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Cerecor Announces Clinical Updates on CERC-301 and CERC-802

- CERC-301 Enrolls First Patient in Diabetic Orthostatic Hypotension Trial
-CERC-802 Completes Phase I Safety Study in Healthy Volunteers

ROCKVILLE, Md., Nov. 11, 2019 (GLOBE NEWSWIRE) -- Cerecor Inc. (NASDAQ: CERC), a biopharmaceutical company focused on becoming a leader in development and commercialization of treatments for orphan diseases and neurology, announced today that it has achieved significant milestones in its clinical development programs. The first patient has been enrolled in a Phase I Proof-of-Concept Trial investigating the safety, tolerability and effects on blood pressure in patients with orthostatic hypotension associated with diabetes ("DOH"). In addition, in October 2019, the Company completed dosing healthy volunteers in a Phase I Safety Study of CERC-802, an ultra-pure, oral, crystalline formulation of D-mannose currently in development for the treatment of MPI Deficiency ("MPI-CDG").

Dr. Simon Pedder, Executive Chairman of the Board, commented, *"We are very enthusiastic about our continued progress from our research and development team. It's very exciting to see the advancement and expansion of the CERC-301 development program into a substantially broader patient population namely the millions of diabetic patients suffering from the untoward effects of Orthostatic Hypotension."*

"Likewise, the completion of the CERC-802 Phase I Safety Study allows us to advance our second asset for the treatment of Congenital Disorders of Glycosylation (CDGs) getting us another step closer to helping the hundreds of patients and families world-wide. We continue to stay on track against our R&D milestones which could deliver our first product approval as early as 2021 with an associated Priority Review Voucher."

About the CERC-301 Proof-of-Concept Study in DOH
The purpose of this study is to assess the single dose effects of CERC-301 in patients with symptomatic Orthostatic Hypotension ("OH") associated with diabetes. This study is a randomized, double-blind, placebo-controlled, two-way cross-over trial, over two, 24-hour, in-clinic visits. At each visit, patients will receive a single 20 mg dose of CERC-301 or placebo and then undergo a series of orthostatic challenge tests over the 24-hour, in-clinic period. Patients will also complete an OH symptomatic assessment following each orthostatic challenge. Safety, tolerability and pharmacokinetic ("PK") data will also be collected. As part of the routine laboratory tests, particular interest will be paid to the patient's plasma glucose levels over the course of the study.

Clin301-101 was a study in patients with nOH associated with Parkinson's disease. In the Clin301-101 study, a single 20 mg dose of CERC-301 achieved clinically meaningful

improvements over baseline and placebo with a maximum improvement of 29.1 mmHg upon standing throughout the 6-hour study period. All doses tested were safe and well tolerated with no serious adverse event reported.

Of note, in previous clinical studies conducted by Cerecor, 20 mg doses of CERC-301 were safe and well tolerated for up to 28 days.

About CERC-301
CERC-301 is an orally available, NR2B-specific, NMDA receptor antagonist being developed for the treatment of symptomatic OH.

About Orthostatic Hypotension ("OH")
Orthostatic hypotension is a sudden fall in blood pressure that occurs when a person assumes a standing position. It can be due to a lesion of the baroreflex loop, which senses a change in blood pressure and adjusts heart rate and activates sympathetic nerve system fibers to cause the blood vessels to narrow and correct blood pressure. It may also be caused by hypovolemia (a decreased amount of blood in the body), resulting from the excessive use of diuretics, vasodilators, or other types of drugs, dehydration, or prolonged bed rest. The disorder may be associated with Addison's Disease, diabetes, spinal cord injuries, dialysis, advanced age and certain neurological disorders including Multiple System Atrophy with Orthostatic Hypotension (formerly known as Shy-Drager syndrome), autonomic system neuropathies, and other dysautonomias. Symptoms, which generally occur after sudden standing, include dizziness, lightheadedness, blurred vision, and syncope (temporary loss of consciousness).

Current treatment options for OH target symptom burden reductions to increase quality of life such as correcting aggravating factors (i.e. discontinuation of hypotension drugs and correction of anemia and vitamin deficiencies); nonpharmacologic measures such as intravascular volume expansion, increased physical activity, reduction of meal size, compression stocking/abdominal binder, and sleeping arrangement; and drug therapies (i.e. droxidopa, midrodrine).

Orthostatic Hypotension affects numerous comorbid disease conditions with significant underserved patient populations (see chart) in the United States and in the rest of the world.

Comorbid Disease	% with OH	U.S. Based Estimated Population	Estimated # of Patients
Advanced Age	30%[1,2]	46,000,000 > 65 Years of Age[8]	13,800,000
Diabetes	16 to 25%[2,3,4]	30,000,000[9]	6,000,000
Parkinson's Disease	15 to 58%[4]	1,200,000 Patients[10]	420,000
ESRD Dialysis	15 to 50%[5]	660,000 Patients / 1,980,000 Procedures[11]	216,450
Spinal Cord Injury	35 to 60%[6,7]	288,000 Patients[12]	136,800

Professor Christopher Mathias MBBS DPhil DSc FRCP FMedSci, a world-renowned thought leader in Autonomic and Neurovascular Disorders from the Imperial College and University

College London Hospitals stated, *"The unique mechanism of action of CERC-301 and its demonstrated effect on blood pressure clearly warrants further investigation for the treatment of conditions associated with hypotension. Its potential to be, perhaps, safer and more effective in a much broader patient population than our current therapeutic options provide is promising. It's exciting to think of a compound being developed for Orthostatic Hypotension in patients suffering from Parkinson's disease and diabetes that may also have clinical utility for other conditions exacerbated by hypotension such as advanced age, intradialytic hypotension and patients with spinal cord injury."*

About the CERC-802 Phase I Study (Clin802-101)
The single-center, US-based safety, tolerability and PK study was an open-label, randomized, single-dose, 4-way crossover study in 16 healthy adult volunteers, of which 14 completed. CERC-802 had no serious adverse events. All CERC-802 related adverse events were transient and resolved with no sequalae. PK data is expected in early 2020.

"The doses were generally well tolerated *by the majority of healthy volunteers and anticipated PK data should provide a solid foundation for the ongoing development of CERC-802,"* said Dr. Perry Calias, PhD, Chief Scientific Officer at Cerecor. *"We are also very excited about the progress being made with the CDG FIRST Trial our retrospective study seeking to collect natural history, efficacy and safety data from CDG patients treated with monosaccharide substrate replacement therapy for, PGM1-CDG, MPI-CDG and Leukocyte Adhesion Deficiency Type II (LADII) also known as SLC35C1-CDG. We believe the information gathered through this study will be instrumental in facilitating regulatory approval of all three CERC-800 programs through 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 (RPDD) 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 the CDG First Trial
The CDG FIRST (Congenital Disorders of Glycosylation Formative Retrospective Study) 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.

About Cerecor
Cerecor is a biopharmaceutical company focused on becoming a leader in development and commercialization of treatments for orphan diseases and neurological conditions. The Company is building a robust pipeline of innovative therapies in orphan diseases and neurology. The Company's pediatric 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 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 one marketed product, Millipred®, an oral prednisolone indicated across a wide variety of inflammatory conditions and indications.

For more information about Cerecor, please visit www.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, including reliance on investigators and enrollment

of patients in clinical trials; 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; 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,
Chief Commercial Officer
Cerecor Inc.
jharrell@cerecor.com
623.439.2220 *office*

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Source: Cerecor Inc.