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Vertex Pharmaceuticals Reports that Oral Hepatitis C Protease Inhibitor, VX-950, Dramatically Reduces Viral Levels in Phase Ib Clinical Study

-- Five Patients on 14-day Clinical Study Achieve Plasma Levels Below Limit of Quantitation --

Chicago, IL, May 17, 2005 -- In an oral presentation today at the Digestive Disease Week conference, Henk W. Reesink, M.D., Associate Professor of Medicine at Academic Medical Center in Amsterdam, presented results of a Phase Ib clinical trial with the oral hepatitis C virus (HCV) protease inhibitor VX-950, an investigational drug discovered by Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX). In the study, dosing with VX-950 for 5 and 14 days was well-tolerated in both healthy volunteers and in patients with chronic HCV infection. In addition, patients treated with 750 mg of VX-950 every eight hours achieved a median reduction of HCV-RNA of 4.4 log₁₀, equivalent to a 25,000-fold reduction in viral levels, at the end of 14 days of treatment. At the end of 14 days of treatment, 4 of 8 patients in the 750 mg dose group tested HCV-RNA negative in the quantitative Roche COBAS TaqMan[®] assay (<30 IU/mL); 2 of these 4 patients tested undetectable in the qualitative Roche TaqMan[®] assay (limit of detection 10 IU/mL). A patient in another VX-950 dose group also achieved plasma HCV-RNA below the limit of quantitation by the end of treatment. All patients in the clinical trial had genotype 1 HCV infection, the most difficult strain to treat, and were either non-responsive to prior treatment or treatment-naive. VX-950 is one of the most advanced of a new class of direct antivirals for hepatitis C.

"Preliminary results from this early Phase Ib clinical study suggest that the investigational drug VX-950 produces a rapid and profound reduction in HCV-RNA as a single agent," said Prof. Reesink. "In the best dose group in the Phase Ib clinical study, VX-950 reduced HCV viral load in some patients to below the limit of detection of the most sensitive assays in two weeks. VX-950 was also well-tolerated in this study. These data further support the view that HCV protease is the most potent single mechanism for suppressing hepatitis C viral replication."

"The antiviral effect of VX-950 in this first study has exceeded our expectations," said Joshua Boger, Ph.D., Chairman, President and Chief Executive Officer of Vertex Pharmaceuticals. "The rapidity of viral load decline and the achievement of viral levels to below detection in some patients means that we have a broad opportunity to explore VX-950 in more advanced studies in hepatitis C patients. Vertex is committed to moving as rapidly as possible to advance VX-950 to the next stage of clinical development."

Key Phase Ib Study Findings

Significant reductions in HCV-RNA were observed in HCV patients taking VX-950 across three dose groups - 450 mg every 8 hours, 1250 mg every 12 hours, or 750 mg every 8 hours - over a period of 14 days. Within three days of treatment, the median reduction in HCV-RNA was greater than 3 log₁₀, a reduction of at least 1,000-fold, 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 a median HCV-RNA reduction of 4.4 log₁₀ at day 14. Trough VX-950 plasma concentrations were highest in the 750 mg every 8 hour dose group. At the end of treatment, 4 of 8 patients in the 750 mg dose group tested HCV-RNA negative in the quantitative Roche COBAS TaqMan[®] assay (<30 IU/mL), and 2 of these 4 patients tested undetectable in the qualitative Roche COBAS TaqMan[®] assay (limit of detection 10 IU/mL).

At the end of dosing, a total of 5 patients in the Phase Ib study across the dose groups tested HCV-RNA negative in the quantitative Roche COBAS TaqMan[®] assay (<30 IU/mL), reaching this level sometime between day 11 and 14. Following completion of the 14-day dosing period, a slow increase in HCV-RNA levels was observed during a 28-day post-dosing period in these patients. Twenty-eight days after receiving their last dose of VX-950, there were two patients that had viral levels of more than 1 log₁₀ below their pre-treatment levels.

Preliminary data indicate that across the three dose groups, VX-950 was well-tolerated, with no serious adverse events or treatment discontinuations reported. In addition, no elevations of ALT/AST or other clinical chemistry findings were reported.

