November 17, 2020

# Cerecor to Collaborate with Frontiers CDG Consortium on Pivotal Trial of CERC-801 for the Treatment of PGM1-CDG

ROCKVILLE, Md., Nov. 17, 2020 (GLOBE NEWSWIRE) -- Cerecor Inc. (NASDAQ: CERC), a biopharmaceutical company focused on becoming a leader in the development and commercialization of treatments for rare and orphan diseases, today announced a collaboration with The Frontiers in Congenital Disorders of Glycosylation Consortium (FCDGC) led by Eva Morava-Kozicz, M.D. Ph.D., Principal Investigator of the trial, Professor of Medical Genetics, Senior Associate Consultant, Department of Clinical Genomics, Mayo Clinic and Editor in Chief of the *Journal of Inherited Metabolic Disease* on a prospective pivotal trial evaluating the safety and efficacy of CERC-801 in patients suffering from Phosphoglucomutase-1 deficiency related congenital disorders of glycosylation (PGM1-CDG).

Dr. H. Jeffrey Wilkins, M.D., Chief Medical Officer for Cerecor, stated, *"We are delighted to be working with the premier group of thought leaders from The FCDGC to study CERC-801 in patients suffering from PGM1-CDG. We intend to use the data generated from this prospective trial to support our submission package to the FDA for CERC-801 as the first approved product for the treatment and management of PGM1-CDG."*

This trial will study the safety, tolerability, and efficacy of CERC-801 in patients with PGM1-CDG using daily therapeutic doses of CERC-801 in approximately ten patients. Outcome measures will include evaluation of clinical symptoms and clinically meaningful biomarkers.

Andrea Miller, JD, MHA, President & Founder of CDG CARE, stated, *"We are truly excited to see the collaboration between FCDGC and Cerecor and this prospective trial in PGM1-CDG. CDG is an area of high unmet need where there are no approved therapies today. The possibility for there to be an approved therapy for PGM1-CDG is exciting and has the potential to improve the quality of life of patients suffering from this ultra rare form of CDG."*

**About PGM1-CDG**

CDGs are a group of rare, inherited, metabolic disorders caused by glycosylation defects that present as a broad range of clinical symptoms, including coagulopathy, hepatopathy, myopathy, hypoglycemia, protein-losing enteropathy and reduced cell counts. CDG patients are born with a genetic defect that hinders their ability to utilize certain monosaccharides in the production of glycoproteins. A deletion or misplacement of a sugar subunit produces a dysfunctional glycoprotein, resulting in a myriad of medical issues.

While there are no U.S. Food and Drug Administration-approved treatments for the

treatment of CDGs, dietary monosaccharide formulations have been shown to alleviate several of the clinical manifestations in CDG patients. These restorative monosaccharide therapies work by increasing the availability of metabolic intermediates for glycoprotein synthesis. PGM1-CDG is caused by mutation in the PGM1 gene encoding an enzyme responsible for the interconversion of glucose-6-phosphate to glucose-1-phosphate. Glucose-1-phosphate can be utilized to supply UDP-galactose, a substrate that donates galactose subunits for glycoprotein synthesis. CERC-801 uses therapeutic doses of D-galactose to restore glycosylation in patients with PGM1 deficiency.

**About FCDGC**

The Frontiers in CDG Consortium leverages cross-disciplinary, team-based clinical science to address decades of unresolved questions, increase clinical trial readiness, advance and share knowledge, develop treatments, and address current unmet patient needs. The Consortium establishes a nation-wide network of ten regional academic centers, the Sanford Burnham Presbyterian Medical Discovery Institute and the patient advocacy group CDG CARE.

CDG CARE (Community Alliance and Resource Exchange) is the Patient Advocacy Group representing all Congenital Disorders of Glycosylation (CDG) and Deglycosylation (CDDG) for the FCDGC. CDG CARE is a nonprofit 501(c)(3) organization founded by parents seeking information and support for a group of disorders known as CDG. Their mission is to promote greater awareness and understanding of CDG, to provide information and support to families affected by CDG, and to advocate for and fund scientific research to advance the diagnosis and treatment of all CDGs.

**About CERC-800s**

CERC-801, CERC-802 and CERC-803 are restorative monosaccharide therapies with known therapeutic utility for the treatment of select CDGs. Oral administration at therapeutic doses of CERC-801, CERC-802, and CERC-803 replenishes critical metabolic intermediates that are reduced or absent due to genetic mutation, overcoming single enzyme defects in respective CDGs to support glycoprotein synthesis, maintenance and function.

**About Cerecor**

Cerecor is a biopharmaceutical company focused on becoming a leader in the development and commercialization of treatments for rare and orphan diseases. The company is advancing its clinical-stage pipeline of innovative therapies that address unmet patient needs within rare and orphan diseases. The company's rare disease pipeline includes CERC-801, CERC-802 and CERC-803 ("CERC-800 compounds"), which are therapies for inherited metabolic disorders known as congenital disorders of glycosylation. The U.S. Food and Drug Administration ("FDA") granted Rare Pediatric Disease Designation (RPDD) and Orphan Drug Designation (ODD) to all three CERC-800 compounds, thus potentially qualifying the Company to receive a Priority Review Voucher (PRV) upon approval of each new drug application (NDA). The company is also developing CERC-002, CERC-006 and CERC-007. CERC-002 is an anti-LIGHT monoclonal antibody being developed for the treatment of severe pediatric-onset Crohn's disease, and is also being studied for COVID-19 acute respiratory distress syndrome. CERC-006 is a dual mTOR inhibitor being developed for the treatment of complex lymphatic malformations and has been granted ODD and

RPDD by the FDA, thus potentially qualifying the company to receive a fourth PRV upon approval of an NDA. CERC-007 is an anti-IL-18 monoclonal antibody being developed for the treatment of autoimmune inflammatory diseases such as Still's disease and multiple myeloma.

For more information about Cerecor, please visit[www.cerecor.com](http://www.cerecor.com).

**Forward-Looking Statements**
This press release may include forward-looking statements made pursuant to the Private Securities Litigation Reform Act of 1995. Forward-looking statements are statements that are not historical facts. Such forward-looking statements are subject to significant risks and uncertainties that are subject to change based on various factors (many of which are beyond Cerecor's control), which could cause actual results to differ from the forward-looking statements. Such statements may include, without limitation, statements with respect to Cerecor's plans, objectives, projections, expectations and intentions and other statements identified by words such as "projects," "may," "might," "will," "could," "would," "should," "continue," "seeks," "aims," "predicts," "believes," "expects," "anticipates," "estimates," "intends," "plans," "potential," or similar expressions (including their use in the negative), or by discussions of future matters such as: the development of product candidates or products; timing and success of trial results and regulatory review; potential attributes and benefits of product candidates; and other statements that are not historical. These statements are based upon the current beliefs and expectations of Cerecor's management but are subject to significant risks and uncertainties, including: drug development costs, timing and other risks, including reliance on investigators and enrollment of patients in clinical trials, which might be slowed by the COVID-19 pandemic; regulatory risks; Cerecor's cash position and the potential need for it to raise additional capital; general economic and market risks and uncertainties, including those caused by the COVID-19 pandemic; and those other risks detailed in Cerecor's filings with the Securities and Exchange Commission. Actual results may differ from those set forth in the forward-looking statements. Except as required by applicable law, Cerecor expressly disclaims any obligations or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in Cerecor's expectations with respect thereto or any change in events, conditions or circumstances on which any statement is based.

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Source: Cerecor Inc.