Soleno Therapeutics Announces Presentation of Body Composition Results from DESTINY PWS, a Phase III Trial of DCCR in Prader-Willi Syndrome

November 3, 2020

REDWOOD CITY, Calif., Nov. 03, 2020 (GLOBE NEWSWIRE) -- Soleno Therapeutics, Inc. (“Soleno”) (NASDAQ: SLNO), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, today announced presentation of body composition data from the Company’s Phase III trial, DESTINY PWS (C601), evaluating once-daily Diazoxide Choline Controlled Release (DCCR) tablets for patients with Prader-Willi Syndrome (PWS). The results were presented today by Parisa Salehi, M.D., Clinical Director of the Prader-Willi Syndrome Clinic at Seattle Children’s Hospital, in a late-breaking oral presentation at The Obesity Society’s ObesityWeek ® 2020 meeting. Dr. Salehi presented the data on behalf of the DESTINY PWS Investigators.

DESTINY PWS is a randomized, double-blind, placebo-controlled Phase III study of once daily oral administration of DCCR in 127 PWS patients conducted at 29 sites in the U.S. and U.K. The objective of the study was to assess the efficacy and safety of DCCR in subjects with genetically-confirmed PWS aged four years and older and weighing between 20 and 134 kg. Patients who completed the double-blind study enrolled in study C602, an ongoing open-label, extension study. Updated top-line results were previously announced in September 2020 that demonstrated DCCR’s beneficial impact on hyperphagia, the predominant symptom of PWS, other behaviors and body composition abnormalities typical of PWS.

Key results for effects on body composition and adipokines following DCCR treatment were presented at ObesityWeek 2020 as follows:

Fat mass and body mass:

- Significant reductions in fat mass (p=0.0027) were observed after 13 weeks of double-blind treatment
  - Greater mean reductions in fat mass were observed in those participants weighing more than 100 kg at baseline (placebo-adjusted difference -4.82 kg)
- Linear exposure-response relationship for fat mass was significant with greater fat loss occurring at higher circulating drug concentrations (p<0.0001)
- Trend towards increased lean body mass for DCCR compared to placebo (p=0.058)
- Significant increase in lean body mass/fat mass ratio (p=0.001)

Leptin:

- Significant reduction in leptin while it increased in placebo-treated subjects (p<0.0001)
- Linear exposure-response relationship for leptin was significant with greater reductions in leptin occurring at higher circulating drug concentrations (p<0.0001)
- Reductions in leptin were greater than would be predicted solely by the loss of body fat, suggesting an improvement in leptin sensitivity

Adiponectin:

- Adiponectin, a cardioprotective hormone, increased in DCCR-treated subjects, but decreased in placebo-treated participants (p<0.0001)

“We are pleased to present further results from the DESTINY PWS study that demonstrate DCCR’s effect on body composition. In the DCCR group compared to placebo, we have observed a significant reduction in fat mass and leptin. Improvements in leptin sensitivity, as suggested by the results, may have an important impact on regulating hyperphagia,” said Dr. Salehi. “The data presented to date show that DCCR has the potential to manage both behavioral and co-morbid metabolic components of PWS.”

“These data represent additional means by which DCCR may address the significant unmet medical needs and the life-threatening comorbidities associated with PWS,” said Anish Bhatnagar, M.D., Chief Executive Officer of Soleno Therapeutics. “We remain focused on advancing DCCR as a potential treatment for PWS and look forward to our regulatory interactions to establish the path forward for making DCCR available as a treatment for PWS patients.”

About PWS
The Prader-Willi Syndrome Association USA estimates that one in 15,000 live births in the U.S. 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 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 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 III 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.

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, Diazoxide Choline Controlled-Release (DCCR) tablets, a once-daily oral tablet for the treatment of Prader-Willi Syndrome (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 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.

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

Source: Soleno Therapeutics