About VX-950

VX-950 is an oral inhibitor of hepatitis C virus protease, an enzyme essential for viral replication. Vertex completed a Phase Ia clinical study of VX-950 in healthy volunteers in 2004, which indicated that VX-950 was well-tolerated in ascending single doses up to 1250 mg. Pharmacokinetic results from the Phase Ia study suggested that VX-950 can achieve liver concentrations substantially greater than IC₅₀ and IC₉₀ observed in non-clinical studies. Preclinical studies, presented at various medical

conferences in 2003 and 2004, demonstrated that VX-950 significantly reduces levels of HCV RNA in both an in vitro replicon system and infectious virus assays. Vertex researchers were the first to solve the three-dimensional crystal structure of HCV protease, and have used structural insights to enable the design of small molecule HCV protease inhibitors, including VX-950.

Next Steps

The Company is actively planning more advanced studies to evaluate 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.

Web Cast Conference Call on May 17

Vertex Pharmaceuticals will host a conference call on May 17, 2005 at 4:00 p.m. Eastern Time (EDT) to review results from the Phase Ib clinical trial of VX-950. This call will be broadcast via the Internet at www.vrtx.com in the investor center and will be available until end of day on May 31, 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 24, 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.

Background

Phase Ib Trial Design: Healthy Volunteers

The Phase Ib clinical trial involved dosing of VX-950 in healthy volunteers as well as patients chronically infected with genotype 1 HCV. Initially, 24 healthy volunteers were randomized to receive one of three doses of VX-950 - 450 mg every 8 hours, 750 mg every 8 hours, or 1250 mg every 8 hours - or placebo, for five days. The purpose of dosing in healthy volunteers was to evaluate the safety and pharmacokinetics of multiple doses of VX-950 before proceeding to dosing in patients.

Healthy Volunteer Results

Full analysis of the data in healthy volunteers has been completed. No serious adverse events or treatment discontinuations were reported. The most common adverse events considered to be possibly related to study drug were headache (5 of 24 subjects), diarrhea (3 subjects), nausea (2 subjects), frequent urination (2 subjects) and sleepiness/drowsiness (2 subjects); all were mild in severity. No changes were observed in vital signs, physical examinations, or heart rhythm and electrical intervals (as measured by digital ECGs).

Phase Ib Trial Design: HCV Patients

Double-blind, randomized placebo-controlled dosing of VX-950 in HCV patients was then conducted 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. Each dose was administered over a period of 14 days, with additional post-treatment follow-up. A key goal of this portion 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 enrolled 25 patients who were non-responders to prior interferon-based regimens, and 9 patients who were treatment-naïve. In the HCV patient dosing portion of the study, patient demographics were similar across the three dose groups. Median serum viral load at study entry ranged between 6.13 log₁₀ and 6.48 log₁₀ HCV-RNA (approximately 1.5- 3 million IU/mL).

Vertex continues to evaluate data from the Phase Ib clinical study. Complete analyses of the safety and tolerability of VX-950, as well as pharmacokinetic and viral sequencing analyses for all patients, are underway.

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. In collaboration with GlaxoSmithKline, Vertex co-promotes the HIV protease inhibitor, Lexiva[®].

Lexiva[®] is a registered trademark of the GlaxoSmithKline group of companies.

About Digestive Disease Week

Digestive Disease Week (DDW) is the largest international gathering of physicians, researchers and academics in the fields of

gastroenterology, hepatology, endoscopy and gastrointestinal surgery. Jointly sponsored by the American Association for the Study of Liver Diseases (AASLD), the American Gastroenterological Association (AGA), the American Society for Gastrointestinal Endoscopy (ASGE) and the Society for Surgery of the Alimentary Tract (SSAT), DDW takes place May 14-19, 2005 in Chicago. The meeting showcases approximately 5,000 abstracts and hundreds of lectures on the latest advances in GI research, medicine and technology.

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 produces rapid and profound reduction in HCV-RNA as a single agent; (ii) that Vertex expects to explore development of VX-950 as monotherapy and as part of combination therapy; (iii) protease inhibitor-based therapy has the potential to become a powerful option in future HCV therapy; and (iv) 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; (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; (iii) further clinical trials will not confirm the potential of VX-950 for the treatment of HCV infection; and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission on March 16, 2005.

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