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Vertex Provides Update to Ongoing Phase 2 Study Evaluating Combinations of Telaprevir and VX-222 for the Treatment of Hepatitis C

-Two-drug treatment arm of telaprevir and VX-222 alone discontinued-

-Study continues with three arms, including all-oral combination of Vertex's lead protease and polymerase inhibitors with ribavirin-

-Both of the four-drug treatment arms are fully enrolled; the majority of patients in these arms have reached 8 weeks or more of treatment-

CAMBRIDGE, Mass., Dec 21, 2010 (BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced a modification of its Phase 2 clinical trial evaluating 12-week, response-guided regimens of its lead investigational hepatitis C virus (HCV) protease inhibitor, telaprevir, in combination with its lead investigational HCV polymerase inhibitor, VX-222. The company has discontinued the second two-drug treatment arm of telaprevir and VX-222 alone as a result of meeting a pre-defined stopping rule related to viral breakthrough. This two-drug arm was designed to evaluate a 12-week combination regimen of VX-222 (400 mg) and telaprevir (1,125 mg) dosed twice daily without pegylated-interferon and ribavirin. The first two-drug arm was discontinued in October 2010 and was designed to evaluate a 12-week combination regimen of VX-222 (100 mg) and telaprevir (1,125 mg).

The study will continue as planned with three treatment arms. Two of the treatment arms are fully enrolled and are evaluating four-drug combinations of telaprevir (1,125 mg), VX-222 (400 mg or 100 mg), Pegasys® (pegylated-interferon alfa-2a) and Copegus® (ribavirin). The last patient was randomized and began treatment with a four-drug regimen in November 2010. There are patients in the four-drug treatment arms who have recently started treatment and have not yet reached week 8 of therapy. More than half of patients in the treatment arms have received eight weeks or more of treatment and approximately one third of patients are in weeks 10 through 12 of treatment. Some patients in this study have completed therapy. Interim data from both of the four-drug treatment arms are expected in the first quarter of 2011. In November 2010, Vertex announced the planned addition of a new three-drug treatment arm designed to evaluate the potential of an all-oral, interferon-free regimen of telaprevir (1,125 mg), VX-222 (400 mg) and ribavirin dosed twice daily. Enrollment in this new treatment arm is expected to begin in the first quarter of 2011.

"This trial has provided important information regarding telaprevir and VX-222-based combination regimens, and three of the five treatment arms are proceeding as planned," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer for Vertex. "We are pleased with the progress of both four-drug treatment arms and look forward to the first quarter of 2011 when on-treatment data from these arms will become available and enrollment in the three-drug treatment arm is expected to begin."

About the Ongoing Phase 2 Trial of Telaprevir and VX-222

In August 2010, patients enrolled in this randomized, parallel-group, dose-ranging Phase 2 trial started receiving treatment. The primary endpoint of this trial is to assess safety and tolerability of 12-week, telaprevir/VX-222-based combination therapy in people with genotype 1 chronic hepatitis C. A secondary endpoint of this study is to assess on-treatment antiviral activity and the proportion of patients in each study arm who achieve a sustained viral response (SVR; defined as undetectable HCV RNA 24 weeks after the end of treatment). Patients who meet the response-guided criteria during treatment (undetectable HCV RNA at week 2 and week 8 of treatment) stop all therapy at week 12.

The planned addition of the three-drug treatment arm to the study took into account an initial review of adverse events among people treated with telaprevir/VX-222 combination regimens in this study. Enrollment in this new study arm is expected to begin in the first quarter of 2011 pending completion of institutional review board (IRB) approvals and consultations with regulatory agencies. A sixth and final arm may be added to the trial per protocol based on data from the study expected in the first quarter of 2011.

