Soleno Therapeutics Presents Updated Safety and Efficacy Data from Clinical Trial of DCCR for Treatment of Prader-Willi Syndrome

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Updated Data Highlighted in Poster Presentation at 10th International Meeting of Pediatric Endocrinology

Phase III Study Expected to Begin by End of 2017

REDWOOD CITY, Calif., Sept. 15, 2017 (GLOBE NEWSWIRE) -- Soleno Therapeutics, Inc. (NASDAQ:SLNO), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, announced today that updated safety and efficacy data from the pilot clinical trial of Diazoxide Choline Controlled Release Tablet (DCCR) for the treatment of Prader-Willi syndrome (PWS) were the subject of a poster presentation at the 10th International Meeting of Pediatric Endocrinology, being held September 14-17 in Washington, D.C. The data were presented by Virginia Kimonis, M.D., Division of Genetics and Metabolism, School of Medicine, University of California, Irvine, and Principal Investigator of the trial.

The purpose of the study was to assess the safety and efficacy of multiple dose levels of DCCR in subjects ages 10-22 years with genetically-confirmed PWS. The study consisted of a 10-week open-label treatment phase, during which subjects were escalated from 1.5 mg/kg to a maximum of 5.1 mg/kg of DCCR and then treated stably for the remainder of the phase. Subjects with an improvement in hyperphagia and/or an increase in resting energy expenditure during the open-label phase were classified as responders and were eligible to enter a 4-week double-blind placebo-controlled withdrawal treatment phase, in which they were randomized to DCCR or placebo. Thirteen subjects were enrolled; 11 subjects completed the open-label phase, were classified as responders and randomized into the double-blind phase.

Key efficacy results included a significant improvement from baseline in hyperphagia (mean baseline score = 15 out of possible scores of 0-34) starting within two weeks of DCCR treatment. As previously reported, a highly statistically significant reduction (-4.32, p=0.0055) was seen in hyperphagia from baseline to the end of the open-label treatment phase. In addition, greater improvement in hyperphagia from baseline was observed in those subjects with moderate to severe hyperphagia who received DCCR doses ≥ 4.2 mg/kg (the planned population and target dose for the Phase III study) with a reduction of -7.83 (n =3). A significant improvement in the number of subjects reporting one or more aggressive and destructive behaviors was seen (p=0.006).

During the open-label treatment phase, a mean decrease in body fat mass (p=0.02) and increases in lean body mass (p=0.003) and lean body mass / fat mass ratio (p=0.004) were seen. These changes were associated with a statistically significant reduction in waist circumference (-3.45 cm, p=0.006), consistent with the loss of visceral fat. Leptin levels were significantly decreased (p=0.007) from baseline, while ghrelin levels remained unchanged (p=0.93).

The safety profile of DCCR in this study was consistent with the known profile of diazoxide. The most commonly reported treatment-emergent adverse events observed are also common in PWS patients, including glycemic impacts, peripheral edema, upper respiratory tract infections, constipation, and somnolence. Insulin sensitivity as measured by reduction HOMA-IR was improved.

“DCCR treatment of adolescent and adult PWS patients in this study positively impacted a number of the highest priority unmet needs in the disease, including hyperphagia and body fat,” said Dr. Kimonis. “DCCR has the potential to become a safe and effective therapeutic option for PWS patients and their families. I look forward to the further clinical evaluation of DCCR in PWS in a Phase III clinical trial.”

“The updated analyses of the data from the pilot study provide further evidence of the efficacy of DCCR in patients with PWS,” said Anish Bhatnagar, Chief Executive Officer of Soleno Therapeutics. “We are finalizing the design of a randomized, double-blind placebo-controlled study that will treat approximately 100 patients. The dose response data obtained from this study will help inform dosing in and design of the upcoming Phase III trial.”

DCCR has Orphan Designation for the treatment of PWS in the U.S.

About PWS

PWS is a rare and complex genetic neurobehavioral/metabolic disorder affecting appetite, growth, metabolism, cognitive function and behavior. The committee on genetics of the American Academy of Pediatrics states PWS affects both genders equally and occurs in people from all geographic regions: its estimated incidence is one in 15,000 to 25,000 births. This disorder is typically characterized by hyperphagia, a chronic feeling of insatiable hunger, behavioral problems, cognitive disabilities, low muscle tone, short stature (when not treated with growth hormone), the accumulation of excess body fat, developmental delays, and incomplete sexual development. Hyperphagia, in the absence of effective limitations to access to food, can lead to morbid obesity. In a global survey conducted by the Foundation for Prader-Willi Research, 96.5% of respondents (parent and caregivers) rated hyperphagia, which is the unrelenting hunger that severely diminishes the quality of life for patients and their families, as the most important or a very important symptom to be relieved by a new medicine. There are currently no approved therapies to treat the hyperphagia/appetite, metabolic, cognitive function, or behavioral aspects of the disorder.

About Diazoxide Choline Controlled-Release Tablet

Diazoxide choline controlled-release tablet is a novel, proprietary controlled-release, crystalline salt formulation of diazoxide, which is administered once-daily. The parent molecule, diazoxide, as an oral suspension, has been used for decades in thousands of patients in a few rare diseases in neonates, children and/or adults, but not in PWS. Soleno conceived of and is pursuing an extensive patent portfolio relating to various aspects of the therapeutic use of diazoxide and DCCR in patients with PWS. The DCCR development program is supported by data from two completed Phase II clinical studies and five completed Phase I clinical studies in various metabolic indications, as well as a pilot study in PWS patients. In the PWS pilot

study, DCCR showed promise in addressing the hallmark symptoms of PWS, most notably hyperphagia.

About Soleno Therapeutics, Inc.
Soleno Therapeutics, Inc. is focused on the development and commercialization of novel therapeutics for the treatment of rare diseases. The company is currently advancing its lead candidate, DCCR, a once-daily oral tablet for the treatment of PWS, into a Phase III clinical development program at the end of 2017. Soleno, through its wholly-owned subsidiary, Capnia, Inc., continues to market Capnia's innovative medical device, the CoSense® End-Tidal Carbon Monoxide (ETCO) monitor, which measures ETCO and is used by hospitals to detect hemolysis in newborns. It is expected that this product will be monetized and will not be a focus for the company in the long term.

For more information, please visit www.soleno.life.

Forward-Looking Statements
This press release contains forward-looking statements that are subject to many risks and uncertainties. Forward-looking statements include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our ability to initiate the Phase III clinical development program of DCCR in PWS by the end of 2017. We may use terms such as "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "approximately" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained herein, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this presentation. As a result of these factors, we cannot assure you that the forward-looking statements in this presentation will prove to be accurate. Additional factors that could materially affect actual results can be found in Capnia's Form 10-Q filed with the Securities and Exchange Commission on August 11, 2017, including under the caption titled "Risk Factors." Capnia expressly disclaims any intent or obligation to update these forward-looking statements, except as required by law.

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