b) hepatitis

C who are new to treatment and will not use response-guided treatment criteria. If successful, Vertex plans to submit a New Drug Application (NDA) to the FDA for its first all-oral regimen as early as the end of 2014.

Vertex recently announced interim data from the two all-oral treatment arms of the ongoing Phase 2a ZENITH study that is evaluating VX-222 in combination with INCIVEK and ribavirin in people with genotype 1a or 1b hepatitis C who were new to treatment. As previously announced, viral loads were undetectable (< 25 IU/mL) for 83 percent (38/46) of patients with genotype 1 hepatitis C at week 12. Nine of the 11 patients eligible to stop all treatment at 12 weeks achieved SVR4 (undetectable hepatitis C virus four weeks after the end of all treatment).

The three-drug regimen was generally well tolerated. The majority of adverse events were reported as mild. There were no cases of moderate or severe rash and no discontinuations due to rash or anemia in the interferon-free study arms. There were two discontinuations due to adverse events in the genotype 1b arm of the study. Additional data from this study will be presented at the 14th International Symposium on Viral Hepatitis and Liver Disease in Shanghai, China, June 22 to 25, 2012.

Nucleotide Polymerase Inhibitor Data Expected in the Second Quarter

Vertex and its collaborator Alios BioPharma are conducting seven-day viral kinetic studies of two potent, pan-genotypic, structurally distinct nucleotide polymerase inhibitors, known as ALS-2200 and ALS-2158. The first data from these studies are expected in the second quarter of 2012. Based on data from these studies, Vertex plans to begin Phase 2 studies in the second half of 2012 to evaluate combination regimens of ALS-2200 or ALS-2158 with INCIVEK or VX-222 with or without ribavirin, as well as potential dual nucleotide regimens and other interferon-free combination regimens.

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 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 people 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®.

About IL28B

IL28B is a gene related to the interferon system. A genetic region near the IL28B gene is referred to as an IL28B genotype. There are three variations of IL28B genotypes: CC, CT or TT. These variations have been associated with a person's response to treatment for hepatitis C with pegylated-interferon and ribavirin. Studies have shown that people with the CC variation respond better to treatment with pegylated-interferon and ribavirin than those with the CT or TT variations. The CC variation is more frequent in Caucasians compared to African Americans (39 percent versus 16 percent), which may partially explain the lower response to treatment observed among African Americans in most clinical trials of pegylated-interferon and ribavirin.[1]

About ALS-2200 and ALS-2158

ALS-2200 and ALS-2158 are nucleotide analogues that appear to have a high barrier to drug resistance based on non-clinical and *in vitro* studies. Both compounds are designed to inhibit the replication of the hepatitis C virus by acting on the NS5B polymerase. Each compound is structurally distinct (adenosine and uridine) and has its own unique mechanism of action, which supports the potential for developing these compounds together as a dual nucleotide regimen and as part of combination therapy regimens, including regimens with INCIVEK and VX-222. Data from *in vitro* studies showed that both ALS-2200 and ALS-2158 had a synergistic effect when combined together and with INCIVEK and VX-222. Additionally, *in vitro* studies of both compounds showed antiviral activity across all genotypes, or forms, of the hepatitis C virus, including genotypes more prevalent outside of the United States.

Vertex gained worldwide rights to ALS-2200 and ALS-2158 through an exclusive worldwide licensing agreement signed with

Alios BioPharma, Inc. in June 2011. The agreement also includes a research program that will focus on the discovery of additional nucleotide analogues that act on hepatitis C polymerase. Vertex has the option to select additional compounds for development emerging from the research program.

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] However, approximately 60 percent of people do not achieve SVR,[4,5,6] or viral cure,[7] after treatment with 48 weeks of pegylated-interferon and ribavirin alone. If treatment is not successful and a person does not achieve a viral cure, they remain at an increased risk for progressive liver disease.[8,9]

More than 170 million people worldwide are chronically infected with hepatitis C.[7] In the United States, up to 5 million people have chronic hepatitis C and 75 percent of them are unaware of their infection.[10,11] Hepatitis C is four times more prevalent in the United States compared to HIV.[11] The majority of people with hepatitis C in the United States were born between 1945 and 1965, accounting 82 percent of people with the disease.[12] Hepatitis C is the leading cause of liver transplantations in the United States and is reported to contribute to 15,000 deaths annually.[13,14] 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, epilepsy and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, MA, 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 *Science* magazine named Vertex number one on its 2011 list of 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 Dr. Wright's statements in the fourth paragraph of this press release and statements regarding (i) Vertex's plans to advance INCIVEK and a broad portfolio of medicines in development with the goal of further expanding and improving treatment for people with hepatitis C by shortening treatment time, improving convenience and tolerability and increasing viral cure rates for people with hepatitis C; (ii) Vertex's four direct-acting antivirals allowing for the clinical exploration of multiple all-oral (interferon-free) combination regimens; (iii) the data from a retrospective subanalysis of the PROVE 2 study supporting Vertex's ongoing Phase 3b study; (iv) the possibility that some patients may be able to achieve a cure in as short as 12 weeks with INCIVEK combination therapy; (v) the company's plans to submit applications for approval of additional indications for INCIVEK in 2013; (vi) the company's plan to submit an NDA to the FDA for its first all-oral regimen as early as the end of 2014; and (vii) the company's expectations regarding the timing and structure of its ongoing and planned clinical trials of INCIVEK, VX-222, ALS-2200 and ALS-2158, including expectations regarding enrollment and when the company will obtain data from such clinical trials. 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 from clinical trials of INCIVEK, VX-222, ALS-2200 and/or ALS-2158 may not be favorable 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 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.

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**.

(VRTX-GEN)

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