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Vertex Broadens its Commitment to Improving HCV Care with Clinical Trial to Evaluate Combination Regimens Based on Oral Antiviral Therapies

-Trial will evaluate safety and SVR rates with multiple 12-week response-guided regimens of telaprevir/VX-222-based combination therapy, including two-drug regimens of telaprevir and VX-222-
-Interim clinical data expected in the second half of 2010-
-Multiple clinical trial sites in the U.S. to enroll patients-

CAMBRIDGE, Mass., Mar 01, 2010 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it is initiating the first clinical trial evaluating Vertex's lead investigational hepatitis C virus (HCV) protease inhibitor, telaprevir, dosed in combination with the company's lead investigational HCV polymerase inhibitor, VX-222. This Phase 2 trial will evaluate sustained viral response rates (SVR; defined as undetectable HCV RNA 24 weeks after the end of treatment) using multiple 12-week response-guided regimens of telaprevir/VX-222-based combination therapy, including two-drug regimens that contain only telaprevir and VX-222. The trial is expected to enroll approximately 100 treatment-naïve genotype 1 HCV patients at multiple clinical trial sites, the majority of which will be located in the U.S. Enrollment is expected to be completed in the second quarter of 2010. Vertex expects to obtain interim clinical data, including safety and viral kinetic data, from this trial in the second half of 2010.

"Vertex is committed to improving patient care in HCV, and the announcement of this clinical trial combining two oral agents, telaprevir and VX-222, signifies our first exploration into this combination regimen's potential role to further improve the treatment of HCV," said Peter Mueller, Ph.D., Vertex's Chief Scientific Officer and Executive Vice President, Global Research and Development.

"The completion of the Phase 3 development program for telaprevir remains our primary focus, and we are on track to submit a New Drug Application for telaprevir in the second half of 2010. We believe telaprevir could represent a significant opportunity to improve the treatment of HCV, and simultaneously, we are focused on evaluating additional opportunities to potentially enhance HCV therapy even more in the years ahead using novel combination regimens based on oral antiviral agents. We believe the trial announced today will inform the development path for telaprevir/VX-222-based combination therapy, and we look forward to obtaining the first clinical data from the trial later this year," continued Dr. Mueller.

About the Phase 2 Trial of Telaprevir and VX-222

The randomized, parallel-group, dose-ranging trial announced today is designed to evaluate the safety and antiviral activity, including SVR, of multiple 12-week response-guided telaprevir/VX-222-based combination regimens. The primary endpoint of this trial is to assess safety and tolerability of telaprevir/VX-222-based combination therapy. A secondary endpoint of this study is to assess the proportion of patients in each study arm who achieve SVR. The trial is expected to enroll approximately 100 treatment-naïve genotype 1 HCV patients at approximately 20 clinical trial sites, predominantly in the U.S. Vertex expects to complete enrollment for the trial in the second quarter of 2010. The trial will consist of four arms, as noted below:

12-Week Treatment Regimens	Patient Enrollment
Telaprevir (1125 mg BID) + VX-222 (100 mg BID)	25
Telaprevir (1125 mg BID) + VX-222 (400 mg BID)	25
Telaprevir (1125 mg BID) + VX-222 (100 mg BID) + peg-IFN + RBV	25
Telaprevir (1125 mg BID) + VX-222 (400 mg BID) + peg-IFN + RBV	25
BID = twice daily, peg-IFN = pegylated interferon, RBV = ribavirin	

Response-Guided Trial Design

The trial will utilize response-guided criteria aimed at evaluating shorter-duration treatment regimens. All patients, regardless of treatment group, whose HCV RNA levels are undetectable (<10 IU/mL) at week 2 and week 8 of treatment, will stop their assigned treatment at week 12. Patients who do not meet these criteria will complete their assigned treatment and at week 12,

those in the dual-drug regimen will receive follow-on therapy of 24 weeks of pegylated-interferon (peg-IFN) and ribavirin (RBV), for a total of 36 weeks of treatment. Patients in the quad-therapy regimens who do not meet these criteria at week 12 will receive an additional 12 weeks of follow-on therapy with peg-IFN and RBV for a total of 24 weeks of peg-IFN and RBV therapy.

