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Cerecor Announces Submission of Three Orphan Drug Designation Requests for Substrate Replacement Therapies to treat Congenital Disorders of Glycosylation

BALTIMORE, Oct. 31, 2018 (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, announced today that it has submitted to the U.S. Food and Drug Administration (FDA) three Orphan Drug Designation (ODD) Requests for substrate replacement therapies to treat ultra-rare inherited metabolic disorders known as Congenital Disorders of Glycosylation (CDGs).

Submissions were made for 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). Each indication is an ultra-rare CDG estimated to have less than 1,000 patients in the United States. All three programs have been awarded Rare Pediatric Disease Designation (RPDD) by the FDA. Cerecor has already held pre-IND meetings with the FDA and seeks to leverage existing clinical and nonclinical data in conjunction with sponsor-initiated studies to accelerate development and approval via the 505(b)(2) pathway.

Peter Greenleaf, Cerecor CEO stated, *“These assets have an important place in the treatment of pediatric patients who are suffering from CDGs. Glycosylation is the process by which carbohydrates are utilized to modify certain proteins as it relates to protein structure and function. Glycoproteins are fundamental building blocks in the body and CDG patients are born with a genetic defect that reduces the ability to synthesize glycoproteins. These substrate replacement therapies work by increasing the availability of metabolic intermediates for glycoprotein synthesis. We are proud and excited to be a leader in CDG drug development and to continue working with patients, their families and medical professionals to achieve FDA approval for these products and subsequently world-wide.”*

There are numerous benefits associated with receipt of both ODD and RPDD, including:

- 7-year marketing exclusivity (upon approval) in the United States.;
- Tax credits (up to 50% 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.

Mr. Greenleaf further commented, *"The recent granting of the RPDD of CERC-801 as well as the submissions of all three of these assets for Orphan Drug Designation further reinforces our acquisition of Ichorion and accelerates our research and development pipeline."*

Andrea Miller, President of CDG CARE, a nonprofit organization focused on promoting awareness of CDG, commented, *"We are thankful and excited that companies such as Cerecor are focusing their efforts on developing treatments for Congenital Disorders of Glycosylation. Patients and their caregivers welcome FDA-approved products. Cerecor has worked in collaboration with CDG CARE to establish the first global patient registry for CDGs, known as CDG Connect."* For more information on CDG Connect, please visit http://cdgcare.com/.

About CDG:
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 Compounds for CDG:

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 Cerecor

Cerecor is a biopharmaceutical company focused on becoming a leader in the development of neurologic and pediatric therapies that make a difference in the lives of patients. The Company's pipeline is led by CERC-301, which Cerecor is currently exploring as a novel treatment for orphan neurological indications. Cerecor is also developing three pre-clinical stage compounds, CERC-611. CERC-406 and CERC-913. The Company's R&D efforts are supported by revenue from its franchise of commercial medications led by Poly-Vi-Flor® (multivitamin and fluoride supplement tablet, chewable) and Tri-Vi-Flor® (multivitamin and fluoride supplement suspension/drops). In February 2018, the Company added to its marketed product portfolio by acquiring Karbinal™ ER, AcipHex® Sprinkle™, Cefaclor for Oral Suspension, and Flexichamber™.

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, 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; 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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