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Cerecor Receives Fast Track Designation from FDA for CERC-802 for the Treatment of Mannose-Phosphate Isomerase Deficiency

ROCKVILLE, Md., Aug. 21, 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 U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for CERC-802, an ultra-pure, oral formulation of D-mannose currently in development for the treatment of Mannose-Phosphate Isomerase Deficiency, also known as MPI-CDG or CDG-1b.

"We believe that the granting of Fast Track designation for CERC-802 is another crucial step in the development of a potential treatment for this ultra-rare condition," said Dr. Simon Pedder, Executive Chairman of the Board for Cerecor. *"We continue to work closely with the FDA to advance this development program forward expeditiously. We're currently collecting retrospective data through the CDG FIRST trial to support a New Drug Application for a much-needed therapy."*

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.

Fast Track Designation

Fast Track Designation is granted to drugs being developed for the treatment of serious or life-threatening diseases or conditions where there is an unmet medical need. The purpose of the Fast Track Designation provision is to help facilitate development and expedite the review of drugs to treat serious and life-threatening conditions so that an approved product can reach the market expeditiously.

Sponsors of drugs that receive Fast Track Designation have the opportunity for more frequent interactions with the FDA review team throughout the development program. These can include meetings to discuss study design, data required to support approval, or other aspects of the clinical program. Additionally, products that have been granted Fast Track Designation may be eligible for priority review of NDA and the FDA may consider reviewing portions of an NDA before the sponsor submits the complete application (Rolling Review).

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 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 diseases, neurology and pediatric healthcare. The Company's pediatric orphan 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 Pediatric Disease Designation and Orphan Drug Designation ("ODD") 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, CERC-913, for the treatment of mitochondrial DNA Depletion Syndrome. The Company's neurology pipeline is led by CERC-301, a Glutamate NR2B selective, NMDA Receptor antagonist, which Cerecor is currently exploring as a novel treatment for orthostatic hypotension. The Company is also developing CERC-406, a CNS-targeted COMT inhibitor 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 Millipred®, Karbinal™ ER, AcipHex® Sprinkle™, and Cefaclor for Oral Suspension.

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.

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