Potential Additional Arms of Telaprevir/VX-222-based Combination Treatment

Based on an evaluation of on-treatment safety, pharmacokinetic and antiviral data from patients in each arm of the trial, Vertex may elect to enroll up to two additional treatment arms that will evaluate telaprevir/VX-222-based combination therapy. The components of the treatment regimens of these arms will be selected based on clinical data that emerges from the four initially-studied regimens. If enacted, up to 25 patients are expected to enroll in each additional treatment arm.

Recent Clinical Trials Support Evaluation of Telaprevir/VX-222-based Combination Regimens

Phase 1b/2a Clinical Trial of VX-222 in HCV Patients

Interim clinical results from a two-part Phase 1b/2a clinical trial of VX-222 showed that in the multiple-dose Phase 1b viral kinetic portion of the trial (Part A), VX-222 was well-tolerated across four VX-222 dose groups with no serious adverse events reported. Part A enrolled 32 genotype 1 HCV patients to receive three days of dosing, and a mean HCV RNA decline of greater than 3 $\log_{10}$ was observed across all four VX-222 dose groups. An increasing dose response was observed across the four dose groups, with the results with 500 mg and 750 mg BID, and 1500 mg QD (once-daily) being very similar. The mean HCV RNA decline achieved after three days of dosing with 250 mg, 500 mg, and 750 mg of VX-222 every 12 hours (q12h) was 3.1 $\log_{10}$, 3.4 $\log_{10}$, and 3.2 $\log_{10}$, respectively. Additionally, the mean HCV RNA decline achieved after three days of dosing with 1500 mg of VX-222 every 24 hours (QD) was 3.4 $\log_{10}$. For patients who received placebo, the mean HCV RNA decline after three days of dosing was 0.1 $\log_{10}$. The majority of the patients enrolled in Part A had genotype 1a chronic HCV infection. Full results including the final safety analysis from Part A of this trial are expected to be presented at a medical meeting in 2010.

The interim results of Part A of this trial are consistent with the findings from a previously-conducted three-day, five-patient viral kinetic study of VX-222. Part B of the study, which will be initiated shortly will evaluate 12 weeks of VX-222 dosed in combination with peg-IFN and RBV in treatment-naïve HCV patients.

Drug-Drug Interaction Study of Telaprevir and VX-222 in Healthy Volunteers

Vertex also recently completed a Phase 1 study of telaprevir and VX-222 designed to evaluate the safety, tolerability and drug-drug interaction of telaprevir and VX-222 in approximately 20 healthy volunteers. In the study, the 10-day telaprevir/VX-222-based combination regimens were well-tolerated with no serious adverse events reported.

In this study, an increase in the plasma exposure of VX-222 was observed when dosed in combination with telaprevir, while the plasma exposure of telaprevir was not affected when dosed in combination with VX-222. Based on this observation, Vertex selected VX-222 doses of 100 mg twice-daily BID and 400 mg BID for evaluation in the Phase 2 trial of telaprevir/VX-222-based combination therapy announced today. The VX-222 doses of 100 mg BID and 400 mg BID are expected to provide plasma exposures similar to those observed with doses of 250 mg BID and 750 mg BID, respectively, in the previously conducted viral kinetic studies of VX-222 monotherapy in HCV patients.

The results of both the three-day viral kinetic study of VX-222 and the drug-drug interaction study of telaprevir/VX-222-based regimens support the Phase 2 combination trial of telaprevir and VX-222 in HCV patients, as announced today.

About Telaprevir and VX-222

Telaprevir is an investigational oral inhibitor of HCV protease, an enzyme essential for viral replication, and is being evaluated as part of a global Phase 3 registration program in more than 2,200 treatment-naïve and treatment-failure patients. Vertex is collaborating with Tibotec and Mitsubishi Tanabe Pharma to develop telaprevir. Vertex retains commercial rights to telaprevir in North America. Tibotec has rights to commercialize telaprevir in Europe, South America, Australia, the Middle East and other countries. Mitsubishi has rights to commercialize telaprevir in Japan and certain Far East countries.

