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## **New Data Suggest Vertex's Oral Hepatitis C Virus Protease Inhibitor VX-950 May Reduce Liver Injury; VX-950 Clinical Milestones on Track**

**Montreal, Canada, October 3, 2005** - New data show that patients with genotype 1 hepatitis C virus (HCV) infection treated with VX-950, an investigational oral HCV protease inhibitor being developed by Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX), rapidly achieved substantial reductions in alanine aminotransferase (ALT) levels after 14 days of treatment. The findings were presented today by researchers at the 12th International Symposium on Hepatitis C and Related Viruses (HCV 2005) in Montreal, Canada. Vertex also provided an update on clinical development of VX-950, which is one of the most advanced of a new class of medicines in development for the treatment of chronic hepatitis C infection.

Data from a 14-day clinical study demonstrated that treatment with any one of three doses of VX-950 resulted in median serum ALT declines of 25-32 U/L in all dose groups. In the placebo group, a median 8 U/L increase was observed. Prior to treatment with VX-950, serum ALT levels were elevated in approximately 70 percent of patients in the study. In the VX-950 dose groups, 83 percent (15 of 18) of patients with elevated ALT levels at baseline (prior to treatment) had achieved normalization of ALT levels at day 14, compared to 0 percent (0 of 6) in the placebo group. Elevated ALT levels are common in HCV patients and are considered to be a marker of liver injury due to HCV infection. Mean levels of serum neopterin also were observed to decrease with VX-950 treatment in the study. Decreased neopterin levels may be a further signal of a reduction in inflammation associated with HCV infection.<sup>1</sup>

A study of viral isolates from patients at baseline in a 14-day clinical study, also presented at the conference, found heterogeneity among viral sequences in the HCV protease domain. In vitro analysis indicated that all baseline viral isolates were sensitive to VX-950.<sup>2</sup>

"To date, data from early clinical studies have suggested that VX-950 is well-tolerated and can rapidly reduce HCV viral levels in patients over a short treatment period," said John Alam, M.D., Senior Vice President of Drug Evaluation and Approval at Vertex. "In addition, we now have evidence that treatment with VX-950 appeared to lead to a dramatic decline in markers of liver injury associated with viral infection."

### **Clinical Update**

Vertex affirmed today that it remains on track to achieve key milestones in its VX-950 clinical program in the fourth quarter of 2005, including initiation of a 14-day Phase Ib combination study of VX-950 and pegylated interferon in Europe and filing of an investigational new drug (IND) application in the United States to support Phase II development of VX-950. Vertex anticipates that it will initiate a 28-day, Phase II combination study of VX-950 and pegylated interferon by year-end. Vertex expects to present additional VX-950 clinical data at two more medical conferences in the fourth quarter of 2005.

### **Clinical Need and Market Opportunity in HCV Infection**

Chronic hepatitis C virus (HCV) infection is a serious public health concern affecting approximately 2.7 million people in the United States. HCV causes inflammation of the liver, which may lead to fibrosis and cirrhosis, liver cancer, and ultimately, liver failure. Cirrhosis of the liver resulting from chronic HCV infection is the leading reason for liver transplantation in the U.S. Due to the asymptomatic nature of HCV infection, it often goes undetected for up to 20 years following initial infection. Worldwide, the disease afflicts as many as 170 million people. Each year, 8,000 to 10,000 people in the U.S. die from complications of HCV infection.

### **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 major pharmaceutical companies. Vertex's product pipeline is principally focused on viral diseases, inflammation, autoimmune diseases and cancer. Vertex co-promotes the HIV protease inhibitor, Lexiva(R), with GlaxoSmithKline.

This press release may contain forward-looking statements, including statements that (i) VX-950 is well-tolerated, can dramatically reduce HCV viral levels over short treatment periods, and can lead to a reduction in liver injury associated with viral infection; (ii) Vertex is on track to achieve milestones in its VX-950 clinical program in the fourth quarter of 2005, including the initiation of a Phase 1b clinical trial in Europe, the filing of an IND in the U.S. and commencement of a Phase II clinical trial in

the U.S.; and (iii) Vertex expects to present additional VX-950 clinical data at one or more medical conferences in the fourth quarter of 2005. While management makes its best efforts to be accurate in making forward-looking statements, such statements are subject to risks and uncertainties that could cause Vertex's actual results to vary materially. These risks and uncertainties include, among other things, the risks that early clinical trial results will not be duplicated in later, larger trials, that planned clinical trials for VX-950 may not proceed as expected due to technical, scientific, or patient enrollment issues, that the planned IND filing will be delayed due to operational issues or the unavailability of required clinical data, or that, when filed, the IND will not be allowed by the FDA to open without additional studies or data which may not be readily available, and other risks listed under Risk Factors in Vertex's form 10-K filed with the Securities and Exchange Commission on March 16, 2005.

Lexiva(R) is a registered trademark of the GlaxoSmithKline group of companies.

**Vertex Contacts:**

Lynne Brum, Vice President, Corporate Communications and Financial Planning, (617) 444-6614

Michael Partridge, Director, Corporate Communications, (617) 444-6108

Lora Pike, Manager, Investor Relations, (617) 444-6755

Zachry Barber, Media Relations Specialist, (617) 444-6470

<sup>1</sup>H Gelderblom et al, "Decline in Serum Neopterin Concentration Correlates with HCV RNA Decline During Administration of VX-950, a Hepatitis C Virus Protease Inhibitor," 12th Annual International Symposium on Hepatitis C and Related Viruses, Montreal Canada.

<sup>2</sup>T Kieffer et al, "Genetic Heterogeneity in the HCV NS3 Protease of Untreated Genotype 1 Patients Has Little Effect on the Sensitivity to VX-950," 12th Annual International Symposium on Hepatitis C and Related Viruses, Montreal Canada.