January 20, 2011

U.S. FDA and Health Canada Grant Priority Reviews for Telaprevir for the Treatment of Hepatitis C

-Six-month review date of May 23, 2011 set by FDA-

CAMBRIDGE, Mass., Jan 20, 2011 (BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that the U.S. Food and Drug Administration (FDA) has accepted the New Drug Application (NDA) for telaprevir and granted the company's request for six-month Priority Review. Telaprevir is Vertex's lead medicine in development for people with genotype 1 chronic hepatitis C. The FDA grants Priority Review to medicines that offer major advances in treatment or provide a treatment where no adequate therapy exists. A target review date of May 23, 2011 is set under the Prescription Drug User Fee Act (PDUFA) for the FDA's approval decision, which is four months earlier than the standard review time of 10 months.

Additionally, Vertex today announced the completion of a New Drug Submission (NDS) to the Therapeutic Product Directorate (TPD) of Health Canada seeking approval for telaprevir in Canada. Telaprevir was also granted Priority Review in Canada, which allows for faster review for promising medicines that address life-threatening or severely debilitating conditions and for which there are few effective therapies already available. Standard review in Canada takes 18 months or more and Priority Review typically shortens the review time to approximately six to nine months.

In December 2010, Janssen-Cilag International NV announced that the European Medicines Agency (EMA) accepted telaprevir for accelerated assessment in Europe, which is granted to new medicines of major public health interest.

"Data from Phase 3 studies showed that when compared to currently available medicines, telaprevir-based combination therapy nearly doubled viral cure rates and cut treatment time in half for the majority of patients new to treatment," 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 and Health Canada to make telaprevir available as quickly as possible for people with hepatitis C."

Data to Support the Telaprevir Submissions

The regulatory submissions in the United States, Canada and Europe are supported by data from three Phase 3 studies, known as ADVANCE, ILLUMINATE and REALIZE, which evaluated up to 12 weeks of telaprevir in combination with Pegasys® (pegylated-interferon alfa-2a) and Copegus® (ribavirin) in people chronically infected with genotype 1 hepatitis C virus (HCV) who were new to treatment as well as those who were treated before with currently available medicines but did not achieve a sustained viral response (SVR, or viral cure). In these studies, treatment with telaprevir-based combination therapy resulted in significantly higher viral cure rates compared to approved medicines, regardless of prior treatment experience, race or stage of liver disease. Up to 75 percent of people new to treatment achieved a viral cure with telaprevir-based therapy. The majority of these people were able to complete their course of treatment at six months — half the time needed with currently available medicines. Among those who did not achieve a viral cure with a prior treatment course of currently available medicines, Phase 3 data showed that telaprevir-based combination therapy resulted in viral cure rates three to five times higher compared to re-treatment with currently available medicines. The safety and tolerability results of telaprevir-based combination therapy were consistent across the Phase 3 studies. The most common adverse events regardless of treatment regimen were rash, fatigue, pruritis, headache, nausea, anemia, insomnia, diarrhea, flu-like symptoms and pyrexia, with the majority being mild or moderate in severity.

Vertex provided a summary of Phase 3 results, including SVR and safety data for telaprevir, in its November 23, 2010 press release announcing the NDA submission.

About Telaprevir

Telaprevir is an investigational, oral inhibitor that acts directly on the HCV protease, an enzyme essential for viral replication. To date, more than 2,500 people with genotype 1 hepatitis C have received telaprevir in Phase 2 and Phase 3 studies.

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.

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] 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,[2,3,4] or viral cure.[5] If treatment is not successful and a person does not achieve a viral cure, they remain at an increased risk for progressive liver disease.[6,7,8.9,10]

Hepatitis C in the United States

Up to 3.9 million people in the United States have chronic hepatitis C and 75 percent of them are unaware of their infection.[11] The majority of people with hepatitis C in the U.S. were born between 1946 and 1964, accounting for two of every three people with chronic hepatitis C.[10] Hepatitis C is the leading cause of liver transplantations in the U.S. and is reported to contribute to 4,600 to 12,000 deaths annually.[7] 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.[10]

Hepatitis C in Canada

Approximately 250,000 people in Canada have chronic hepatitis C and more than a third of them do not know they are infected.[12] Three provinces account for 80 percent of hepatitis C infections in Canada: Ontario (42 percent), British Columbia (22 percent) and Quebec (16 percent).[13] Each year up to 5,000 people are newly infected with hepatitis C and in 2007 alone, nearly 8,000 people were infected.[12, 13] In 2010, the annual cost of hepatitis C due to medical treatment and lost productivity in Canada was estimated to reach $1 billion.[14] By 2022, the number of hepatitis C-related deaths is expected to increase by one-third.[15]

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

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Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements, including statements regarding (i) the FDA's target review date for the telaprevir NDA, (ii) Priority Review in Canada allowing for faster review of New Drug Submissions, typically shortening the review time to approximately six to nine months and (iii) Vertex working with the FDA and Health Canada to make telaprevir available as quickly as possible for people with hepatitis C. 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; that there may be varying interpretations of the data from the telaprevir clinical trials; that future outcomes from clinical trials of telaprevir may not be favorable; that future scientific, clinical, competitive or other market factors may adversely affect the potential for telaprevir-based therapy 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.

