Running for Research – Prader-Willi Syndrome to Fund Multi-Center Study of DCCR in Early Phase Prader-Willi Syndrome

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REDWOOD CITY, Calif., March 09, 2022 (GLOBE NEWSWIRE) -- Soleno Therapeutics, Inc. (“Soleno”) (NASDAQ: SLNO), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, today announced that Running for Research - Prader-Willi Syndrome (“RFR”), an organization committed to raising private donations in support of advancing the science around Prader-Willi syndrome (“PWS”), will fund an investigator-sponsored clinical study to evaluate Diazoxide Choline Extended-Release tablets (“DCCR”) in patients with early phase PWS.

The study is expected to be a multi-center trial conducted in approximately 40 subjects at four institutions in the U.S. The goal of this study is to evaluate the effects of DCCR in pre-hyperphagic individuals with PWS based on various behavioral and metabolic parameters and to assess if DCCR has the potential to prevent progression to hyperphagia and late-phase PWS.

“Patients with PWS progress through several nutritional phases before they reach Nutritional Phase 3, characterized by hyperphagia, as well as other behavioral issues and significant metabolic abnormalities,” said Jennifer L. Miller, M.D., Professor in the Division of Pediatric Endocrinology at the University of Florida and Principal Investigator of the study. “We believe that biomarker data generated to date in DCCR-treated patients in Soleno’s ongoing Phase 3 program show promising evidence of the potential of DCCR to impact the progression of the disease. This study will evaluate the impact of DCCR on patients with PWS before they develop hyperphagia and evaluate its potential to slow or prevent progression of PWS. We are excited to further evaluate this promising product candidate in the clinic through this study.”

“Running for Research – Prader-Willi Syndrome was founded for the purpose of funding important scientific research,” said Kelly Shad Guillou, co-founder of Running for Research – Prader-Willi Syndrome. “We have vastly exceeded our funding goal for this clinical trial, more than quadrupling our original target. This is by far the largest amount we have raised to date on behalf of any specific research project. We are thrilled with our success and believe it is indicative, in large part, of the high level of enthusiasm from the PWS community for DCCR and this study.”

“We believe the data generated to date from the ongoing clinical program suggest the potential of DCCR as a treatment option for early phase PWS patients. Soleno is pleased to provide DCCR for this study to evaluate DCCR in pre-hyperphagic patients to test this hypothesis. We are grateful for the support from Dr. Miller, RFR and the entire PWS community as we continue the development of DCCR for PWS,” said Anish Bhatnagar, M.D., Chief Executive Officer of Soleno Therapeutics.

A Phase 3 program is currently ongoing to study DCCR in individuals with PWS who have hyperphagia. Soleno has been in ongoing discussions with the U.S. Food and Drug Administration regarding additional data needed to support the submission of a New Drug Application, and has recently submitted a study proposal involving subjects currently enrolled in the ongoing open-label C602 study.

About PWS
The Prader-Willi Syndrome Association USA estimates that PWS occurs in one in every 15,000 live births in the U.S. 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., obesity, diabetes, cardiovascular disease) and mortality (e.g., stomach rupture, 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 and 92.9% body composition 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 EU, and Fast Track Designation in the U.S.

About DCCR (Diazoxide Choline Extended-Release) Tablets
DCCR is a novel, proprietary extended-release dosage form containing the crystalline salt of diazoxide and 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 1 clinical studies in healthy volunteers and three completed Phase 2 clinical studies, one of which was in PWS patients. In the PWS Phase 3 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 other metabolic parameters. Soleno has been in ongoing discussions with the FDA regarding additional data needed to support the submission of an NDA.

About Running for Research – Prader Willi Syndrome
Running for Research – Prader-Willi Syndrome is an organization committed to raising private donations in support of advancing the science around PWS that can lead to improved treatments and quality of life for patients. Specifically, RFR’s focus is the PWS research that will be conducted at the University of Florida under the direction of Dr. Jennifer Miller. RFR is a 501(c)(3) nonprofit organization contained within the University of Florida Foundation and is 100% volunteer driven.

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 Prader-Willi Syndrome (PWS), is currently being evaluated in an ongoing Phase 3 clinical development program. For more information, please visit www.soleno.life.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended. All statements other than statements of historical facts contained in this press release are forward-looking statements, including statements regarding timing of any regulatory process or ultimate approvals and determining a path forward for DCCR for the treatment of PWS. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplates," "believes," "estimates," "predicts," "potential" or "continue" or the negative of these terms or other similar expressions. These forward-looking statements speak only as of the date of this press release and are subject to a number of risks, uncertainties and assumptions, including the risks and uncertainties associated with market conditions, as well as risks and uncertainties inherent in Soleno's business, including those described in the company's prior press releases and in the periodic reports it files with the SEC. The events and circumstances reflected in the company's forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, the company does not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

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