Presidio Pharmaceuticals Announces Phase 1a-1b Clinical Results with PPI-668, a Potent Pan-genotypic HCV NS5A Inhibitor

San Francisco, CA – April 19, 2012 – Presidio Pharmaceuticals, Inc. announced results today from a Phase 1a-1b clinical trial of PPI-668, a potent, pan-genotypic HCV NS5A inhibitor being developed for the treatment of patients with chronic hepatitis C.

PPI-668 is Presidio's second NS5A inhibitor to progress to clinical testing after Phase 1 clinical evaluation of PPI-461 (results previously reported at the annual meetings of the American Association for the Study of Liver Diseases (AASLD) in 2010 and 2011). Compared to PPI-461, PPI-668 exhibits more potent activity *in vitro* against HCV genotypes 3a and 6a and has a superior resistance profile.

The two-part Phase 1a-1b clinical trial is being conducted at six clinical centers in the United States, New Zealand, and Australia. This study is designed to evaluate the dose-related safety and pharmacokinetics (PK) of PPI-668 in healthy adult volunteers (Part I), and then the safety, PK, and dose-related antiviral effects in treatment-naïve adult patients with HCV genotype-1 infection (Part II). Part I of the study has been successfully completed. In Part II, clinical proof-of-concept has been established with regard to the initial safety and efficacy of PPI-668 in patients with HCV genotype-1 infection.

In Part I, the study sequentially assessed single doses of 80, 160, and 320 mg in three cohorts of 8 volunteers each, randomized 6:2 to PPI-668 capsules or matching placebo; a fourth cohort received 320 mg once daily (QD) for five days. PPI-668 was well-tolerated at all tested doses with no pattern of clinical side effects or laboratory abnormalities related to treatment. Substantial blood levels of PPI-668 were achieved rapidly, and concentrations of PPI-668 that are potentially inhibitory for all major HCV genotypes were maintained for more than 24 hours, supporting evaluation of QD dosing for patients.

In Part II of the study, sequential cohorts of HCV genotype-1 patients received QD doses of 80, 160 or 240 mg for three consecutive days. Within each cohort, 10 patients were randomized 8:2 to PPI-668 or placebo. The first two cohorts have completed study treatment, with no premature discontinuations, no serious or severe clinical adverse events, and no apparent pattern of treatment-related clinical side effects or laboratory abnormalities. Partial data are available for the third dosing cohort (8/10 patients).

Rapid, marked anti-HCV efficacy has been observed for all three PPI-668 dose levels tested, with mean maximal HCV RNA reductions ranging from 3.5 to 3.7 $\log_{10}$ IU/mL, corresponding to viral load reductions exceeding 99.9% in the first 2-3 days of PPI-668 treatment. With proof-of-concept established for PPI-668 in genotype-1 hepatitis C patients, recruitment is currently underway to evaluate PPI-668 treatment in HCV-infected patients with genotypes 2a and 3a.

These Phase 1a-1b results support advancement to Phase 2 clinical trials in combination with other oral HCV inhibitors.

About Hepatitis C

Chronic hepatitis C is a persistent, potentially progressive inflammatory liver disease caused by chronic infection with the hepatitis C virus (HCV). Worldwide, there are an estimated 130 to 170 million persons with chronic HCV infection, resulting in more than 350,000 deaths each year.

The current standard treatment for patients with hepatitis C genotype-1 infection in the United States is combined administration of pegylated-interferon-alfa, ribavirin, and an HCV protease inhibitor. This multi-drug treatment is characterized by incomplete efficacy for HCV genotype-1 patients, variations in efficacy according to patients' underlying human genetic factors, no established efficacy for patients infected with other HCV genotypes, severe side effects in some patients, and dosing inconveniences. In countries where the current HCV protease inhibitors are not yet regulatory-approved, standard treatment is only pegylated-interferon-alfa plus ribavirin, which is less effective.

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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