Soleno Therapeutics Announces Peer-Reviewed Publication of Data Comparing DCCR Treatment to the Natural History of Prader-Willi Syndrome

April 30, 2024 12:00 PM EDT

Statistically Significant Improvements with DCCR Compared to Natural History of PWS from the PATH for PWS Study in Hyperphagia and PWS-related Behaviors

REDWOOD CITY, Calif., April 30, 2024 (GLOBE NEWSWIRE) -- Soleno Therapeutics, Inc. (Soleno) (NASDAQ: SLNO), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of rare diseases, today announced the publication of the comparison of results from the Company's Phase 3 placebo-controlled study (C601) and open-label extension study (C602) evaluating investigational, once-daily DCCR (Diazoxide Choline) Extended-Release tablets in patients with Prader-Willi syndrome (PWS), to data from the PATH for PWS (PATH) natural history study, in the *Journal of Neurodevelopmental Disorders*. The article, entitled, "Behavioral Changes in Patients with Prader-Willi Syndrome Receiving Diazoxide Choline Extended-Release Tablets Compared to the PATH for PWS Natural History Study," can be found HERE.

Data from DCCR-treated participants in the C602/C602 cohort were compared to results from a cohort of comparable participants from PATH using the same caregiver-completed questionnaires to measure hyperphagia (Hyperphagia Questionnaire for Clinical Trials [HQ-CT]) and PWS-related behaviors (the Prader-Willi Syndrome Profile [PWSP]) in six domains: aggressive behaviors, anxiety, compulsivity, depression, disordered thinking, and rigidity-irritability.

Hyperphagia: Participants treated with DCCR showed highly statistically significant and clinically meaningful improvement with DCCR administration relative to participants in the PATH study at 6 and 12 months ($p<0.001$ for both comparisons).

PWS-related behaviors: As with hyperphagia, highly statistically significant and clinically meaningful improvements were seen in DCCR treated participants compared to those in the PATH study in all behavioral domains of the PWSP at 6 and 12 months ($p\leq0.003$ for all domains).

"The PATH for PWS study is an important evaluation of the natural history of individuals with PWS," said Dr. Theresa Strong, Director of Research Programs for FPWR. "These highly significant improvements with long-term DCCR treatment compared to the PATH data suggest that DCCR has the potential to provide much needed and substantial improvement in the lives of those living with PWS and their families. We are excited to see DCCR advance through the regulatory process and look forward to continuing to support Soleno in its efforts."

"DCCR has the potential to substantially improve the quality of life for individuals with PWS and their families," said PATH Study Principal Investigators, Jennifer Miller, M.D. and Shawn McCandless, M.D. "These data clearly demonstrate that long-term treatment with DCCR resulted in changes in hyperphagia and other behavioral complications of PWS that are meaningfully improved compared to the natural history of the disease. We believe these results support the significant potential of DCCR in PWS and are eager to offer a much-needed treatment option to patients in need, if approved."

About the DCCR C601/602 Dataset

C602 an open-label extension study enrolled participants who completed DESTINY PWS (C601), an international, multi-center, randomized, double-blind, placebo-controlled study of DCCR in 127 PWS patients at 29 sites in the U.S. and UK.

About PATH for PWS

The PATH from PWS (PATH) study (https://www.clinicaltrials.gov/study/NCT03718416) is a recently completed study that was concurrently recruited with DESTINY PWS and sponsored by the Foundation for Prader-Willi Research (FPWR). The key objective of PATH is to advance the understanding of the natural history in individuals with PWS, particularly long-term behavioral changes in the syndrome.

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 for 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 and Breakthrough Designations 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 (diazoxide choline) 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 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 the projected timeline of our NDA submission, whether FDA will agree with our interpretation of the data or the adequacy of data to support an NDA, the FDA's review of our NDA, 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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Source: Soleno Therapeutics