About Vertex in Canada

In 2009, Vertex established a research and development site in Laval, Quebec through the acquisition of Virochem Pharma, Inc. Vertex is expanding its existing research and development infrastructure with the addition of commercial and medical teams to support the potential launch of telaprevir in Canada.

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

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References:

[1] Centers for Disease Control and Prevention. Hepatitis C Fact Sheet: CDC Viral Hepatitis. Available at: http://www.cdc.gov/hepatitis/HCV/PDFs/HepCGeneralFactSheet.pdf. Accessed May 25, 2010.

[2] Manns MP, McHutchison JG, Gordon SC, et al. Peginterferon alfa-2b plus ribavirin compared with interferon alfa-2b plus ribavirin for initial treatment of chronic hepatitis C: a randomised trial. *Lancet.* 2001;358:958-965.

[3] Fried MW, Shiffman ML, Reddy KR, et al. Peginterferon alfa-2a plus ribavirin for chronic hepatitis C virus infection. *N Engl J Med.* 2002;347:975-982.

[4] McHutchison JG, Lawitz EJ, Shiffman ML, et al; IDEAL Study Team. Peginterferon alfa-2b or alfa-2a with ribavirin for treatment of hepatitis C infection. *N Engl J Med*. 2009;361:580-593.

[5] Ghany MG, Strader DB, Thomas DL, Seeff, LB. Diagnosis, management and treatment of hepatitis C; An update. *Hepatology*. 2009;49 (4):1-40.

[6] Morgan TR, Ghany MG, Kim HY, Snow KK, Lindsay K, Lok AS. Outcome of sustained virological responders and non-responders in the Hepatitis C Antiviral Long-Term Treatment Against Cirrhosis (HALT-C) trial. *Hepatology*. 2008;50(Suppl 4):357A (Abstract 115).

[7] Davis GL, Alter MJ, El-Serag H, Poynard T, Jennings LW. Aging of hepatitis C virus (HCV)-infected persons in the United States: A multiple cohort model of HCV prevalence and disease progression. *Gastroenterology*. 2010;138:513-521.

[8] Volk MI, Tocco R, Saini S, Lok, ASF. Public health impact of antiviral therapy for hepatitis C in the United States. *Hepatology*. 2009;50(6):1750-1755.

[9] Veldt BJ, Heathcote J, Wedmeyer H. Sustained virologic response and clinical outcomes in patients with chronic hepatitis C and advanced fibrosis. Annals of Internal Medicine. 2007; 147: 677-684.

[10] Pyenson B, Fitch K, Iwasaki K. Consequences of hepatitis C virus (HCV): Costs of a baby boomer epidemic of liver disease. http://www.natap.org/2009/HCV/051809_01.htm. Updated May 2009. *This report was commissioned by Vertex Pharmaceuticals, Inc.*

[11] Institute of Medicine of the National Academies. Hepatitis and liver cancer: a national strategy for prevention and control of hepatitis B and C. Colvin HM and Mitchell AE, ed. http://www.iom.edu/Reports/2010/Hepatitis-and-Liver-Cancer-A-National-Strategy-for-Prevention-and-Control-of-Hepatitis-B-and-C.aspx. Updated January 11, 2010. Accessed May 25, 2010.

[12] Public Health Agency of Canada. Hepatitis C: Get the facts. You could have it and not know it. http://www.phac-aspc.gc.ca/hepc/pubs/getfacts-informezvous/index-eng.php. Updated September 21, 2010. Accessed January 18, 2011.

[13] Public Health Agency of Canada. Modeling the incidence of prevalence of hepatitis C infection and its sequelae in Canada, 2007. http://www.phac-aspc.gc.ca/sti-its-surv-epi/model/results-eng.php. Updated October 20, 2010. Accessed January 18, 2011.

[14] Public Health Agency of Canada. A renewed public health response to address hepatitis C: A summary report of the priority-setting process and strategic framework to action. http://www.phac-aspc.gc.ca/hepc/sr-rs/pdf/srhepc-eng.pdf. Updated June 2009. Accessed January 2011.

[15] Sherman M, Sharfran S, Burak K, et al. Management of chronic hepatitis C consensus guidelines. *Can J Gastroenterol*. 2007;21 (Suppl C):25C-34C.

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Source: Vertex Pharmaceuticals Incorporated