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Presidio Pharmaceuticals Announces a High Rate of Virologic Response in an Ongoing Phase 2 Hepatitis C Trial of a New All-Oral Combination of Presidio's PPI-668 with Boehringer Ingelheim's Faldaprevir and Deleobuvir

San Francisco, CA – November 2, 2013 – Presidio Pharmaceuticals, Inc. announced today positive preliminary results from an ongoing Phase 2 clinical trial of an investigational, interferon-free, all-oral combination treatment for patients with chronic hepatitis C virus (HCV) infection. The collaborative trial is evaluating Presidio's pan-genotypic HCV NS5A inhibitor (PPI-668) in combination with Boehringer Ingelheim's HCV protease inhibitor faldaprevir (BI 201335) and non-nucleoside HCV polymerase inhibitor deleobuvir (BI 207127), with and without ribavirin (RBV).

An abstract with interim data from this trial was accepted as a late-breaker presentation at the annual meeting of the American Association for the Study of Liver Diseases (AASLD), which is currently ongoing (Oct 31-Nov 5) in Washington DC. The interim data will be presented in an AASLD poster session on Monday, November 4.

Presidio is conducting the trial at a clinical site in the U.S., in close collaboration with Boehringer Ingelheim. Both companies continue to retain all rights to their respective compounds during this collaboration.

The reported clinical trial is a Phase 2 study of 12 weeks of treatment of hepatitis C patients with the investigational three-drug regimen, with and without ribavirin. The patients enrolled in this trial represent a relatively difficult-to-treat patient population. All patients have HCV genotype-1a infection, and most (64%) exhibit the less-responsive CT or TT genotypes at the IL28B human gene locus. The primary endpoint of the trial is sustained virologic response (non-detectable HCV RNA) 12 weeks post-treatment (called "SVR12"). At the time of the preliminary analysis all patients had completed four or more weeks of treatment.

The trial assesses three variations of the three-drug regimen, using three 12-patient cohorts (total 36 patients). The first 24 eligible patients were randomly assigned (1:1) to Cohort 1 or Cohort 2, which correspond to the following oral treatment regimens:

Cohort 1 (12 patients): PPI-668 200 mg once daily, faldaprevir 120 mg once daily, and deleobuvir 600 mg twice daily, plus weight-based ribavirin twice daily.

Cohort 2 (12 patients): PPI-668 200 mg once daily, faldaprevir 120 mg once daily, and deleobuvir 400 mg twice daily, plus weight-based ribavirin twice daily.

After the initial two-week efficacy and safety results for the first 12 enrolled patients in Cohorts 1 and 2 met pre-established efficacy and safety criteria, a third 12-patient cohort was enrolled to assess a ribavirin-free regimen:

Cohort 3 (12 patients): PPI-668 200 mg once daily, faldaprevir 120 mg once daily, and deleobuvir 600 mg twice daily, with no ribavirin.

The study, which is ongoing, has been fully-enrolled for two months and includes assessments of safety (clinical adverse events and laboratory abnormalities) and antiviral efficacy (HCV RNA reductions, and clearance to non-detectable levels) using the Roche TaqMan™ assay (v2.0, High Pure system). This assay has a lower limit of HCV RNA quantification (LLOQ) of 25 IU/mL and an estimated lower limit of HCV RNA detection (LLOD) of 10 IU/mL.

The study also includes assessments of the pharmacokinetics (PK) of each of the three study drugs, to determine whether there are PK interactions when the three drugs are co-administered in a combination regimen. In addition, viral resistance analyses are being conducted for all patients to assess resistance for all 3 study drugs in pre-treatment (baseline) sera and in serum samples obtained during any observed virologic rebounds in study patients.

