



Solenio Therapeutics Announces Positive Statistically Significant Top-line Results from Randomized Withdrawal Period of Study C602 of DCCR for Prader-Willi Syndrome

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Study Met Primary Endpoint; Highly Statistically Significant Difference in Change from Baseline in HQ-CT Total Score for DCCR Compared to Placebo (p=0.0022)

Solenio Intends to Submit a New Drug Application for DCCR in PWS Mid-Year 2024

Company to Host Conference Call and Webcast Today at 9:00 AM ET

REDWOOD CITY, Calif., Sept. 26, 2023 (GLOBE NEWSWIRE) -- September 26, 2023 – Solenio Therapeutics, Inc. (Solenio) (NASDAQ: SLNO), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, today announced positive top-line results from the randomized withdrawal period of Study C602, a long-term treatment study of DCCR (Diazoxide Choline) Extended-Release tablets for the treatment of Prader-Willi syndrome (PWS).

“We are delighted with the highly statistically significant results from the randomized withdrawal phase of Study C602,” said Anish Bhatnagar, M.D., Chief Executive Officer of Solenio. “These results will support our planned submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) mid-year of next year. We would like to thank the patients, families, investigators, study site personnel and the advocacy community involved in this study, as well as the entire Solenio team for their support of the DCCR development program. We remain committed to the goal of delivering DCCR, if approved, as an effective and safe therapy to individuals with PWS as expeditiously as possible.”

The FDA previously acknowledged that data from this study have the potential to support an NDA submission for DCCR, which has Orphan Drug designation for the treatment of PWS in the U.S. and E.U. and Fast Track designation from the FDA.

C602 Randomized Withdrawal Period Design:

The multi-center, randomized, double-blind, placebo-controlled randomized withdrawal period enrolled 77 patients previously enrolled in Study C602 who had been on open-label treatment with DCCR for between two and four years. Participants were randomized 1:1 to receive either DCCR (n=38) or placebo (n=39) for a period of four months. The primary endpoint was the change from baseline in hyperphagia-related behaviors as assessed by the hyperphagia questionnaire for clinical trials (HQ-CT), a caregiver-completed nine item validated questionnaire for assessing hyperphagia in PWS. Secondary endpoints included investigator assessments of participants’ overall severity of illness and change in condition, as measured by Clinical Global Impression of Severity (CGI-S) and Clinical Global Impression of Improvement (CGI-I) ratings, respectively.

Key Top-line Results:

- Hyperphagia-related behaviors markedly worsened in the placebo group compared to DCCR, represented by a highly statistically significant, clinically meaningful difference in mean change from baseline in the HQ-CT total score of 5.0 at week 16 (p=0.0022).
- Secondary endpoints of CGI-S and CGI-I both showed strong trends towards worsening in the placebo group compared to DCCR over the course of the randomized withdrawal period (p=0.08 and 0.09), respectively.
- DCCR continued to be generally well-tolerated in the randomized withdrawal period with no new or unexpected safety signals, including no serious adverse events or discontinuations due to adverse events occurring in any participants in the DCCR group.

Solenio entered into a Securities Purchase Agreement with Nantahala Capital Management, LLC, Abingworth LLP and Vivo Capital, LLC in December 2022, which may result in gross proceeds to Solenio of up to \$60 million (the Securities Purchase Agreement). To date, pursuant to the Securities Purchase Agreement, Solenio has received \$10 million in exchange for Tranche A and Tranche B warrants to purchase common stock. Under the terms of the Securities Purchase Agreement, the investors are required to exercise Tranche A warrants to purchase 8,598,870 shares of common stock at \$1.75 for a total of approximately \$15 million within 30 days of the announcement of positive top-line data from the randomized withdrawal period of Study C602.

Conference Call and Webcast Details

Solenio will host a conference call and webcast to discuss these results today, September 26, 2023 at 9:00 AM ET. Details can be found below:

Title:	Randomized Withdrawal Period of Study C602 Top-line Results
Date:	Tuesday, September 26, 2023
Time:	9:00 AM ET
Conference Call Details:	Toll-free: 1-877-423-9813 International: 1-201-689-8573 Conference ID: 13741535
Call me™ Feature (avoid waiting for operator):	Click Here
Webcast:	Webcast Link – Click here

A replay of the call will be available following the call on the Investors section of the Solenio website.

About PWS

The Prader-Willi Syndrome Association USA estimates that PWS occurs in one in every 15,000 live births. The hallmark symptom of this disorder is hyperphagia, a chronic and life-threatening feeling of intense, persistent hunger, food pre-occupation, extreme drive to food seek and consume food that severely diminish the quality of life for patients with PWS 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% rated body composition as either 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 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, diazoxide choline 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 patients with PWS. In the PWS Phase 3 clinical development program, 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. Diazoxide choline has received Orphan Drug Designation for the treatment of PWS in the U.S. and E.U., and Fast Track Designation in the U.S.

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 extended-release tablets, a once-daily oral tablet for the treatment of PWS, recently completed its Phase 3 development program to support a planned NDA submission. 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 the potential receipt of gross proceeds from the warrant financing under the Securities Purchase Agreement, management's assessment of the top-line data results from the randomized withdrawal period, and the timing and pathway of the regulatory process and clinical development path for seeking approval of 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," "goal," "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 U.S. Securities and Exchange Commission. 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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Source: Soleno Therapeutics