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Vertex Enters Agreement with GlaxoSmithKline for Phase 2 All-Oral Study of VX-135 and GSK2336805 for the Treatment of Hepatitis C

-Companies to evaluate two-drug combination of Vertex's investigational nucleotide analogue VX-135 and GSK's investigational NS5A inhibitor GSK2336805-

-Phase 2 proof-of-concept study to begin in early 2013 to evaluate safety, tolerability and viral cure rates of 12-week treatment regimen-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced it has entered into a non-exclusive agreement with GlaxoSmithKline (GSK) to conduct a Phase 2 proof-of-concept study of an all-oral regimen for the treatment of hepatitis C containing Vertex's nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor VX-135 and GSK's NS5A inhibitor GSK2336805. Vertex and GSK plan to initiate the study in early 2013, pending discussions with regulatory authorities. The study is expected to evaluate safety, tolerability and viral cure rates using a 12-week combination of VX-135 and GSK2336805. The companies will jointly fund costs associated with the study. There are no up-front or milestone payments associated with the agreement. VX-135 is an investigational uridine nucleotide analogue pro-drug designed to inhibit the replication of the hepatitis C virus by acting on the NS5B polymerase. GSK2336805 is an investigational NS5A replication complex inhibitor being developed by GSK for the treatment of hepatitis C.

"This agreement underscores our broad commitment to develop all-oral treatment regimens for people with hepatitis C using medicines within our own pipeline and by working in collaboration with other companies like GSK who share our commitment to further improve treatment of this disease," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer at Vertex. "We are focused on the development of new all-oral treatments for hepatitis C that have the potential to provide a high cure rate with only 12 weeks of treatment, and we look forward to the start of this study with GSK."

Clinical Development Plans

The Phase 2 proof-of-concept study is expected to begin in early 2013, pending discussions with regulatory authorities, and will enroll people who have chronic non-cirrhotic genotype 1 hepatitis C and have not previously been treated (treatment-naïve). The study will be conducted in the U.S. by Vertex, and costs associated with the study will be split equally between the two companies. The goals of the study will be to evaluate safety, tolerability and viral cure rates (SVR12; undetectable hepatitis C virus 12 weeks after the end of treatment) of multiple 12-week combination regimens of VX-135 and GSK2336805, with and without ribavirin. Additional information on the Phase 2 study will be provided upon initiation of the study. Further clinical development activities beyond the Phase 2 proof-of-concept study are not covered as part of this agreement.

About VX-135

VX-135 (ALS-2200) is a uridine nucleotide analogue pro-drug that appears to have a high barrier to drug resistance based on *in vitro* studies. It is designed to inhibit the replication of the hepatitis C virus by acting on the NS5B polymerase. *In vitro* studies of the compound showed antiviral activity across all genotypes, or forms, of the hepatitis C virus, including genotypes more prevalent outside of the United States.

Earlier this year, Vertex announced the first 7-day viral kinetic data for VX-135. Based on these data, the company plans to initiate multiple all-oral, Phase 2 proof-of-concept studies, including a study of VX-135 and ribavirin and a study of VX-135 and telaprevir, the company's approved protease inhibitor marketed as INCIVEK for people with chronic genotype 1 hepatitis C. Vertex is on track to initiate the study of VX-135 in combination with ribavirin by the end of 2012, followed by the study with telaprevir in early 2013. The studies will evaluate safety, tolerability and viral cure rates (SVR12; undetectable hepatitis C virus 12 weeks after the end of treatment) of 12-week combination regimens in people with chronic non-cirrhotic genotype 1 hepatitis C who have not previously been treated (treatment-naïve).

Vertex gained worldwide rights to VX-135 through an exclusive 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.[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] However, approximately 60 percent of people do not achieve SVR,[3,4,5] or viral cure,[6] 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.[7,8]

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.[9,10] Hepatitis C is four times more prevalent in the United States compared to HIV.[10] 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.[11] Hepatitis C is the leading cause of liver transplantations in the United States and is reported to contribute to 15,000 deaths annually.[12,13] 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.[10]

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.

IMPORTANT SAFETY INFORMATION FOR INCIVEK

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.

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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, Dr. Kauffman's statements in the second paragraph of the press release and statements regarding Vertex's expectations with respect to the timing and structure of a Phase 2 study evaluating the combination of VX-135 and GSK2336805 and other planned all-oral Phase 2 studies with VX-135. While Vertex 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 initiation of planned studies may be delayed or prevented, that the outcomes of Vertex's planned clinical studies may not be favorable and 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. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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