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Vertex Pharmaceuticals to Begin Phase 3 Development of Telaprevir, Investigational Hepatitis C Protease Inhibitor

-- Primary Phase 3 trial will focus on studying 24-week telaprevir-based regimens -- Vertex plans concurrent second study to support registration -- Final data from both trials anticipated in mid- 2010

CAMBRIDGE, Mass., Jan 23, 2008 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it will begin Phase 3 evaluation of telaprevir, Vertex's lead investigational hepatitis C protease inhibitor. The primary focus will be a global, 3-arm pivotal controlled trial that will evaluate two 24-week telaprevir-based regimens in approximately 1050 treatment-naive genotype 1 HCV patients. In this study, rapid viral response (RVR) criteria will be used to determine which telaprevir patients can stop all treatment at 24 weeks. A second study of approximately 400-500 HCV patients is planned to evaluate a 48-week telaprevir-based regimen, to confirm the results from Phase 2 studies and provide additional evidence that supports the 24-week regimen that is being evaluated in the primary Phase 3 trial. The Company expects that both studies will run concurrently and that the first trial will begin enrolling patients in March 2008.

"Data presented in late 2007 from two large Phase 2b studies suggest that telaprevir, dosed in combination with pegylated interferon and ribavirin, may be able to meaningfully increase the proportion of treatment-naive genotype 1 HCV patients who achieve a sustained viral response, and also cut the current treatment duration in half, to 24 weeks," said John McHutchison, M.D., Principal Investigator for the primary telaprevir Phase 3 pivotal study and Associate Director of Duke Clinical Research Institute. "Telaprevir is the most advanced protease inhibitor in development for hepatitis C, and the initiation of Phase 3 clinical development for this investigational drug will begin the process of helping to further assess its potential efficacy and the safety in a larger number of patients."

Pivotal Trial to Evaluate 24-Week Telaprevir-Based Treatment Regimens

In accordance with the design and protocol Vertex submitted to the FDA, the primary pivotal trial will focus on evaluation of 24 weeks of telaprevir-based therapy and will enroll approximately 1050 treatment-naive, genotype 1 HCV patients, who will be randomized equally across three treatment arms (approximately 350 patients per arm).

The study will be conducted at approximately 100 centers in the U.S., E.U. and certain other countries. The study arms will include:

-- 24 weeks of therapy, with telaprevir dosed at 750 mg every eight hours (q8h) for 12 weeks in combination with standard doses of pegylated interferon alfa-2a (peg-IFN) and ribavirin (RBV) for 12 weeks, then continuing for another 12 weeks with peg-IFN and RBV alone;

-- 24 weeks of therapy, with telaprevir dosed at 750 mg every eight hours (q8h) for 8 weeks in combination with standard doses of peg-IFN and RBV for 8 weeks, then continuing for another 16 weeks with peg-IFN and RBV alone; and

-- A control arm with standard doses of peg-IFN and RBV dosed for 48 weeks.

Patients in both telaprevir arms who achieve rapid viral response (RVR), defined as undetectable (less than 10 IU/mL) viral levels by the end of week 4, and who stay undetectable at week 12 will receive 24 weeks of treatment. Patients in these treatment arms who do not meet the RVR criteria but are undetectable at week 24 will continue on peg-IFN and RBV for a total duration of 48 weeks.

Concurrent 48-Week Second Study to Support Registration

Vertex has agreed to conduct a second well-controlled clinical study as part of the registration program for a treatment-naive indication. The objective of this second study would be to develop additional sustained viral response (SVR) and relapse rate data with 48-weeks treatment duration that confirm results from the Phase 2 studies, thereby providing additional evidence supporting the 24-week regimen in the Phase 3 trial. The design of this second study is being finalized, but at this time Vertex expects this study to enroll approximately 400-500 patients, including patients in the control arm.

The primary objective of the two studies will be to assess the proportion of patients in each study arm who achieve SVR, defined as undetectable (less than 10 IU/mL, as measured by the Roche TaqMan(R) assay) HCV RNA 24 weeks after the completion of dosing. Vertex expects to have SVR data from both studies by mid-2010.

Update on Meeting with FDA

Vertex and the FDA met in mid-January 2008 to discuss telaprevir's Phase 3 development program. This meeting included a review of available data from Phase 2b clinical trials of telaprevir, including newly available post-treatment data from the 48-week treatment arms in PROVE 1. In the control arm of PROVE 1, on an ITT basis, 37% of patients had undetectable HCV RNA at 12 weeks post-treatment follow-up. In the 48-week ("12+36") telaprevir-based treatment arm in PROVE 1, also on an ITT basis, 66% of patients had undetectable HCV RNA at 12 weeks post-treatment follow-up. The relapse rate in the 48-week telaprevir-based arm in PROVE 1 was 6%.

Additional HCV Studies

Vertex and Tibotec continue to conduct additional clinical studies to evaluate the potential role of telaprevir treatment for important HCV sub-populations as well as different dosing regimens for telaprevir.