VX-222 is an investigational oral non-nucleoside inhibitor of HCV NS5B polymerase. Vertex added VX-222 to its development pipeline as part of the acquisition of ViroChem Pharma Inc. in March 2009. Vertex retains worldwide commercial rights to VX-222.

About Hepatitis C

Hepatitis C is a liver disease caused by the hepatitis C virus (HCV), which is found in the blood of people with the disease. HCV,

a serious public health concern affecting approximately 3.2 million individuals in the United States, is spread through direct contact with the blood of infected people.[1] Though many people with HCV infection may not experience symptoms, others may have symptoms such as jaundice, abdominal pain, fatigue and fever.[1] Chronic HCV significantly increases a person's risk for developing long-term infection, chronic liver disease, cirrhosis or death.[1]

Current therapies for HCV typically provide sustained benefit in about half of patients with genotype 1 HCV, the most common strain of the virus.[2] If treatment is not successful and patients do not achieve an SVR, they remain at risk for progressive liver disease.[1] In a recent study, the risk of liver failure, cancer or death following unsuccessful HCV treatment was assessed at 23% after 4 years, and 43% after 8 years.[3]

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, cystic fibrosis, inflammation, autoimmune diseases, epilepsy, cancer, and pain. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

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

[1] Centers for Disease Control and Prevention. Hepatitis C Fact Sheet: CDC Viral Hepatitis. Available at: http://www.cdc.gov/hepatitis/HCV/PDFs/HepCGeneralFactSheet.pdf. Accessed, September 24, 2009.

[2] Strader DB, Wright T, Thomas DL, Seeff LB, AASLD practice guideline: diagnosis, management and treatment of hepatitis C. Hepatology: 2004(39):1147-1171.

[3] Veldt et al, "Sustained virologic response and clinical outcomes in patients with chronic hepatitis C and advanced fibrosis," Annals of Internal Medicine, 20 November 2007; 147: 677-684.

Special Note Regarding Forward Looking Statements

This press release contains forward-looking statements including (i) the expectation that the trial described in this press release will evaluate safety and SVR rates with multiple 12-week response-guided regimens of telaprevir/VX-222-based combination therapy, including two-drug regimens of telaprevir and VX-222 (ii) statements regarding the design of the trial, including the number of patients the Company expects to enroll, the primary endpoints, the expected treatment regimens, the response-guided design and the potential additional treatment arms; (iii) expectations regarding completing enrollment in the second quarter of 2010 and obtaining interim clinical data, including safety and viral kinetic data, from the trial in second half of 2010; (iv) Dr. Mueller's statements in paragraph 2 and 3 of this press release, including his statements regarding the telaprevir development program, the timing for submitting a New Drug Application for telaprevir and the trial announced today informing the future development path for telaprevir/VX-222-based combination therapy (v) statements regarding recent clinical trials supporting the evaluation of telaprevir/VX-222-based combination regimens and the expectations regarding the plasma exposures that are expected to be provided by the VX-222 doses selected for the clinical trial; and (vi) the expectation that Part B of the two part Phase 1b/2a trial of VX-222 will be initiated shortly. While the Company believes the forward-looking statements contained in this press release are accurate, those statements are subject to risks and uncertainties that could cause actual outcomes to vary materially from the outcomes referenced in the forward-looking statements. These risks and uncertainties include, among other things, the risks that efforts to develop telaprevir and VX-222 separately or in combination may not proceed due to technical, scientific, commercial, financial or other reasons, that clinical trials may not proceed as planned due to drug supply or patient enrollment issues, that additional clinical trials of telaprevir and VX-222 will not reflect the results obtained to date, that an adverse event profile for telaprevir or VX-222 could be revealed in further nonclinical or clinical studies that could put further development of telaprevir or VX-222 in jeopardy or adversely impact their therapeutic value, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the Company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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