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FDA Accepts IND Application for Cerecor's Investigational Drug CERC-802 for the treatment of MPI-CDG

ROCKVILLE, Md., July 29, 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 it has received notification from the U.S. Food and Drug Administration (FDA), stating that the FDA has accepted the CERC-802 MPI deficiency Investigational New Drug (IND) application filing and the proposed study can begin immediately. CERC-802, an ultra-pure, oral formulation of D-mannose is currently in development for the treatment of Mannose Phosphate Isomerase (MPI) Deficiency, also known as MPI-CDG or CDG-1b.

Cerecor's CSO, Perry Calias, PhD, stated, *"We are pleased to be able to move forward with our second clinical program exploring the potential benefit of substrate replacement therapies for the community of patients suffering from a congenital disorder of glycosylation, or CDG. These studies, along with the data obtained from the CDG FIRST study, should provide valuable insights as we continue to work with the FDA to accelerate development and approval of CERC-802 via the 505(b)(2) pathway with a targeted NDA submission of 2021."*

The clinical development program for CERC-802 will commence with a Phase I study in healthy volunteers. The goals of the study will be to assess the single dose tolerability and pharmacokinetics of CERC-802. Cerecor seeks to leverage existing clinical and nonclinical data in conjunction with sponsor-initiated studies, such as this Phase I study and the recently initiated CDG FIRST Trial, to accelerate development and approval of CERC-802 via the 505(b)(2) pathway.

About CERC-802

CERC-802 is an ultra-pure formulation of D-mannose, a naturally occurring monosaccharide commonly found in animals, microorganisms, and plants, including edible fruits and herbs. D-mannose 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-802 has been granted Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation by the FDA, making the Company eligible to receive a Priority Review Voucher (PRV) upon approval of an NDA.

About MPI-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. CDGs 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. Biallelic pathogenic variants of the MPI gene lead to enzymatic deficiencies of mannose-6-phosphate isomerase (MPI enzyme) associated with the clinical syndrome MPI-CDG. The overall estimated occurrence of MPI-CDG worldwide is less than 50 cases, although MPI-CDG is suspected to be under-diagnosed.

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. CDGs are 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.

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 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 the Company to receive 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 CERC-406 for Parkinson's Disease. The Company also has a diverse portfolio of marketed products, led by our prescribed dietary supplements 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; 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; regulatory 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.

For media and investor inquiries

James Harrell
Cerecor Inc.
jharrell@cerecor.com
623.439.2220 *office*

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