November 10, 2010

Vertex Announces Plans to Enroll Additional Treatment Arm in Ongoing Phase 2 Combination Study of Telaprevir and VX-222 for the Treatment of People with Hepatitis C

-New treatment arm to evaluate all oral, triple combination regimen of telaprevir, VX-222, and ribavirin-

CAMBRIDGE, Mass., Nov 10, 2010 (BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced plans to enroll an additional treatment arm as part of its ongoing Phase 2 clinical trial evaluating 12-week regimens of Vertex's lead investigational hepatitis C virus (HCV) protease inhibitor, telaprevir, in combination with its lead investigational HCV polymerase inhibitor, VX-222. The planned treatment arm is supported by emerging data from multiple ongoing clinical trials of direct-acting antiviral (DAA) therapies, including the trial of telaprevir/VX-222-based combination therapy, which suggest that adding ribavirin to a DAA treatment regimen may increase antiviral activity. In the additional arm, Vertex plans to evaluate a 12-week combination of three oral therapies — VX-222, telaprevir and ribavirin — dosed twice a day within a response-guided regimen.

"We are encouraged by the high viral cure rates and shorter treatment durations reported in Phase 3 studies of telaprevir-based combination therapy, and we remain focused on continuing to develop new potential treatments for hepatitis C," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer for Vertex. "Evaluating a 12-week combination of telaprevir, VX-222 and ribavirin will provide us with important information about the potential for this all-oral regimen that could be taken twice a day."

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

Beginning in August 2010, patients enrolled in the 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). If patients meet response-guided criteria during treatment (undetectable HCV RNA at week 2 and week 8 of treatment), they may be eligible to stop all therapy at 12 weeks.

The study includes treatment arms that are evaluating 12-week, response-guided regimens of two- and four-drug telaprevir/VX-222 combination therapy, given twice daily, with and without Pegasys® (pegylated-interferon alfa-2a) and Copegus® (ribavirin). Trial sites for the two- and four-drug ongoing arms completed enrollment in October 2010. The additional three-drug treatment arm of telaprevir, VX-222 and ribavirin announced today is expected to begin patient enrollment in the first quarter of 2011, pending completion of institutional review board (IRB) approvals and consultation with regulatory agencies. Based on further results from the ongoing treatment arms, Vertex may add an additional arm in this study.

Additional Clinical Trial of VX-222 Combination Therapy

Vertex is also conducting a Phase 2 clinical trial evaluating the safety, tolerability and antiviral activity of VX-222 in combination with pegylated-interferon and ribavirin, which began in August 2010. Enrollment is ongoing and Vertex expects to enroll a total of 50 patients. Patients in this trial will receive one of two doses of VX-222 (400 mg or 750 mg twice daily) in combination with pegylated-interferon alfa-2a and ribavirin for 12 weeks, followed by pegylated-interferon alfa-2a and ribavirin alone for 36 weeks.

About Telaprevir and VX-222

Telaprevir is an investigational, oral inhibitor of HCV protease, an enzyme essential for viral replication, and is being developed by Vertex Pharmaceuticals in collaboration with Tibotec Pharmaceuticals and Mitsubishi Tanabe Pharma. Phase 3 studies of telaprevir in combination with pegylated interferon alfa-2a and ribavirin are complete and Vertex is on track to complete its rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration by the end of 2010.

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 liver disease caused by the hepatitis C virus, which is found in the liver and blood of people with the disease.[2] According to a 2010 report from the Institute of Medicine, 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] Approximately 60 percent of genotype 1 patients who undergo an initial 48-week regimen with pegylated-interferon and ribavirin, the currently approved treatment regimen, do not achieve SVR,[4,5,6] or viral cure.[1]

Hepatitis C is spread through direct contact with the blood of infected people.[2] Though many people with hepatitis C may not experience symptoms, others may have symptoms such as fatigue, fever, jaundice and abdominal pain.[2] Chronic hepatitis C can lead to serious and life-threatening liver problems, including liver damage, cirrhosis, liver failure or liver cancer.[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.[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] 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] 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, including a hepatitis C backgrounder and glossary of common terms, 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.

Lexiva is a registered trademark of the GlaxoSmithKline group of companies.

PEGASYS® and COPEGUS® are registered trademarks of Hoffman-La Roche.

References:

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

[2] 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.

[3] 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.

[4] 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.

[5] 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.

[6] 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.

[7] 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).

[8] 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.

[9] 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.

[10] 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.

[11] 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.*

[12] Picchio G, et al. Discrepancies between definitions of null response to treatment with peginterferon alfa-2a and ribavirin: Implications for new HCV drug development. [poster 289]. In: *Program and Abstracts of the 2010 International Liver Conference by the European Association for the Study of Liver Disease*. . Athens, Greece: April 2010.

[13] United States Food and Drug Administration. Chronic hepatitis C virus infection: developing direct-acting antiviral agents for treatment. http://www.federalregister.gov/articles/2010/09/14/2010-22806/draft-guidance-for-industry-on-chronic-hepatitis-c-virus-infection-developing-directacting-antiviral. Updated September 14, 2010. Accessed September 14, 2010.

Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements including statements regarding (i) Vertex's plan to enroll an additional treatment arm as part of its ongoing Phase 2 clinical trial evaluating telaprevir in combination with VX-222; (ii) the support provided by emerging data suggesting that adding ribavirin to a direct-acting antiviral treatment regimen may increase antiviral activity; (iii) the plan to evaluate a 12-week combination of the three oral therapies — VX-222, telaprevir and ribavirin — dosed twice a day within a response-guided regimen; (iv) the expectation that evaluating a 12-week combination regimen of telaprevir, VX-222, and ribavirin will provide Vertex with important information about the potential for this all oral regimen that could be taken twice daily; (v) the expectation that the additional three-drug treatment arm will begin patient enrollment in the first quarter of 2011, pending completion of IRB approvals and consultation with regulatory agencies; (vi) the possibility that Vertex may add an additional treatment arm to this study; (vii) expectations regarding the additional clinical trial of VX-222 combination therapy; and (viii) Vertex being on track to complete its rolling NDA submission to the U.S. Food and Drug Administration by the end of 2010. 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.

(VRTX-GEN)

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

Source: Vertex Pharmaceuticals Incorporated