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# FDA Advisory Committee Unanimously Recommends Approval of Telaprevir for People with Hepatitis C

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the Antiviral Drugs Advisory Committee to the U.S. Food and Drug Administration (FDA) voted unanimously to recommend FDA approval of telaprevir for people with genotype 1 chronic hepatitis C. The Committee recommended by a vote of 18-0 the approval of telaprevir for those who were not treated previously and those who were treated previously but not cured with currently available medicines. Telaprevir was studied in all major subgroups of people who were treated previously and not cured: relapsers, partial responders and null responders. The FDA is expected to make a decision on the approval of telaprevir by May 23, 2011, under the Prescription Drug User Fee Act (PDUFA). The FDA is not bound by the Committee's recommendation, but usually follows its advice.

In Phase 3 studies, telaprevir was given for 12 weeks in combination with pegylated-interferon and ribavirin (P/R) followed by P/R alone for a total of 24 weeks or 48 weeks of treatment. Data from these studies that were reviewed by the Committee showed that people who received telaprevir-based combination therapy achieved significantly higher rates of sustained viral response (SVR, or viral cure) compared to treatment with 48 weeks of P/R alone, regardless of their experience with prior treatment. Among people who were not treated previously, 79 percent achieved a viral cure with telaprevir-based combination therapy compared to 46 percent who achieved a viral cure with P/R alone.

Approximately two-thirds of people in Phase 3 studies who were not treated previously and who received telaprevir-based combination therapy were eligible to complete their treatment in six months — half the time needed with currently available medicines. Today, the FDA Committee discussed Vertex's request for the approval of response-guided therapy to allow for a six-month treatment duration for people who were not treated previously as well as for those who relapsed after prior treatment with P/R alone (prior relapsers). Side effects observed with telaprevir-based combination therapy were consistent across the Phase 3 studies. Rash and anemia occurred more frequently among those treated with telaprevir-based combination therapy compared with those who received pegylated-interferon and ribavirin alone.

"Hepatitis C is a curable disease with potentially devastating consequences if left untreated, so we are pleased by the Committee's unanimous recommendation to approve telaprevir for a broad group of people with hepatitis C," said Peter Mueller, Ph.D., Chief Scientific Officer and Executive Vice President of Global Research and Development at Vertex. "We look forward to working with the FDA as it prepares to make its decision next month."

**Safety and Tolerability Information for Telaprevir**

The safety profile of telaprevir has been well characterized. Data from more than 40 clinical studies across a broad group of nearly 4,000 people were included in the new drug application. The side effects observed with telaprevir-based combination therapy were consistent across the Phase 3 studies. The most common side effects, regardless of treatment arm, were fatigue, pruritus (itchiness), nausea, headache, rash, anemia, flu-like symptoms, insomnia and diarrhea with the majority being mild to moderate.

Rash and anemia occurred more frequently among those treated with telaprevir-based combination therapy. In Phase 3 studies, discontinuation of all medicines due to either rash or anemia during the telaprevir/placebo treatment phase was approximately 1 percent for rash and 1 percent for anemia. Rash was primarily characterized as eczema-like, manageable and resolved following discontinuation of telaprevir. More than 90 percent of rash was mild to moderate and investigators in the studies primarily used topical corticosteroids and/or antihistamines to treat rash. Anemia was primarily managed by reducing the dose of ribavirin.

**About Telaprevir**

Telaprevir is an investigational, oral inhibitor that acts directly on the hepatitis C virus protease, an enzyme essential for viral replication. More than 2,800 people with hepatitis C have received telaprevir-based combination therapy as part of Phase 2 and Phase 3 studies. The Phase 3 registration studies, ADVANCE, ILLUMINATE and REALIZE, evaluated telaprevir (750 mg, taken three times daily) in combination with Pegasys® (pegylated-interferon alfa-2a) and Copegus® (ribavirin) in people with hepatitis C who had not been treated before as well as those who were treated before but not cured with a prior treatment

course of P/R.

Vertex is developing telaprevir 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.

For complete information on the telaprevir clinical trials or a fact sheet on the trial designs visit: www.vrtx.com/press.cfm.

**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.[i] Chronic hepatitis C can lead to serious and life-threatening liver problems, including liver damage, cirrhosis, liver failure or liver cancer.[i] Though many people with hepatitis C may not experience symptoms, others may have symptoms such as fatigue, fever, jaundice and abdominal pain.[i]

Unlike HIV and hepatitis B virus, chronic hepatitis C is curable.[ii] However, approximately 60 percent of people who undergo treatment with an initial 48-week regimen of pegylated-interferon and ribavirin, the currently approved medicines for genotype 1 hepatitis C, do not achieve SVR,[iii,iv,v] or viral cure.[vi] If treatment is not successful and a person does not achieve a viral cure, they remain at an increased risk for progressive liver disease.[vii,viii]

More than 170 million people worldwide are chronically infected with hepatitis C.[vi] In the United States, nearly 4 million people have chronic hepatitis C and 75 percent of them are unaware of their infection.[ix] Hepatitis C is four times more prevalent in the United States compared to HIV.[ix] The majority of people with hepatitis C in the United States were born between 1946 and 1964, accounting for two of every three people with chronic hepatitis C.[x] Hepatitis C is the leading cause of liver transplantations in the United States and is reported to contribute to 4,600 to 12,000 deaths annually.[xi,xii] 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.[ix]

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**Special Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements, including statements regarding (i) the expectation that the FDA will make a decision on approval of telaprevir by May 23, 2011 and (ii) the FDA usually following the advice of the Committee. 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 Vertex could experience unforeseen delays in obtaining approval to market telaprevir 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.

**About Vertex**

Vertex creates new possibilities in medicine. Our team aims to discover, develop and commercialize 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, 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.

For more information and to view Vertex's press releases, please visit www.vrtx.com.

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Vertex Pharmaceuticals Incorporated
**Media:**
Dawn Kalmar, 617-444-6992
mediainfo@vrtx.com
or
Amy Pasqua, 617-444-6992
mediainfo@vrtx.com
or
Zachry Barber, 617-444-6992
mediainfo@vrtx.com
or
**Investors:**
Michael Partridge, 617-444-6108
or
Lora Pike, 617-444-6755
or
Matthew Osborne, 617-444-6057

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