



Pharmasset Announces Results of a 28-day Phase 2a Study with PSI-7977 for the Treatment of Chronic Hepatitis C Infection

--94% or 93% of patients achieved undetectable HCV RNA levels following 28 days of treatment with PSI-7977 200mg or 400mg QD, respectively, in combination with Pegasys(R) and Copegus(R) --Safety and tolerability across all doses were comparable to placebo administered with Pegasys(R) and Copegus(R) -- Conference call scheduled for Tuesday, May 4, 2010 at 8:00 AM ET (US)

PRINCETON, N.J., May 4, 2010 /PRNewswire via COMTEX News Network/ -- Pharmasset, Inc. (Nasdaq: VRUS) announced today the efficacy and preliminary safety results from its 28 day phase 2a study with PSI-7977 dosed once daily (QD) in combination with Pegasys(R) (peginterferon alfa-2a) and Copegus(R) (ribavirin), the current standard of care (SOC) in patients with hepatitis C virus (HCV) genotype 1 who were naïve to antiviral therapy. PSI-7977 is one of Pharmasset's investigational nucleotide analogs.

In this study, PSI-7977 demonstrated potent short term antiviral activity and was generally safe and well tolerated. All patients receiving active PSI-7977 demonstrated continuous and substantial declines in HCV RNA with no viral breakthrough during 28 days of therapy at any dose.

"We are encouraged by the emerging clinical profile of PSI-7977, which was well-tolerated by the patients in this study, and which confirms the consistently high RVR rates of these nucleoside and nucleotide analogs," stated M. Michelle Berrey, M.D., MPH, Chief Medical Officer of Pharmasset. "Our platform technology continues to generate nucleoside/tide analogs with a high degree of efficacy, high barrier to resistance, and a safety profile that we believe differentiates them from other classes of direct acting antivirals (DAA). We plan to quickly progress PSI-7977 into phase 2b studies starting in the fourth quarter 2010, to generate longer term efficacy and safety data."

Intent-to-Treat (ITT) 28-day RVR data from the trial are summarized as follows:

Study Arm	N	Mean decrease in HCV RNA (log ₁₀ IU/mL) at Day 28	Percentage of Patients with HCV RNA below LOD (<15 IU/mL) at Day 28
100mg PSI-7977 QD + SOC	16	-5.3	88% (14/16)
200mg PSI-7977 QD + SOC	18	-5.1	94% (17/18)
400mg PSI-7977 QD + SOC	15	-5.3	93% (14/15)
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Placebo + SOC	14	-2.8	21% (3/14)
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Potent and consistent antiviral activity was demonstrated in this study following 28 days of treatment with PSI-7977 in combination with SOC. Patients receiving PSI-7977 100mg QD with SOC achieved a mean 5.3 log₁₀ IU/mL decrease in HCV RNA and 88% (14 of 16) had HCV RNA levels below the limit of detection (<15 IU/mL), or a Rapid Virologic Response (RVR). Following 28 days with PSI-7977 200mg QD with SOC, patients achieved a mean 5.1 log₁₀ IU/mL decrease in HCV RNA and 94% (17 of 18) achieved an RVR. Following 28 days with PSI-7977 400mg QD with SOC, patients achieved a mean 5.3 log₁₀ IU/mL decrease in HCV RNA and 93% (14 of 15) achieved an RVR. In the control group, following 28 days of treatment with placebo and SOC, patients achieved a mean 2.8 log₁₀ IU/mL decrease in HCV RNA and 21% (3 of 14) achieved an RVR. The baseline HCV RNA for all patients enrolled in the study was approximately 6.5 log₁₀ IU/mL, and was similar across all treatment arms. Patients were stratified by IL28B status to ensure balance across cohorts and no patient was excluded based on their status.

Preliminary Safety Summary

Preliminary safety and tolerability for the 28 day treatment period were similar for PSI-7977 with SOC compared to placebo with SOC. There were no serious adverse events reported during the 28 day treatment period, and no adverse events leading to treatment discontinuation. All adverse events reported were of mild to moderate intensity, of which the majority were mild. A similar proportion of patients in each cohort reported adverse events, with the most common adverse events reported as fatigue, nausea, and arthralgias. The frequency and severity of these adverse events, as well as general body system observations, were similar to clinical experience with the standard of care. There were no dose-related changes in safety laboratory assessments, vital signs or ECGs. A dose-dependent decrease in serum ALT was observed coincident with HCV RNA decline.

Overall, there were no drug-related discontinuations, no serious adverse events, and no dose-related trends in adverse events or laboratory abnormalities as compared to placebo with standard of care.

