

August 4, 2020



Cerecor Receives Rare Pediatric Disease Designation for CERC-006 in Lymphatic Malformations

ROCKVILLE, Md. and CHESTERBROOK, Pa., Aug. 04, 2020 (GLOBE NEWSWIRE) -- Cerecor Inc. (NASDAQ: CERC), a leading biopharmaceutical company in the development and commercialization of treatments for rare pediatric and orphan diseases, today announced that the U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease Designation (RPDD) to CERC-006, a dual inhibitor of mTOR complexes 1 and 2 for the treatment of lymphatic malformations (LM).

Lymphatic malformations are rare, non-malignant masses consisting of fluid-filled channels or spaces thought to be caused by the abnormal development of the lymphatic system. LM occurs mostly in infancy or early childhood and can persist throughout life. It is estimated that 30,000 to 60,000 Americans have this condition in the United States.¹

"We are very pleased to receive FDA Rare Pediatric Disease Designation for CERC-006. This designation underscores the high unmet need in this family of serious and rare diseases of infants and children that so often lead to disability and in some cases, death," said Mike Cola, Chief Executive Officer, Cerecor.

The FDA grants RPDD to programs addressing rare diseases or conditions that are serious or life-threatening in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. The term "rare disease or condition" means any disease or conditions affecting less than 200,000 people in the United States. If a new drug application (NDA) for CERC-006 is approved, Cerecor would be eligible to receive a priority review voucher for another compound, which it could use itself, or sell to another company.

About CERC-006

CERC-006 is an orally available blocker of mTOR complex 1 and 2 that is being developed for the treatment of serious lymphatic malformations (LM) not treatable with surgery or sclerotherapy. Because a large majority of LM patients have activating mutations in the PK/AKT/mTOR pathway, we believe CERC-006 has the potential to specifically reduce proliferation of the abnormal cells that cause LM, reduce the size of neoplastic lesions and restore lymphatic function; ultimately improving and prolonging the lives of many affected children. A Phase 1b/2a proof-of-concept trial to test the safety and efficacy of CERC-006 in LM is planned to begin in 2021.

About Cerecor

Cerecor is a leading biopharmaceutical company focused on in development and commercialization of treatments for rare pediatric and orphan diseases. The Company is advancing an emerging clinical-stage pipeline of innovative therapies. 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 ("CDGs"). The FDA granted Rare Pediatric Disease Designation ("RPDD") and Orphan Drug Designation ("ODD") to all three CERC-800 programs, thus potentially qualifying the Company to receive a Priority Review Voucher ("PRV") upon approval of a new drug application ("NDA") of a drug that is a part of such program. The Company is also developing CERC-002, CERC-006 and CERC-007. CERC-007 is an anti-IL-18 monoclonal antibody being developed for the treatment of autoimmune inflammatory diseases such as Adult Onset Still's Disease ("AOSD") and Multiple Myeloma ("MM"). CERC-006 is a dual mTOR inhibitor being developed for the treatment of complex Lymphatic Malformations. CERC-002 is an anti-LIGHT monoclonal antibody being developed for the treatment of COVID-19 ARDS and Pediatric-onset Crohn's Disease.

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," "might," "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; 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, which might be slowed by the COVID-19 pandemic; regulatory risks; Cerecor's cash position and the need for it to raise additional capital; general economic and market risks and uncertainties, including those caused by the COVID-19 pandemic; 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.

1. Perkins et al. 2010

For media and investor inquiries for Cerecor, Inc.

James Harrell,
Investor Relations
Chief Commercial Officer
Cerecor Inc.
jharrell@cerecor.com
623.439.2220 *office*



Source: Cerecor Inc.