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Vertex Pharmaceuticals Reports Completion of Dosing in the Phase Ia Clinical Study of VX-950, an Oral HCV Protease Inhibitor for the Treatment of Hepatitis C

Cambridge, MA, September 7, 2004 -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced it has successfully completed the dosing portion of a Phase Ia clinical trial for VX-950, an investigational oral protease inhibitor for the treatment of hepatitis C virus (HCV) infection. The study, involving 35 healthy volunteers and conducted in Europe, was designed to assess safety, tolerability and pharmacokinetics in escalating, single doses of VX-950. Based on the results from this study and preclinical studies, the Company expects to begin a Phase Ib clinical study of VX-950 in HCV-infected patients by the end of the year.

"As an oral, direct antiviral therapy, VX-950 represents an exciting novel approach for the treatment of chronic hepatitis C infection," stated John J. Alam, M.D., Senior Vice President of Drug Evaluation and Approval at Vertex. "VX-950 is a key compound in our core HCV drug development portfolio, and the completion of the Phase I study represents another important step forward for this clinical development program."

In the Phase Ia study, single doses ranging from 25 mg to 1250 mg were administered. No dose-limiting toxicities were identified, and a maximum tolerated dose was not reached. However, blood concentrations of VX-950 were observed that exceeded the concentration known to demonstrate potent antiviral activity in preclinical laboratory experiments, and at certain dose levels these target concentrations were maintained for more than 12 hours. Analysis of combined clinical and preclinical pharmacokinetic results for VX-950 suggest that liver concentrations 10- to 30-fold above the replicon 50% inhibitory concentration ("IC50") are achievable in humans using practical doses and regimens. The liver is the target organ for antiviral therapies directed against hepatitis C infection.

"The data from the Phase I study met our expectations, and we look forward to the first evaluation of VX-950 in HCV-infected patients," stated Dr. Alam. "While extrapolations based on single dose studies must be made with caution, the concentrations of VX-950 we observed in the bloodstream of healthy volunteers are at levels which we would expect to be required to demonstrate antiviral activity when VX-950 is dosed in HCV-infected patients."

In the fourth quarter of 2004, Vertex expects to initiate a multi-dose, Phase Ib clinical study with VX-950. This placebo-controlled trial will be designed to evaluate the safety, tolerability, and pharmacokinetics of up to 14 days of dosing with VX-950 in both healthy volunteers and HCV-infected patients. The data from the Phase Ia study, together with data from nonclinical studies, are being compiled for submission to the appropriate regulatory authorities for review prior to initiation of the Phase Ib study.

About VX-950 and Hepatitis C
VX-950 is Vertex's lead oral HCV protease inhibitor and one of the most advanced of a new class of antivirals in development for HCV. Preclinical data have shown that VX-950 significantly reduces levels of HCV-RNA in both the replicon system and infectious virus assays within days. Preclinical pharmacokinetic studies completed to date have indicated that VX-950 is orally bioavailable and achieves excellent exposure in the liver, the target organ for HCV treatment.

Chronic hepatitis C virus 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.

Collaboration with Mitsubishi Pharma Corporation
In June 2004, Vertex and Mitsubishi Pharma Corporation signed an agreement to develop and commercialize VX-950 in Japan and certain Far East countries. Mitsubishi will make pre-commercial payments to Vertex to support clinical development of VX-950. Additionally, Mitsubishi will pay royalties to Vertex on commercial sales of VX-950 in Mitsubishi's territories. Vertex owns development and commercialization rights to VX-950 in the rest of the world, including North America and Europe.

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(R), with GlaxoSmithKline.

This press release may contain forward-looking statements, including statements that (i) preclinical and Phase I clinical results support the initiation of a Phase Ib clinical study in HCV-infected patients; (ii) a Phase Ib study is planned for the fourth quarter of 2004; and (iii) the concentrations of VX-950 observed in the bloodstream of healthy volunteers suggest that antiviral activity will be observed in HCV-infected patients. 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 VX-950 may not proceed as planned due to technical, scientific, supply or patient enrollment issues, that subsequent clinical studies of VX-950 will not reflect the results obtained in nonclinical and initial clinical testing, that clinical results may not demonstrate the value of VX-950, 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(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
Jaren Irene Madden, Manager, Media Relations, (617) 444-6750