Soleno Therapeutics Announces Expansion of Ongoing Phase III Trial of DCCR in Prader-Willi Syndrome to Include Younger Patients

September 6, 2018

Amendment Lowers Minimum Age Inclusion Criteria to Four Years, from Eight Years, to Better Reflect Demographics of PWS Population

Top-line Data from Phase III Trial Expected in First Half of 2019

REDWOOD CITY, Calif., Sept. 06, 2018 (GLOBE NEWSWIRE) -- Soleno Therapeutics, Inc. ("Soleno") (NASDAQ: SLNO), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, today announced a protocol amendment to its ongoing Phase III trial, DESTINY PWS, studying Diazoxide Choline Controlled-Release (DCCR) tablets in Prader-Willi syndrome (PWS). The protocol amendment reduces the age of inclusion to children as young as four years of age, from eight years of age previously. The amendment is the result of U.S. Food and Drug Administration (FDA) feedback regarding Soleno's Initial Pediatric Investigational Plan. Hyperphagia, the central characteristic of PWS, is present in nearly all PWS patients by age 8, and can occur in children as young as four years of age. The protocol amendment has been approved by an independent Institutional Review Board and is in the process of being implemented at clinical trial sites.

"The hallmark symptom of PWS is hyperphagia, an unrelenting hunger that causes significant challenges to both patients and their caregivers, which manifests in early childhood," said Jennifer L. Miller, M.D., Associate Professor in the Division of Pediatric Endocrinology at the University of Florida and a Principal Investigator in the Soleno study. "Managing hyperphagia in PWS patients is a critical unmet need for which there are no currently approved treatments. Because the parent molecule in DCCR has been approved for use in neonates through adults for other conditions, and at much higher daily doses than are being used in this study, its safety profile is well understood. We are pleased to continue evaluating this promising product candidate in PWS patients with hyperphagia in all ages in the ongoing Phase III trial."

"This important protocol change to the ongoing Phase III trial of DCCR in PWS allows enrollment of hyperphagic PWS patients as young as four years of age, permitting inclusion of those children with an earlier age of hyperphagia onset," said Dr. Anish Bhatnagar, Chief Executive Officer of Soleno. "We continue to work with our investigators, as well as the PWS community, to complete enrollment and advance this trial through the clinic as expeditiously as possible."

The DESTINY PWS Phase III trial is a randomized, double-blind, placebo-controlled study of once daily oral administration of DCCR versus placebo in approximately 100 patients with a confirmed diagnosis of PWS. The primary endpoint is change from baseline hyperphagia score at Week 13. The trial is being conducted at approximately 15 sites in several states in the U.S. Patients who complete the double-blind study are eligible to enroll in an open-label, 9-month safety extension study. The double-blind Phase III trial, which was initiated in May 2018, is expected to be completed in the first half of 2019.

In July 2018, the FDA designated the investigation of DCCR for the treatment of PWS to be a Fast Track development program. Prior to this, diazoxide choline received orphan designation for the treatment of PWS in the U.S. and in the E.U.

For further information about the trial (NCT03440814), please visit: www.clinicaltrials.gov.

About PWS
The Prader-Willi Syndrome Association USA estimates that one in 12,000 to 15,000 people in the US have PWS. The hallmark symptom of this disorder is hyperphagia, a chronic feeling of insatiable hunger that severely diminishes the quality of life for PWS patients and their families. Additional characteristics of PWS include 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 can lead to significant morbidities (e.g., stomach rupture, obesity, diabetes, cardiovascular disease) and mortality (e.g., choking, accidental death due to food seeking behavior). In a global survey conducted by the Foundation for Prader-Willi Research, 96.5% of respondents (parent and caregivers) rated hyperphagia 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. Diazoxide choline has received Orphan Drug Designation for the treatment of PWS in the U.S. and E.U.

About Diazoxide Choline Controlled-Release Tablet
Diazoxide choline controlled-release tablet is a novel, proprietary extended-release, crystalline salt formulation of diazoxide, which is administered once-daily. The parent molecule, diazoxide, has been used for decades in thousands of patients in a few rare diseases in neonates, infants, children and adults, but has not been approved for use in PWS. Soleno conceived of and established extensive patent protection on the therapeutic use of diazoxide and DCCR in patients with PWS. The DCCR development program is supported by positive data from five completed Phase I clinical studies in various metabolic indications or in healthy volunteers and three completed Phase II clinical studies, one of which was in PWS patients. In the PWS Phase II study, DCCR showed promise in addressing hyperphagia, the hallmark symptom of PWS, as well as several other symptoms such as aggressive/destructive behaviors, fat mass and abnormal lipid profiles.

About Soleno Therapeutics, Inc.
Soleno is focused on the development and commercialization of novel therapeutics for the treatment of rare diseases. The company's lead candidate, DCCR, a once-daily oral tablet for the treatment of PWS, is currently being evaluated in a Phase III clinical development program.

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 complete the Phase III clinical development program of DCCR in PWS in 2019.

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 press release will prove to be accurate. Additional factors that could materially affect actual results can be found in Soleno's annual and quarterly reports filed with the Securities and Exchange Commission, including under the caption titled "Risk Factors." Soleno expressly disclaims any intent or obligation to update these forward-looking statements, except as required by law.

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