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65% of People Whose Prior Treatment for Hepatitis C Was Unsuccessful Achieved SVR (Viral Cure) with Telaprevir-Based Therapy in Phase 3 REALIZE Study

-17% of people achieved SVR with pegylated-interferon and ribavirin alone in the control arm-
-Safety and tolerability results were consistent with prior Phase 3 studies-
-Completion of rolling New Drug Application submission on track for the fourth quarter 2010-

CAMBRIDGE, Mass., Sep 07, 2010 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that 65% of people overall achieved a sustained viral response (SVR or viral cure) with a telaprevir-based regimen in the pivotal Phase 3 REALIZE study, as compared to 17% of people in the control arm who received pegylated-interferon and ribavirin alone. REALIZE enrolled three groups of patients with genotype 1 hepatitis C who had undergone at least one prior treatment course with pegylated-interferon and ribavirin but did not achieve SVR: (1) those who relapsed, (2) those who achieved a partial response and (3) those who had almost no response, known as a null response. REALIZE is the only Phase 3 hepatitis C study to date of an investigational direct-acting antiviral therapy that was designed to evaluate all major subgroups of people whose prior treatment was unsuccessful, including those who had a null response. The safety and tolerability results were consistent with results from the other two Phase 3 studies of telaprevir. The REALIZE study was conducted by Vertex's collaborator, Tibotec.

"The REALIZE data represent a major milestone in the development of new treatments for hepatitis C, as patients who received telaprevir-based therapy had a viral cure rate almost four times greater than the cure rate in those treated with available medicines," said Stefan Zeuzem, M.D., Professor of Medicine and Chief of the Department of Medicine at the JW Goethe University Hospital, Frankfurt, Germany and Principal Investigator of the trial. "These results may provide hope to people who have not been cured and who are in need of new treatment options, including those with advanced liver disease."

"Along with results from ADVANCE and ILLUMINATE, the REALIZE data provide us with a strong understanding of telaprevir's potential role in helping many people with hepatitis C achieve a cure, regardless of their treatment history," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer for Vertex. "With these data, we look forward to completing our rolling New Drug Application submission for telaprevir later this year."

Overview of SVR Results

The primary endpoint of the study was SVR in each of the two telaprevir arms compared to the control arm, as well as across the three subgroups of people included in the study. One of the telaprevir treatment arms was designed to evaluate, for the first time, whether there was any further improvement in viral cure rates when delaying the start of telaprevir by four weeks, during which time patients received four weeks of pegylated-interferon and ribavirin alone, compared to a simultaneous start. The SVR rates between these two arms were similar and there was no clinical benefit to the telaprevir delayed start treatment arm in any of the subgroups of patients. The table below combines the two telaprevir arms compared to the control.

	Relapsers (n=354)	**Partial Responders (n=124)**	**Null Responders (n=184)**	**Overall (ITT) (n=662)**
Telaprevir-based Treatment Arms[+]	86%[*] (n=245/286)	57%[*] (n=55/97)	31%[*] (n=46/147)	**65%[*] (n=346/530)**
	Pooled Analysis: 78% (n=300/383)[**]			
Control Arm[++]	24% (n=16/68)	15% (n=4/27)	5% (n=2/37)	**17% (n=22/132)**
	Pooled Analysis: 21% (n = 20/95)[**]			

[*]Combined endpoint analysis: The SVR rates observed in the overall combined telaprevir-based arms were statistically significant when compared with the control arm ($p < 0.0001$). Additionally, the SVR rates observed in each of the three groups of patients evaluated were statistically significant when compared with the control arm (relapsers and partial responders ($p<0.0001$) and null responders ($p<0.001$)).

[+]Reflects SVR rates from the combined telaprevir-based treatment groups. There were two telaprevir-based treatment groups:

1. 12 weeks of telaprevir (750 mg, q8h), pegylated-interferon (Peg-IFN) & ribavirin (RBV), followed by 36 weeks of Peg-IFN & RBV alone or
2. 4 weeks of Peg-IFN & RBV alone followed by 12 weeks of telaprevir (750 mg, q8h), Peg-IFN & RBV, followed by 32 weeks of Peg-IFN & RBV alone

[++]12 weeks of placebo, Peg-IFN & RBV, followed by 36 weeks of Peg-IFN and RBV alone

[**]Supplemental analysis

Null Responder: Defined as a person who achieved a less than 2 $\log_{10}$ reduction in HCV RNA at week 12 of a prior course of therapy.

Relapser: Defined as a person whose hepatitis C virus was undetectable at the completion of at least 42 weeks of a prior course of therapy but whose virus became detectable during the follow-up period.