European and United States Regulatory Submissions for Telaprevir

On December 17, 2010, Janssen-Cilag International NV announced the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for telaprevir in combination with pegylated-interferon and ribavirin for the treatment of people with genotype 1 hepatitis C. Additionally, Janssen announced that the EMA has accepted telaprevir for accelerated assessment, which is granted to new medicines of major public health interest. In November 2010, Vertex announced it has completed the submission of a New Drug Application to the U.S. Food and Drug Administration for telaprevir in combination with pegylated-interferon and ribavirin for the treatment of people with genotype 1 hepatitis C. The submission included a request for six-month Priority Review, which can be granted for several reasons, including if the medicine is considered a major advance in treatment.

Telaprevir is being developed by Vertex Pharmaceuticals in collaboration with Tibotec BVBA and Mitsubishi Tanabe Pharma. Vertex has rights to commercialize telaprevir in North America. Through its affiliate, Janssen, Tibotec has rights to commercialize telaprevir in Europe, South America, Australia, the Middle East and certain other countries. Mitsubishi Tanabe Pharma has rights to commercialize telaprevir in Japan and certain Far East countries.

About Telaprevir and VX-222

Telaprevir is an investigational, oral inhibitor of HCV protease, an enzyme essential for viral replication. VX-222 is an investigational, oral, non-nucleoside inhibitor of HCV NS5B polymerase. Vertex added VX-222 to its development pipeline as part of the acquisition of ViroChem Pharma Inc. in March 2009. Vertex retains worldwide commercial rights to VX-222.

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] Up to 3.9 million people in the United States have chronic hepatitis C and 75% of those infected are unaware of their infection.[3] The majority of people with hepatitis C were born between 1946 and 1964, accounting for two of every three people with chronic hepatitis C.[11] 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]

Approximately 60 percent of genotype 1 patients who undergo an initial 48-week regimen with pegylated-interferon and ribavirin, the currently approved medicines, do not achieve SVR, [4,5,6] or viral cure.[1] If treatment is not successful and a person does not achieve a viral cure, they remain at an increased risk for progressive liver disease.[7,8,9,10,11] In the United States, hepatitis C is the leading cause of liver transplantations and is reported to contribute to 4,600 to 12,000 deaths annually.[8] By 2029, total annual medical costs in the U.S. for people with hepatitis C are expected to more than double, from $30 billion in 2009 to approximately $85 billion.[11]

Additional resources for media are available at: http://investors.vrtx.com/press.cfm.

About Vertex

Vertex Pharmaceuticals Incorporated is a global biotechnology company committed to the discovery and development of breakthrough small molecule drugs for serious diseases. The Company's strategy is to commercialize its products both independently and in collaboration with other pharmaceutical companies. Vertex's product pipeline is focused on viral diseases, cystic fibrosis, inflammation, autoimmune diseases, epilepsy, cancer and pain.

Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

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Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements including statements regarding (i) that the study will continue with three treatment arms, including an all-oral, interferon-free regimen of telaprevir, VX-222 (400 mg) and ribavirin; (ii) the expectation that interim data from both of the four-drug treatment arms are expected in the first quarter of 2011; (iii) Vertex looking forward to on-treatment data that is expected to become available from these arms in the first quarter of 2011; (iv) the plan to evaluate a 12-week combination of the three oral therapies — VX-222, telaprevir and ribavirin — dosed twice daily within a response-guided regimen; (v) the expectation that the additional three-drug treatment arm announced in November 2010 will begin patient enrollment in the first quarter of 2011, pending completion of IRB approvals and consultation with regulatory agencies; and (vi) the possibility that a sixth and final arm may be added to the trial. While Vertex believes the forward-looking statements contained in this press release are accurate, these statements are subject to risks and uncertainties that could cause actual outcomes to vary materially from the outcomes referenced in the forward-looking statements. These risks and uncertainties include, among other things, the risks that efforts to develop telaprevir and VX-222 separately or in combination may not proceed due to technical, scientific, commercial, financial or other reasons, that clinical trials may not proceed as planned, that additional clinical trials of telaprevir and VX-222 will not reflect the results obtained to date, that an adverse event profile for telaprevir or VX-222 could be revealed in further nonclinical or clinical studies that could put further development of telaprevir or VX-222 in jeopardy or adversely impact their therapeutic value, 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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