Full analyses of safety, efficacy, and resistance will be presented at a scientific meeting later in 2010.

About Hepatitis C

Hepatitis C is a blood-borne infectious disease of the liver and is a leading cause of chronic liver disease and liver transplants. The WHO estimates that nearly 180 million people worldwide, or approximately 3% of the world's population, are infected with hepatitis C virus (HCV). The CDC has reported that almost three million people in the United States are chronically infected with HCV.

Conference Call

Pharmasset will host a conference call at 8:00 AM ET (US) on Tuesday, May 4, 2010 to discuss these PSI-7977 phase 2a study results.

Dial-in Information:

US/Canada Toll-Free callers: +1 (877) 771-7028

US/Canada Toll or International Toll callers: +1 (973) 200-3092

Live audio of the conference call will be simultaneously broadcast over the internet via a webcast. To access the live webcast, log on to the "Events & Presentations" section of the Investor Center on Pharmasset's corporate website at <http://investor.pharmasset.com/events.cfm> .

Please connect to the company's website at least ten minutes prior to the start of the presentation to ensure adequate time for a reliable connection and any software download that may be necessary to listen to the webcast. The archived replay of the webcast will be available on the Pharmasset website for two weeks following the conference call.

About the Phase 2a trial

The Phase 2a trial enrolled 63 chronic hepatitis C virus infected patients who have not been treated previously. The primary goal of the study was to determine the safety and tolerability of PSI-7977 in combination with SOC. The primary efficacy endpoint of the trial was the proportion of patients who achieve an RVR, defined as HCV RNA below the limit of detection (<15 IU/ml) as measured by Roche HCV TaqMan assay 28 days after the initiation of treatment. Patients were randomized to receive one of four treatments:

- 16 subjects taking PSI-7977 100mg QD in combination with SOC for four weeks, followed by 44 weeks of SOC
- 18 subjects taking PSI-7977 200mg QD in combination with SOC for four weeks, followed by 44 weeks of SOC
- 15 subjects taking PSI-7977 400mg QD in combination with SOC for four weeks, followed by 44 weeks of SOC
- A control arm of 14 subjects taking placebo with SOC for four weeks, followed by 44 weeks of SOC

About Pharmasset

Pharmasset is a clinical-stage pharmaceutical company committed to discovering, developing, and commercializing novel drugs to treat viral infections. Pharmasset's is focusing primarily on the development of oral therapeutics for the treatment of hepatitis C virus (HCV) and, secondarily, on the development of Racivir(TM) for the treatment of human immunodeficiency virus (HIV). Our research and development efforts focus on nucleoside/tide analogs, a class of compounds which act as alternative substrates for the viral polymerase, thus inhibiting viral replication. We currently have four clinical-stage product candidates. RG7128, a cytosine nucleoside analog for chronic HCV infection, is in two Phase 2b clinical trials in combination with Pegasys (R) plus Copegus(R) and is also in the INFORM studies, the first series of studies designed to assess the potential of combinations of small molecules without pegylated interferon and ribavirin to treat chronic HCV. These clinical studies are being conducted through a strategic collaboration with Roche. Our other, unpartnered, clinical stage candidates for the treatment of HCV include PSI-7977, a uracil nucleotide analog that is in a Phase 2a trial, and PSI-938, a guanine nucleotide analog that is in a Phase 1 trial. We also have in our pipeline an additional guanine nucleotide analog, PSI-661, in advanced preclinical development for the treatment of HCV. Racivir, for the treatment of HIV, has completed a Phase 2 clinical trial.

Pegasys(R) and Copegus(R) are registered trademarks of Roche.

Contact

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Forward-Looking Statements

Pharmasset "Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995: Statements in this press release regarding our business that are not historical facts are "forward-looking statements" that involve risks, uncertainties and other important factors, including, without limitation, the risk that adverse events could cause the cessation or delay of any of the ongoing or planned clinical trials and/or our development of our product candidates, the risk that the results of previously conducted studies involving our product candidates will not be repeated or observed in ongoing or future studies involving our product candidates, the risk that our collaboration with Roche will not continue or will not be successful, and the risk that any one or more of our product candidates will not be successfully developed and commercialized. For a discussion of these and other risks, uncertainties and important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended September 30, 2009 and our Quarterly Report on Form 10-Q for the period ended December 31, 2009 filed with the Securities and Exchange Commission and discussions of potential risks, uncertainties and other important factors in our subsequent filings with the Securities and Exchange Commission.

SOURCE Pharmasset, Inc.

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