Partial Responder: Defined as a person who achieved at least a 2 $\log_{10}$ reduction at week 12, but whose hepatitis C virus never became undetectable by week 24 of a prior course of therapy.

Backgrounders on hepatitis C treatment response and the REALIZE study (including trial design diagram) can be found at http://investors.vrtx.com/press.cfm

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SVR rates for the telaprevir simultaneous start arm and the delayed start arm were 64% and 66%, respectively, overall, based on an intent-to-treat (ITT) analysis. For the primary analysis, the SVR rates for the telaprevir simultaneous start arm, delayed start arm and control arm, respectively, were 83%, 88% and 24% in relapsers ($p<0.0001$); 59%, 54% and 15% in partial responders, ($p<0.0001$); and 29%, 33% and 5% in null responders, ($p<0.001$).

Safety & Tolerability Results

The safety and tolerability results of the telaprevir-based regimens in the REALIZE study were consistent with results reported from the Phase 3 ADVANCE and ILLUMINATE studies. The most common adverse events, reported in any treatment arm during the telaprevir dosing periods and up to week 16 to account for the telaprevir delayed start arm in order of frequency, were fatigue, pruritis, headache, rash, flu-like symptoms, nausea and anemia, with the majority being mild to moderate. Of these, fatigue, pruritis, rash, flu-like symptoms, nausea and anemia were more common in the telaprevir-based treatment arms compared to control. Adverse events leading to discontinuation of all study drugs during the telaprevir dosing period and up to week 16 occurred in 4% of people in the combined telaprevir arms and 3% in the control arm during the same period. Discontinuation of all drugs due to anemia and rash during the telaprevir dosing period and up to week 16 occurred in 0.6% and 0.4% of patients, respectively, in the combined telaprevir arms, while discontinuation of all three drugs due to rash and anemia did not occur in the control arm during the same period. As in ADVANCE and ILLUMINATE, the use of erythropoiesis-stimulating agents (ESAs) was not allowed in this study.

Telaprevir is an investigational, oral inhibitor of HCV protease, an enzyme essential for viral replication, and is being developed by Vertex Pharmaceuticals in collaboration with Tibotec Pharmaceuticals and Mitsubishi Tanabe Pharma. With results from the three Phase 3 studies of telaprevir - ADVANCE, ILLUMINATE and REALIZE - Vertex is on track to complete its rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) in the fourth quarter of 2010.

Patient Demographics

REALIZE enrolled people with hepatitis C who did not achieve a viral cure after receiving at least one course of prior treatment with pegylated-interferon and ribavirin. Patients in the study were enrolled based on their response to prior treatment: 53% were prior relapsers, 19% were prior partial responders and 28% were prior null responders. In this study, 26% of patients overall had cirrhosis and 89% of patients overall had a high viral load (HCV RNA greater-than or equal to 800,000 IU/mL) when entering the study. Specifically in the null responder population, there were an even greater number of people with cirrhosis (33%) and high viral load (95%). Approximately 50% of patients were genotype 1a and 50% were genotype 1b.

About the Study

REALIZE was a pivotal Phase 3, randomized, double-blind, placebo-controlled study conducted in 662 people at more than 100 international clinical trial sites with the majority in Europe and North America. The study was designed to evaluate the efficacy, safety and tolerability of telaprevir-based regimens in people infected with genotype 1 chronic hepatitis C who did not achieve a viral cure after at least one prior treatment with interferon-based therapy. There were two telaprevir-based arms (simultaneous and delayed start) and one control arm. Patients were randomized 2:2:1 to the two telaprevir arms and the control arm, respectively.

The primary endpoint of the REALIZE study was SVR, defined as the proportion of people who had undetectable HCV RNA (<25IU/mL undetectable by Roche COBAS Taqman HCV test) 24 weeks after the end of all treatment. REALIZE was designed

to compare the SVR rates for each of the telaprevir-based regimens with the control arm, separately for the prior response subgroups of relapsers and non-responders (null and partial responders), and then for the two subgroups of non-responders. The secondary endpoint was to evaluate the safety and tolerability of telaprevir in combination with pegylated-interferon and ribavirin.

As in all Phase 3 studies of telaprevir, patients received no more than 12 weeks of telaprevir given in combination with pegylated interferon and ribavirin. In REALIZE, the telaprevir arms included 12 weeks of telaprevir in combination with pegylated-interferon and ribavirin with 36 weeks of pegylated-interferon and ribavirin alone for a total of 48 weeks of treatment.