The AASLD poster presentation includes more advanced interim study data than the submitted abstract. The following key results are described in the poster:

- Treatment with the investigational oral combination regimen of PPI-668 + faldaprevir + deleobuvir has consistently produced rapid virologic responses, with or without ribavirin:
    - 35/36 (97%) patients achieved HCV RNA <25 IU/mL (81% < LLOD) by week 4

- To date, all 17 patients who have completed 12 weeks of treatment have non-detectable HCV RNA (<LLOD) at the end of treatment (week 12)

- 13 patients (of the 17 treatment completers to date) have reached their 4 week post-treatment visit, and all 13 exhibit sustained virologic responses at this time point (SVR4)

- Despite potential resistance-associated substitutions and polymorphisms detected in HCV RNA samples of 20 patients at baseline, 19 of these 20 patients have responded well to study treatment to date. Only one patient has developed viral breakthrough. This patient was found to have multiple NS5A resistance substitutions and a polymorphic NS5B substitution in his pre-treatment (baseline) serum. He had an initial HCV RNA response but then experienced confirmed viral breakthrough on treatment by week 5, with his breakthrough sera revealing the emergence of high-level resistance to all three study drugs.

- The resistance-related assessments of HCV RNA from patients' baseline sera indicated that 12 patients had pre-existing Q80K polymorphisms in the HCV NS3 protease gene of their amplified HCV RNA. Notably, all of these patients have had good virologic responses to date, indicating that Q80K NS3 baseline polymorphisms did not affect patients' responses to this faldaprevir-containing study regimen.

- Safety observations in the study to date indicate that mild to moderate rashes and gastrointestinal side effects are common but are usually manageable. The overall safety profile appears similar to observations in previous studies of faldaprevir and deleobuvir. Only one patient has discontinued study treatment prematurely for adverse events. This patient had achieved non-detectable HCV RNA levels before he discontinued treatment at week 9, and his HCV RNA remains non-detectable post-treatment (3 weeks, to date). He will be followed on-study to determine whether he achieves sustained viral clearance despite his shorter treatment.

- The ribavirin-free cohort (Cohort 3) appears to have a better tolerability profile than the RBV-containing cohorts, with preliminary data suggesting similar initial antiviral efficacy

"With the potent antiviral activities and non-overlapping resistance profiles of PPI-668, faldaprevir, and deleobuvir, this Phase 2 collaborative study with Boehringer Ingelheim has produced encouraging preliminary data in a relatively difficult-to-treat HCV patient population. The current data indicate that this investigational, all-oral, triple therapy may have a high degree of virologic efficacy with a 12-week treatment duration, potentially without the use of ribavirin and regardless of patients' IL28B genotype. In the coming months we look forward to additional study data regarding sustained virologic responses 12 and 24 weeks post-treatment," said Dr. Nathaniel Brown, Presidio's Chief Medical Officer.

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 combination treatment is characterized by incomplete efficacy and a variety of potentially severe side effects.

Due to the efficacy limitations and tolerance issues for interferon-based therapies, and the continuing progression of underlying liver damage in the current large population of hepatitis C patients, there is a continuing need for more effective, better-tolerated, interferon-free combination therapies for HCV infection that can be orally administered with convenient, relatively short dosing schedules.

About PPI-668

PPI-668 is a potent, pan-genotypic, once-daily, investigational NS5A inhibitor. In earlier clinical studies in healthy volunteers and HCV-infected patients, PPI-668 has been well-tolerated to date with no serious or severe adverse events and no apparent pattern of treatment-related clinical side effects or laboratory abnormalities. In a clinical study of PPI-668 short-term monotherapy in patients with HCV genotype-1 infection, average dose-related HCV viral load reductions of 3.5 to 3.8 $\log_{10}$ (greater than 99.9%) were achieved in 1-2 days.

About faldaprevir (BI 201335)

Falaprevir is an investigational, potent next-generation once-daily HCV protease inhibitor for the treatment of HCV genotype-1 infection, which is designed to effectively target the HCV reservoir within the liver. Faldaprevir in combination with deleobuvir (BI 207127) and ribavirin produced a high rate of sustained clearance of detectable HCV in patients with HCV genotype-1b infection, without the use of interferon (SOUND-C2 and SOUND-C3 studies).

About deleobuvir (BI 207127)

Deleobuvir is an investigational, potent twice-daily non-nucleoside inhibitor of the HCV polymerase, that inhibits HCV genotype-1 replication and which, when combined with

faldaprevir and other DAAs for HCV infection, has the potential to produce sustained clearance of detectable HCV in patients with HCV genotype-1 infection without the use of interferon, as demonstrated in two recent Phase 2 trials (SOUND-C2 and SOUND-C3 studies).

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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