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Cerecor Announces First Patient Enrolled in CDG FIRST Trial

-A Retrospective Study on Congenital Disorders of Glycosylation

ROCKVILLE, Md., July 15, 2019 (GLOBE NEWSWIRE) -- Cerecor Inc. (NASDAQ: CERC), a biopharmaceutical company focused on becoming a leader in development and commercialization of treatments for rare and orphan diseases in pediatrics and neurology, today announced it has enrolled its first patient into the CDG FIRST (**C**ongenital **D**isorders of **G**lycosylation **F**ormati**v**e **R**etrospective **St**udy) trial.

The CDG FIRST trial is a multi-center, international, non-interventional, retrospective study that follows general principles of periodic assessment of CDG patients in routine practice. The objectives of the study are to collect natural history and treatment-related data of patients diagnosed with PGM1-CDG, MPI-CDG or SLC35C1-CDG who are either treated with or without D-galactose, D-mannose and L-fucose, respectively, as well as patients with other CDGs who are treated with one of the sugars.

"*We are excited to have our first patient enrolled into this retrospective study to investigate the natural course of disease and current treatment approaches for CDGs. The data acquired through this study will be used in our regulatory filings and could help to expedite the first approved treatment(s) for CDGs",* stated Dr. Perry Calias, Ph.D., Cerecor CSO.

Cerecor has three compounds under development for CDGs: CERC-801, D-galactose, to treat Phosphoglucomutase 1 (PGM1) Deficiency; CERC-802, D-mannose, to treat Mannose-Phosphate Isomerase (MPI) Deficiency; and CERC-803, L-fucose, to treat Leukocyte Adhesion Deficiency Type II (LADII) or SLC35C1-CDG. Each indication is an ultra-rare CDG estimated to have less than 1,000 patients in the world. All three programs have been granted Rare Pediatric Disease Designation (RPDD) and Orphan Drug Designation (ODD) by the FDA and CERC-801 has received Fast-Track Designation (FTD) by the FDA. Cerecor has previously held pre-IND meetings with the FDA and plans to leverage data from the CDG FIRST Trial, existing clinical and nonclinical data from published literature and sponsor-initiated studies to accelerate development and approval of all three compounds under the 505(b)(2) pathway.

We anticipate filing the NDA for CERC-801 in the first half of 2021 and hope to obtain NDA approvals in late 2021 and during 2022 for all three compounds. These timelines are dependent on several factors, including the data generated from the CDG FIRST study.

There are numerous benefits associated with receipt of both ODD and RPDD, upon receiving FDA marketing authorization and commercial sale of each product including:

- 7-year marketing exclusivity (upon approval) in the United States.;
- Tax credits (up to 25% of clinical development costs);
- Waiver of PDUFA Application Fees (filing fees); and
- Rare Pediatric Disease Priority Review Voucher (upon approval) for each compound that has been granted RPDD.

Dr. Simon Pedder, Executive Chairman of the Board for Cerecor commented,*"The enrollment of our first patient into CDG FIRST is an exciting milestone reinforcing our commitment to developing treatments for Congenital Disorders of Glycosylation. Achieving regulatory approval of the CERC-800 programs is a top priority for Cerecor, as we seek to make these treatments available to patients as quickly as possible."*

Andrea Miller, President of CDG CARE, a nonprofit organization focused on promoting awareness and education of CDGs, commented, *"We're excited that Cerecor is focusing their efforts on developing treatments for Congenital Disorders of Glycosylation. A retrospective study is an important step towards standardizing and formalizing CDG Natural History and treatment experience that hopefully will enable an expedited path toward regulatory approval. Currently, there are no approved products for these life debilitating conditions. An FDA approved treatment would have a major impact on the quality of life for CDG patients and their caregivers."* Cerecor has also established the first global patient registry for CDGs, known as CDG connect, in collaboration with CDG CARE. For more information on CDG Connect, please visit http://cdgcare.com/.

