Soleno Therapeutics Provides Update on Ongoing DESTINY PWS Phase III Trial of DCCR in Prader-Willi Syndrome

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REDWOOD CITY, Calif., July 25, 2019 (GLOBE NEWSWIRE) -- Soleno Therapeutics, Inc. (NASDAQ: SLNO, the Company or Soleno), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, today provided an update on the Company's ongoing Phase III trial, DESTINY PWS, evaluating once-daily Diazoxide Choline Controlled-Release (DCCR) tablets for patients with Prader-Willi Syndrome (PWS).

As of July 24, 2019, approximately 50% of the targeted number of patients have been enrolled into the DESTINY PWS clinical study and more than 90% of those patients have either successfully completed or continue to be treated on study. Over 90% of subjects who have completed the study have elected to continue in C602, the 9-month open-label safety extension study. More than 95% of the patients who have been enrolled into C602 continue to be treated in the study. Enrollment of patients is spread across approximately 20 sites in the U.S. and Europe. No serious, unexpected adverse events related to DCCR have been reported in these studies.

Based on the interest of the clinical investigators and families, Soleno will continue to make DCCR available to patients enrolled in the current program. The duration of C602 has been increased from 9 to 12 months, and Soleno is in the process of initiating another open-label study for patients who complete the C602 study.

"We are encouraged by the continued interest of families and investigators in keeping patients on DCCR," said Dr. Anish Bhatnagar, Chief Executive Officer of Soleno. "Our enrollment has been impacted by the number of PWS trials currently ongoing in the US and Europe, but with current strong recruitment trends, we expect to conclude enrollment around the end of the year. We currently anticipate the availability of top-line data from DESTINY PWS in the first half of 2020, versus our prior expectation of late this year."

Collaboration with Casimir
Soleno has entered into a collaboration with Casimir Inc., a rare disease research organization that designs outcome measures that capture the real-world impact of treatment interventions on patient quality-of-life. Casimir will collaborate with Soleno in the development of DCCR for patients with PWS. Casimir's previous work includes the design of real-life outcome measures for Duchenne Muscular Dystrophy (DMD), the origins of which were studied in patients being treated with EXONDYS 51® for (DMD).

"The 21st Century Cures Act defines the importance of individual patient experience to the FDA's regulatory decision making process. We are excited to be working with a group as innovative as Casimir to collect these meaningful, individualized outcomes," said Dr. Bhatnagar. "Their work in designing outcome measures for other rare disease therapies, notably EXONDYS 51, the first FDA-approved treatment for DMD, has proven to be groundbreaking. We look forward to utilizing the individualized patient data they collect to support the endpoint data that are already being collected in DESTINY PWS."

"We are delighted to work with Soleno on the DCCR program for PWS," said Mindy Leffler, President of Casimir. "Based on our extensive conversations with caregivers, physicians and family members of PWS patients, we believe we can bring significant value to the program by identifying aspects of DCCR's efficacy that may otherwise not be captured. PWS patients have significant unmet needs and we look forward to collaborating with Soleno to better understand the potential efficacy of DCCR."

DESTINY PWS 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. Patients who complete DESTINY PWS have the option to enroll into C602.

In July 2018, the U.S. Food and Drug Administration (FDA) designated the investigation of DCCR for the treatment of PWS to be a Fast Track development program. Diazoxide choline has also received orphan designation for the treatment of PWS in the U.S. and in the E.U.

For further information about DESTINY PWS (NCT03440814), please visit: www.clinicaltrials.gov.

About PWS
The Prader-Willi Syndrome Association USA estimates that approximately one in 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 (DCCR) 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 data from five completed Phase I clinical studies 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 DESTINY PWS study during 2020. 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.

Corporate Contact:
Brian Ritchie
LifeSci Advisors, LLC
212-915-2578

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