-- The companies are conducting PROVE 3, a Phase 2b clinical trial of telaprevir-based combination therapy in patients with genotype-1 HCV who have not achieved SVR with a previous pegylated interferon-based treatment. Vertex plans to discuss with regulatory authorities in mid-2008 the next steps in the telaprevir development program for treatment-failure HCV patients after the first interim clinical data are available from the PROVE 3 clinical trial.

-- Tibotec is conducting a Phase 2 clinical study in Europe to evaluate 8-hourly and 12-hourly dosing of telaprevir in combination with pegylated interferon (Pegasys(R) or PegIntron(TM)) and ribavirin. Interim 12-week on-treatment data are expected to be available in the second half of 2008.

-- Tibotec is also conducting a Phase 2 viral kinetics study in Europe to evaluate telaprevir in patients infected with genotype 2/3 HCV. Interim on-treatment data are expected to be available in late 2008.

-- In addition, in December, Tibotec initiated a Phase 2 study in Europe to evaluate telaprevir in patients infected with genotype 4 HCV.

Updates on the status of Vertex and Tibotec's clinical trials of telaprevir are available at www.clinicaltrials.gov.

About Telaprevir

Telaprevir (VX-950) is an investigational oral inhibitor of HCV protease, an enzyme essential for viral replication, and is one of the most advanced investigational antiviral agents in development that specifically targets HCV. The types of adverse events that have been commonly observed with Peg-IFN and RBV were seen across all treatment arms in Phase 2b trials of telaprevir. The most common adverse events, regardless of treatment assignment, were fatigue, rash, headache and nausea. Gastrointestinal disorders, skin adverse events (rash, pruritus) and anemia were more common in the telaprevir arms compared to the control arm over the dosing period.

About Hepatitis C

Hepatitis C is a liver disease caused by the hepatitis C virus, which is found in the blood of people with the disease. HCV, a serious public health concern affecting 3.4 million individuals in the United States, is spread through direct contact with the blood of infected people. Though many people with hepatitis C may not experience symptoms, others may have symptoms such as jaundice, abdominal pain, fatigue and fever. Chronic hepatitis C significantly increases a person's risk for developing long-term infection, chronic liver disease, cirrhosis or death. The burden of liver disease associated with HCV infection is increasing, and current therapies typically provide sustained benefit in less than half of patients with genotype 1 HCV, the most common strain of the virus.

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 focused on viral diseases, inflammation, autoimmune diseases, cancer, pain and bacterial infection. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

Disclosure: Dr. McHutchison receives research support, as does the Duke Clinical Research Institute, from Vertex, and he has served in an advisory capacity for the company.

Lexiva is a registered trademark of the GlaxoSmithKline group of companies.

TaqMan(R) is a registered trademark of Hoffman-La Roche Inc.

Webcast and Conference Call on January 23

Vertex Pharmaceuticals will host a conference call on January 23, 2008 at 8:00 a.m. Eastern Time (ET) to review the Phase 3 trial announcement. This call will be broadcast via the Internet at www.vrtx.com from the 'Events & Presentations' page. To listen to the call live on the telephone, dial (800) 374-0296 (U.S. and Canada) or (706) 634-2224 (International). The call will be available for replay via telephone commencing January 23, 2008 at 11:00 a.m. ET running through 5:00 p.m. ET on February 6, 2008. The replay phone number for the US and Canada is (800) 642-1687. The international replay number is (706) 645-9291 and the conference ID number is 32008293. Following the live webcast, an archived version will also be available on Vertex's website until 5:00 p.m. ET on February 6, 2008.

Safe Harbor Statement

This press release contains forward-looking statements, including statements regarding (i) Vertex's plan to begin Phase 3 clinical development of telaprevir; (ii) the designs and protocols of the planned clinical trials, including the anticipated number of patients and the description of the anticipated treatment arms; (iii) Vertex's expectation that the two clinical trials will run concurrently and that the larger trial will begin enrolling patients in March 2008; (iv) the expectation that the second study will evaluate a 48-week telaprevir-based regimen, to confirm the results from Phase 2 studies and provide additional evidence that supports the 24-week regimen that is being evaluated in the primary Phase 3 trial; (v) Vertex's expectation that it will have SVR data for both clinical trials of telaprevir by mid-2010; (vi) Vertex's plan to discuss with regulatory authorities in mid-2008 the next steps in the telaprevir development program for patients with HCV who have failed to achieve sustained viral response with previous treatments; and (vii) the dates by which interim on-treatment data is expected for the Phase 2 clinical trials being conducted by Tibotec in Europe. While Vertex believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. These risks and uncertainties include, among other things, that Vertex's ongoing discussions with regulatory authorities may result in changes to the clinical trials described in this press release, that the outcomes for each of the planned clinical trials of telaprevir may not be favorable or may reflect unanticipated results which could impact the planned development path for telaprevir, that enrollment in the clinical trials may be more difficult or slower than Vertex currently anticipates or that the planned clinical trials may not start on the dates anticipated, and other risks listed under Risk Factors in Vertex's form 10-K filed with the Securities and Exchange Commission on March 1, 2007.

(VRTX-GEN)

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