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FDA Accepts IND Application for Cerecor's Investigational Drug CERC-801 for the treatment of PGM1 Deficiency

ROCKVILLE, Md., Jan. 22, 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, announced today that the FDA has accepted its Investigational New Drug (IND) application for CERC-801, an ultra-pure, oral formulation of D-galactose currently in development for the treatment of Phosphoglucomutase 1 (PGM1) deficiency, also known as PGM1-CDG.

Peter Greenleaf, CEO stated, *"We are in the midst of organizational transformation here at Cerecor into a fully-integrated, innovative biopharmaceutical company. In less than a year, we have established a commercial footprint, doubled the number of programs in our development pipeline and achieved several regulatory milestones with the FDA. The FDA's acceptance of our IND for CERC-801 represents yet another milestone for the organization. We are one step closer to gaining an approval for a much-needed therapy for patients and families suffering from PGM1 Deficiency."*

The clinical development program for CERC-801 will commence with a Phase 1 study in healthy volunteers. The goals of the study will be to assess the single dose tolerability and pharmacokinetics of CERC-801. Cerecor seeks to leverage existing clinical and nonclinical data in conjunction with sponsor-initiated studies, such as this Phase 1 study, to accelerate development and approval of CERC-801 via the 505(b)(2) pathway.

About CERC-801

CERC-801 is an ultra-pure formulation of D-galactose, a naturally occurring monosaccharide found in dairy products and fruit. D-Galactose is consumed by the body to provide substrates for protein glycosylation, the process by which carbohydrates are utilized to modify certain proteins as it relates to protein structure and function. CERC-801 has been granted Orphan Drug Designation (ODD) and awarded Rare Pediatric Disease Designation by the FDA, granting eligibility for receipt of a Priority Review Voucher (PRV) upon approval of an NDA.

About PGM1-CDG

CDG 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 have high infant morbidity and mortality with no FDA-approved treatments. 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.

Dietary monosaccharide formulations have been shown to alleviate several of the clinical manifestations in CDG patients. These substrate replacement 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. Substrate replacement with CERC-801 in patients with PGM1 Deficiency is hypothesized to increase available UDP-galactose pools and repair galactose-deficient glycoproteins.

Additionally, Cerecor has established the first-ever global Patient Insights Network (PIN) for CDGs, known as CDG Connect, in collaboration with CDG Care advocacy organization and Invitae. See here for more info: https://connect.invitae.com/org/cdg.

About Cerecor

Cerecor is a biopharmaceutical company focused on becoming a leader in the development of orphan 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 neurogenic orthostatic hypotension. Cerecor has six additional programs in development, including CERC-406 for Parkinson's Disease, CERC-611 for epilepsy, CERC-801, CERC-802, and CERC 803 for Congenital Disorders of Glycosylation and CERC-913 for DGUOK Deficiency a mitochondrial DNA Depletion Syndrome. The Company's R&D efforts are supported by revenue from its franchise of commercial medications led by Poly-Vi-Flor® and Tri-Vi-Flor® (multivitamin and fluoride supplement tablet, chewable and 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 (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; 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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