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Vertex Pharmaceuticals Announces Initiation of First Human Clinical Trial for VX-950, an Investigational Oral Protease Inhibitor for the Treatment of Hepatitis C

Cambridge, MA, June 8, 2004 -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today the initiation of a Phase I clinical trial for VX-950, an investigational oral protease inhibitor for the treatment of hepatitis C virus (HCV) infection. The objective of this trial is to assess safety, tolerability and pharmacokinetics in escalating single doses of VX-950 in healthy volunteers. Approximately 35 healthy subjects will participate in the study, which is being conducted in Europe. Successful completion of the Phase I clinical trial will enable a first study of VX-950 in HCV-infected patients. Such a study is currently planned to start in the fourth quarter of 2004.

VX-950 is Vertex's lead oral HCV protease inhibitor and one of a new class of direct antivirals in development for the treatment of HCV. Preclinical studies have shown that VX-950 significantly reduces levels of HCV RNA in both an in vitro replicon system and infectious virus assays. At a scientific conference in October 2003, Vertex scientists reported that VX-950 reduced HCV RNA 10,000-fold (4 log₁₀) in nine days in an in vitro replicon assay. Preclinical pharmacokinetic studies have indicated that VX-950 is orally bioavailable and achieves excellent exposure in the liver, the target organ for HCV treatment. The initiation of clinical testing of VX-950 represents a first step towards establishing the safety and tolerability in humans.

"Preclinical data to date have indicated that direct antivirals such as VX-950 may represent a powerful new approach to the treatment of HCV infection," stated John J. Alam, M.D., Senior Vice President of Drug Evaluation and Approval at Vertex. "Initiation of human clinical trials for VX-950 reflects Vertex's commitment to leadership in the development and commercialization of novel antivirals for the treatment of HCV infection, and it is one of several important clinical milestones for Vertex's proprietary development programs in 2004."

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 indication 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 strikes as many as 185 million people. Each year, 8,000 to 10,000 people in the U.S. die from complications of HCV.

The current standard of care in HCV treatment is a combination of weekly injections of pegylated interferon alpha (peg-IFN) and daily oral dosing of ribavirin. This combination therapy provides a sustained viral response for only 40 to 50 percent of patients chronically infected with genotype 1 HCV, the most difficult viral strain to treat and the most common form in the U.S.

Vertex's drug development portfolio includes two different approaches for advancing the future standard-of-care in HCV. In addition to VX-950, Vertex is developing merimepodib, an IMPDH inhibitor in combination with pegylated interferon alpha (peg-IFN) and ribavirin. Addition of merimepodib to standard therapy has the potential to enhance antiviral activity and improve clinical outcomes for a larger percentage of patients. Vertex owns worldwide development and commercialization rights for both merimepodib and VX-950.

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 partners. Vertex's product pipeline is principally focused on viral diseases, inflammation, autoimmune diseases and cancer. Vertex co-promotes the new HIV protease inhibitor, Lexiva(TM), with GlaxoSmithKline.

This press release may contain forward-looking statements, including statements that (i) successful completion of the Phase I trial would enable a first study to assess dosing in patients in the fourth quarter of 2004; (ii) preclinical testing results suggest that VX-950 may represent a powerful new approach in the treatment of HCV infection; and (iii) addition of merimepodib in combination therapy has the potential to improve clinical outcomes. 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 clinical trials for merimepodib or VX-950 may not proceed as planned due to technical, scientific, supply or patient enrollment issues, that actual

clinical studies of VX-950 will not reflect the results obtained in nonclinical testing, that clinical results may not demonstrate the value of combination therapies for HCV patients generally, and further clinical testing of merimepodib will not confirm its potential as an enhancement to combination therapy, and other risks listed under Risk Factors in Vertex's form 10-K filed with the Securities and Exchange Commission on March 15, 2004.

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

Vertex Contact:

Lynne H. Brum, VP, Corporate Communications and Financial Planning, (617) 444-6614

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

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