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Vertex Enters Agreement with Bristol-Myers Squibb for Phase 2 All-Oral Studies of VX-135 in Combination with Daclatasvir for the Treatment of Hepatitis C

-Two Phase 2 studies to evaluate once-daily combination of Vertex's investigational nucleotide analogue VX-135 and BMS' investigational NS5A replication complex inhibitor daclatasvir-

-Study in people with genotype 1 hepatitis C planned to begin in second quarter of 2013-

-Study in people with genotypes 1, 2 and 3 hepatitis C, including people with cirrhosis, planned for second half of 2013-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced it has entered into a non-exclusive agreement with Bristol-Myers Squibb Company (NYSE: BMY) to conduct Phase 2 studies of once-daily all-oral treatment regimens containing Vertex's nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor VX-135 and Bristol-Myers Squibb's NS5A replication complex inhibitor daclatasvir for the treatment of hepatitis C. As part of the agreement, Vertex plans to conduct two Phase 2 studies of the combination, including an initial study in treatment-naïve people with genotype 1 HCV infection planned for the second quarter of 2013. Vertex plans to begin a subsequent study in treatment-naïve people infected with genotype 1, 2 or 3 HCV, including those with cirrhosis, in the second half of 2013, pending data from the initial study.

"With more than 170 million people infected worldwide, there is a critical need for new hepatitis C medicines that can offer people simpler and more tolerable treatment regimens that provide high cure rates," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer at Vertex. "These studies with daclatasvir will provide the first opportunity to evaluate VX-135 as part of all-oral regimens in people with multiple hepatitis C genotypes and in people with cirrhosis."

Clinical Development Plans for VX-135 with Daclatasvir

Under the terms of the agreement, Vertex will conduct two Phase 2 studies of VX-135 in combination with daclatasvir. The first study will enroll approximately 20 non-cirrhotic, treatment-naïve people with chronic genotype 1 HCV infection and is expected to begin in the second quarter of 2013. In the second half of 2013, Vertex plans to conduct a subsequent study in approximately 250 treatment-naïve people with chronic genotype 1, 2 or 3 HCV infection, including those with cirrhosis. Each of these studies is expected to evaluate safety, tolerability, pharmacokinetics and viral cure rates (SVR4 and SVR12) of multiple all-oral regimens of VX-135 and daclatasvir dosed once daily, pending regulatory discussions. Vertex will also conduct co-formulation activities to evaluate the potential for development of a once-daily fixed-dose combination regimen. Further clinical development activities beyond the Phase 2 studies are not covered as part of this agreement.

About VX-135

VX-135 is a uridine nucleotide analogue pro-drug designed to inhibit the replication of the hepatitis C virus by acting on the NS5B polymerase. In people with genotype 1, treatment with a 200mg once-daily dose of VX-135 in a 7-day viral kinetic study was well-tolerated, with no discontinuations due to adverse events, and resulted in a 4.54 $\log_{10}$ median reduction from baseline in HCV RNA. Data from a 7-day viral kinetic study of VX-135 in people with genotypes 2, 3 and 4 were consistent with data observed in people with genotype 1 and have been submitted for presentation at a future medical meeting.

Vertex gained worldwide rights to ALS-2200, known as VX-135 in Phase 2 studies, through an exclusive licensing agreement signed with Alios BioPharma, Inc. in June 2011. The agreement also includes a research program that focuses 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 Daclatasvir

Daclatasvir is an NS5A replication complex inhibitor that is being extensively studied as a key component of potential DAA-based hepatitis C treatment regimens. Studied in more than 4,100 patients to date, daclatasvir is in Phase 3 development.

Daclatasvir is part of a portfolio of investigational compounds with different mechanisms of action that Bristol-Myers Squibb is developing for the treatment of hepatitis C. These compounds are being studied as part of multiple novel treatment regimens with the goal of increasing SVR rates across diverse patient types and geographies.

About Hepatitis C

Hepatitis C is a serious liver infection 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 three fourths of people with the infection.[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 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 studies evaluating the combination of VX-135 and daclatasvir. 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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VRTX — GEN

Vertex Pharmaceuticals Incorporated
Media:
Zach Barber, 617-341-6470
mediainfo@vrtx.com
or
Investors:
Michael Partridge, 617-341-6108
or
Kelly Lewis, 617-961-7530

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