About CDGs

CDGs are a rapidly expanding group of rare Inborn Errors of Metabolism (IEMs) due to defects in glycosylation. Glycosylation is the process by which carbohydrate complexes are created, modified and attached to proteins and lipids, creating glycoconjugates that are essential for cell structure and function in all tissues and organs. CDG is caused by a specific inherited mutation and more than 100 CDGs have been identified to date. CDGs typically present in infancy and can be associated with a broad spectrum of symptoms that include severe, disabling or life-threatening cases.

About CDG CARE

CDG CARE (Community Alliance and Resource Exchange) is a nonprofit 501(c)(3) organization founded by parents seeking information and support for a group of disorders known as Congenital Disorders of Glycosylation (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 scientific research to advance the diagnosis and treatment of CDG.

About CDG Connect

CDG CARE is proud to partner with the Invitae Patient Insights Network (PIN) and Cerecor to offer the first international CDG Patient Registry – CDG Connect. CDG Connect has been created to develop a comprehensive database of individuals with all types of Congenital Disorders of Glycosylation (CDG). It provides CDG patients and families with a secure and confidential platform to share critical information to understand the history and progression of CDG. Through participation, and submission of key clinical information a network is being

built that will make it easier for researchers to study CDG, for patients and families to learn about evolving therapies and treatment options, and for advocates to speak on behalf of the CDG community.

About CERC-800 Series for CDGs

CERC-801, CERC-802 and CERC-803 represent genetically-targeted, small molecule, substrate replacement therapies with established therapeutic utility for the treatment of CDGs. Oral administration of these substrates replenishes critical metabolic intermediates that are reduced or absent due to genetic mutation, overcoming single enzyme defects to support glycoprotein synthesis, maintenance and function.

About the CDG FIRST Study

This is a multi-center, international, non-interventional, retrospective study that follows general principles of periodic assessment of CDG patients in routine practice.

About Cerecor

Cerecor is a fully integrated biopharmaceutical company with commercial operations and research and development capabilities. The Company is building a robust pipeline of innovative therapies in orphan rare diseases, neurology and pediatric healthcare. The Company's pediatric orphan rare disease pipeline is led by CERC-801, CERC-802 and CERC-803 ("CERC-800 programs"), which are therapies for inborn errors of metabolism specifically disorders known as Congenital Disorders of Glycosylation. The FDA granted Rare Disease Designation and Orphan Drug Designation to all three CERC-800 compounds, thus qualifying them for receipt of a Priority Review Voucher ("PRV") upon approval of a new drug application ("NDA"). The PRV may be sold or transferred an unlimited number of times. The Company plans to leverage the 505(b)(2) NDA pathway for all three compounds to accelerate development and approval. The Company is also in the process of developing one other preclinical pediatric orphan rare disease compound for the treatment of mitochondrial DNA Depletion Syndrome. The Company's neurology pipeline is led by CERC-301, which Cerecor is currently exploring as a novel treatment for neurogenic orthostatic hypotension. The Company is also developing two other neurological compounds; CERC-406 for Parkinson's Disease, CERC-611 for epilepsy. The Company also has a diverse portfolio of marketed products. Our marketed products are led by our prescribed dietary supplements and prescribed drugs. Our prescribed dietary supplements include Poly-Vi-Flor® and Tri-Vi-Flor™ which are prescription vitamin and fluoride supplements used in infants and children to treat or prevent deficiency of essential vitamins and fluoride. The Company also markets a number of prescription drugs that treat a range of pediatric diseases, disorders and conditions. Cerecor's prescription drugs include AcipHex®, Cefaclor for Oral Suspension, Karbinal™ ER, Sprinkle™, Millipred® and Ulesfia®.

For more information about Cerecor, please visitwww.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," "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 (including as it may be impacted by government shut-downs); potential attributes and benefits of product candidates; the expansion of Cerecor's drug portfolio; 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; reliance on and the need to attract, integrate and retain key personnel; Cerecor's cash position and the potential need for it to raise additional capital; risks associated with acquisitions, including the need to quickly and successfully integrate acquired assets and personnel; 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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