About the Telaprevir Development Program

To date, more than 2,500 people with hepatitis C have received telaprevir-based therapy as part of Phase 2 studies and the Phase 3 ADVANCE, ILLUMINATE and REALIZE studies. Together, these studies enrolled people with genotype 1 hepatitis C who had not been treated for their disease previously as well as people who had been treated before but did not achieve a viral cure.

Vertex retains commercial rights to telaprevir in North America. Tibotec has rights to commercialize telaprevir in Europe, South America, Australia, the Middle East and certain other countries. Mitsubishi Tanabe Pharma has rights to commercialize telaprevir in Japan and certain Far East countries.

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.[2] According to a 2010 report from the Institute of Medicine, up to 3.9 million people in the United States have chronic hepatitis C and 75% of those infected are unaware of their infection.[3] Approximately 60 percent of genotype 1 patients who undergo an initial 48-week regimen with pegylated-interferon and ribavirin, the currently approved treatment regimen, do not achieve SVR, [4,5,6] or viral cure.[1]

Hepatitis C is spread through direct contact with the blood of infected people.[2] Though many people with hepatitis C may not experience symptoms, others may have symptoms such as fatigue, fever, jaundice and abdominal pain.[2] Chronic hepatitis C can lead to serious and life-threatening liver problems, including liver damage, cirrhosis, liver failure or liver cancer.[2] If treatment is not successful and a person does not achieve a viral cure, they remain at risk for progressive liver disease.[7,8,9,10,11] In the United States, hepatitis C is the leading cause of liver transplantations and is reported to contribute to 4,600 to 12,000 deaths annually.[8] The majority of people with hepatitis C were born between 1946 and 1964, accounting for two of every three people with chronic hepatitis C.[11] By 2029, total annual medical costs in the U.S. for people with hepatitis C are expected to more than double, from $30 billion in 2009 to approximately $85 billion.[11]

Additional resources for media, including a hepatitis C backgrounder and glossary of common terms, are available at: http://investors.vrtx.com/press.cfm

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 is a registered trademark of the GlaxoSmithKline group of companies.

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Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements, including statements regarding (i) the Company being on track to complete the NDA for telaprevir in the fourth quarter of 2010 and (ii) the REALIZE data providing the Company with a strong understanding of telaprevir's potential role in helping many people with hepatitis C achieve a cure, regardless of their treatment history. While the Company 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. Those risks and uncertainties include, among other things, that the Company could experience unforeseen delays in submitting the NDA for telaprevir and/or obtaining approval to market telaprevir; that there may be varying interpretations of the data from the telaprevir clinical trials; that future outcomes from clinical trials of telaprevir may not be favorable; and that future scientific, clinical, competitive or other market factors may adversely affect the potential for telaprevir-based combination therapy and the 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. The Company disclaims any obligation to update the information contained in this press release as new information becomes available.

Investor Conference Call Today at 4:30 p.m. ET

Vertex Pharmaceuticals will host a conference call and webcast today, September 7, at 4:30 p.m. ET. This call and webcast will be broadcast via the Internet at **www.vrtx.com/finances**. It is suggested that webcast participants go to the web site at least 10 minutes in advance of the call to ensure that they can access the slides. The link to the webcast is available on the Events & Presentations button. To listen to the call on the telephone, dial (888) 634-7543 (U.S. and Canada) or (719) 457-2573 (International) and use conference ID number ID 5433422. Vertex is also providing a podcast MP3 file available for download on the Vertex website at www.vrtx.com.

The call will be available for replay via telephone commencing September 8, 2010 at 8:00 p.m. ET running through September 22, 2010 at 5:00 p.m. ET. To listen to the replay dial (888) 203-1112 (U.S. and Canada) or (719) 457-0820 (International) and use conference ID number 5433422. Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. on September 15, 2010.

New York City Investor and Analyst Webcast Tomorrow, September 8, at 8:00 a.m. ET

Vertex Pharmaceuticals will also webcast its investor and analyst meeting from New York City on Wednesday, September 8, 2010 at 8:00 a.m. ET. This webcast will be broadcast via the Internet at **www.vrtx.com/finances**. The link to the webcast is available on the Events & Presentations button. The New York City webcast will not be available via telephone. It is suggested that webcast participants go to the web site at least 10 minutes in advance of the call to ensure that they can access the slides.

(VRTX-GEN)

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Vertex Pharmaceuticals Incorporated
Media:
Zachry Barber, 617-444-6992
or
Amy Pasqua, 617-444-6992
or
Investors:
Michael Partridge, 617-444-6108
or
Lora Pike, 617-444-6755
or
Matthew Osborne, 617-444-6057