



May 10, 2005

## **Vertex Pharmaceuticals Reports that VX-950, an Investigational Oral Hepatitis C Protease Inhibitor, Displays Potent Antiviral Activity in Early Clinical Study**

### **- Presentation of Study Results Planned at DDW -**

**Cambridge, MA, May 10, 2005** -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced interim results that indicate that the investigational oral hepatitis C virus (HCV) protease inhibitor VX-950 was well-tolerated and demonstrated potent antiviral activity in a Phase Ib clinical trial.

The study enrolled 34 patients with chronic genotype 1 HCV infection who were treated for 14 days with placebo or one of three dose regimens of VX-950. Patients receiving 750 mg of VX-950 every eight hours achieved a median reduction in HCV-RNA of greater than 4 log<sub>10</sub>, equivalent to a more than 10,000-fold decrease in viral levels, at the end of 14 days of treatment. A median reduction in HCV-RNA of greater than 2 log<sub>10</sub> was seen in each of the other two VX-950 dose groups at the end of 14 days of treatment. Every patient receiving VX-950 achieved greater than a 2 log<sub>10</sub> reduction in HCV-RNA within the first three days of treatment. Genotype 1 HCV infection is the most difficult strain of HCV to treat and the most prevalent strain in the United States, Western Europe and Japan. Results from the study will be presented by a clinical investigator on May 17, 2005 at Digestive Disease Week(R) (DDW), a medical conference to be held in Chicago, Illinois. In accordance with the embargo policy of the meeting, the specific data from the trial beyond what is described in this press release will not be disclosed until the DDW presentation.

"Vertex is committed to developing innovative compounds for the treatment of chronic HCV infection. VX-950, one of the most advanced agents in a promising new class of direct antivirals, underscores that commitment," said Joshua Boger, Ph.D., Chairman and Chief Executive Officer of Vertex. "The demonstration of antiviral activity in this early clinical study is highly encouraging, and we look forward to sharing these data in greater detail at DDW next week."

Based on the results of the Phase Ib clinical study, the Company plans to explore the development of VX-950 as monotherapy and in combination with other HCV treatments. Vertex plans to consult with the U.S. FDA and European regulatory authorities on the Company's development plans. Vertex expects to file an investigational new drug (IND) application in the second half of 2005 to support Phase II clinical development of VX-950 in the United States. In collaboration with Vertex, Mitsubishi Pharma Corporation is developing VX-950 in Japan and certain Far East countries.

### **Trial Design**

The Phase Ib clinical trial was a double-blind, randomized placebo-controlled study designed to evaluate the tolerability, pharmacokinetics and effect on viral kinetics of three doses of VX-950 - 450 mg every 8 hours, 1250 mg every 12 hours, or 750 mg every 8 hours - over a period of 14 days, with additional post-treatment follow-up. A key goal of the study was to assess different dosing levels and frequencies for VX-950 to provide insight into dose selection for future monotherapy and combination therapy studies. Thirty-four patients with chronic genotype 1 hepatitis C virus infection were enrolled in the study; six patients received placebo and 28 patients received VX-950. The study was conducted at three centers in Europe. The trial included treatment-experienced and treatment-naïve HCV-infected patients.

### **VX-950 Demonstrates Antiviral Activity**

Interim Phase Ib clinical trial results indicate that VX-950 was well-tolerated across all three dose groups with no serious adverse events reported, and no treatment discontinuations. Treatment with VX-950 also resulted in significant reductions in plasma HCV-RNA. Within three days of treatment, the median reduction in HCV-RNA was greater than 3 log<sub>10</sub> in all three VX-950 dose groups. In the dose group receiving 750 mg of VX-950 every 8 hours, there was a further reduction in viral levels between days 3 and 14 of treatment, with mean and median HCV-RNA reductions of greater than 4 log<sub>10</sub> at day 14. Trough plasma concentrations of VX-950 were highest in the 750 mg every 8 hour dose group. In the 450 mg q8h and 1250 mg q12h dose groups, maximal effects were seen between days 3 and 7 of treatment. Subsequently, there was an increase of approximately 1 log<sub>10</sub> in median HCV-RNA between days 7 and 14 evident in both groups. Full analysis of the study, including a detailed pharmacokinetic and viral sequencing evaluation, is underway.

### **Web Cast Conference Call on May 17**

Following the presentation of VX-950 clinical data at DDW, Vertex Pharmaceuticals will host a conference call on May 17, 2005 at 4:00 p.m. Eastern Daylight Time (EDT). This call will be broadcast live via the Internet at [www.vrtx.com](http://www.vrtx.com) in the investor center until end of day on May 30, 2005. Alternatively, to listen to the call live on the telephone, dial (800) 374-0296 (U.S. and

Canada) or (706) 634-2224 (International). The archived call will be available via telephone commencing May 17, 2005 at 8:00 p.m. EDT through 5:00 p.m. EDT on May 23, 2005. The replay phone number for the U.S. and Canada is (800) 642-1687. The international replay number is (706) 645-9291. The conference ID number is 6231209 for both numbers.

#### **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.

#### **Safe Harbor Statement**

This press release may contain forward-looking statements, including statements that (i) Vertex's HCV protease inhibitor VX-950 is well-tolerated and possesses potent antiviral activity; (ii) that Vertex expects to explore development of VX-950 as monotherapy and as part of combination therapy; and (iii) Vertex plans to file an IND during the second half of 2005 to support clinical development of VX-950 in the United States. 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 (i) full analysis of the data, or further testing, will not reflect the interim results, or support any or all of the conclusions provided in this press release; and (ii) clinical trials for VX-950 may not proceed as planned due to technical, scientific, or patient enrollment issues, clinical trial results may not be available when expected, or expected regulatory filings may not occur or may be delayed due to adverse clinical or non-clinical trial developments; 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.

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