Presidio Pharmaceuticals Successfully Completes Phase 1 Proof-of-Concept for PPI-668, its Potent HCV NS5A Inhibitor, in Hepatitis C Patients with Genotype-1 Infection

San Francisco, CA – June 26, 2012 – Presidio Pharmaceuticals, Inc. announced today successful completion of Phase 1b clinical testing of its lead HCV NS5A inhibitor in patients with HCV genotype-1 infection, with positive efficacy and safety observations supporting advancement of PPI-668 to Phase 2 combination studies.

The randomized, blinded Phase 1b trial of PPI-668 involved sequential cohorts of treatment-naïve HCV genotype-1 patients who received oral doses of PPI-668 of 40, 80, 160 or 240 mg, once daily for three consecutive days. Within each 10-patient cohort, patients were randomized 8:2 to PPI-668 or placebo.

In all of the Phase 1b dose cohorts, PPI-668 was well tolerated with no serious or severe adverse events, no premature treatment discontinuations and no apparent pattern of treatment-related clinical side effects or laboratory abnormalities.

The Phase 1 clinical results indicate that PPI-668 had a favorable pharmacokinetic (PK) profile that included rapid achievement of high (micromolar) plasma levels, prolonged maintenance of potentially effective levels between doses, and achievement of steady-state pharmacokinetics after the first dose.

The Phase 1b efficacy observations indicated consistently rapid, marked reductions in patients' serum viral load (HCV RNA levels), that were dose-related. Patients' HCV RNA reductions typically exceeded 3 log10 IU/ml (99.9%) by Day 2. During the 3-day treatment period, mean maximal HCV RNA reductions for the 4 dosing groups were:

- 3.2 $\log_{10}$ IU/mL in the 40 mg dose group
- 3.5 $\log_{10}$ IU/mL in the 80 mg dose group
- 3.5 $\log_{10}$ IU/mL in the 160 mg dose group
- 3.7 $\log_{10}$ IU/mL in the 240 mg dose group

There was only one minimal-responder in the trial. A patient in the 240 mg dose group was found to be fully resistant at baseline with 100% of this patient's pre-treatment HCV RNA containing 3 genetically linked NS5A resistance mutations. This patient was excluded from the efficacy analysis of the 240 mg cohort, since he was pre-resistant and could not contribute to dose-response inferences.

Five other patients with detectable resistance mutations at baseline, including those harboring the relatively common L31M variant, responded well to PPI-668 treatment, with multi-log HCV RNA reductions.

A protocol amendment has been completed to explore the pan-genotypic clinical efficacy of PPI-668 in HCV genotype-2a/3a patients. Recruitment is currently underway for this added cohort.

"The rapid 3.5 to 3.7 $\log_{10}$ HCV RNA reductions observed with PPI-668 at the three higher dose levels and the encouraging safety profile support advancement of PPI-668 to Phase 2 combination studies with other promising HCV antiviral agents", said Nathaniel A. Brown, M.D., Presidio's Chief Medical Officer. "The PK profile of PPI-668 appears to be a major factor in its efficacy profile, with rapid achievement of potentially effective plasma levels and with inter-dose plasma concentrations exceeding those needed to inhibit both wild-type HCV and many naturally-occurring HCV variants."

Detailed results of the completed trial are expected to be presented at a scientific meeting in the fall of 2012.

About Hepatitis C

Chronic hepatitis C is a progressive inflammatory liver disease caused by chronic infection with the hepatitis C virus (HCV). Approximately 170 to 200 million persons have chronic HCV infection worldwide, resulting in more than 350,000 deaths annually.

The current standard treatment for patients with hepatitis C genotype-1 infection in the United States and several other countries is combined administration of pegylated-interferon-alfa, ribavirin, and an HCV protease inhibitor. This treatment is characterized by incomplete efficacy and severe side effects in some patients.

There is a continuing need for all oral, more consistently effective and better tolerated antiviral combinations for HCV infection, regardless of HCV genotype, patient genetic factors, or disease stage.

About Presidio

Presidio Pharmaceuticals, Inc. is a San Francisco-based clinical stage specialty pharmaceutical company focused on the discovery and development of novel oral antiviral therapeutics. For more information, please visit our website at: www.presidiopharma.com

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