

## Cerecor Announces Acquisition of TARPγ8-AMPA Receptor Antagonist (CERC-611) from Lilly

Phase 1 development for epilepsy expected to commence in 2017

BALTIMORE-- Cerecor Inc. (NASDAQ: CERC), a clinical-stage biopharmaceutical company developing treatments to make a difference in the lives of patients with neurological and psychiatric disorders, today announced that it has acquired exclusive, worldwide rights from Eli Lilly and Company ("Lilly") to develop and commercialize LY3130418 (now designated as CERC-611). CERC-611 is a potent and selective Transmembrane AMPA Receptor Regulatory Proteins ("TARP")-γ8-dependent AMPA receptor antagonist. TARPs are a fairly recently discovered family of proteins that have been found to associate with and modulate the activity of AMPA receptors. TARP-γ8-dependent AMPA receptors are localized primarily in the hippocampus, a region of importance in complex partial seizures and particularly relevant to seizure origination and/or propagation. Research suggests that selectively targeting individual TARPs may enable selective modulation of specific brain circuits without globally affecting synaptic transmission which may lead to improved efficacy, safety and tolerability. Cerecor expects to submit an investigational new drug application ("IND") to the United States Food and Drug Administration ("FDA") and, upon acceptance of the IND by the FDA, commence Phase 1 development of CERC-611 in 2017.

CERC-611 was discovered and developed by Lilly for the treatment of epilepsy, a neurological disorder affecting over 50 million people worldwide. 150,000 new cases of epilepsy are diagnosed in the United States annually, and 30%-40% of treated patients are resistant to current pharmacotherapies, with only 8% of treated patients being maintained seizure free. The disorder, if not controlled, can lead to severe pathology and death. "There is a significant unmet need for new mechanisms that provide a new approach to treatment of epilepsy, with improved efficacy, safety, tolerability and ease-of-use," said Ron Marcus, M.D., Chief Medical Officer and Head, Regulatory Affairs at Cerecor.

AMPA receptor antagonists are known anticonvulsant agents, and their ability to down-modulate excitatory neurotransmission is key to their antiepileptic therapeutic potential. However, since AMPA receptor activity is so ubiquitous in the central nervous system ("CNS"), a non-selective AMPA antagonist approach affects many areas of the CNS, resulting in undesired effects, such as ataxia, sedation, falls, and/or dizziness, which are shared by all known general or broad-spectrum AMPA receptor antagonists. Typically, the doses of these medications needed to obtain anti-convulsant activity are close to, or overlap with, doses at which undesired effects are observed. "Because of the predominant hippocampal location of TARP-γ8-dependent AMPA receptors, we believe that the efficacy and side effect profile of CERC-611 may represent an improvement compared to current

antiepileptics," said Uli Hacksell, Ph.D., Cerecor's CEO, President and Chairman. "We are excited to make CERC-611 a key addition to our pipeline and we expect to file an IND and commence Phase 1 development in 2017."

Under the terms of the agreement, Cerecor will immediately assume full development and commercialization responsibilities of CERC-611. Lilly will receive an upfront licensing fee as well as milestone and tiered royalty payments.

## **About CERC-611**

CERC-611 (formerly LY3130481) is a potent and selective TARP- $\gamma$ 8-dependent AMPA receptor antagonist that we believe is the first molecule to selectively target and functionally block regionally-specific AMPA receptors after oral dosing. This selectivity was engineered into CERC-611 using structure-activity relationship information to achieve selective blockade of the AMPA receptor regulator protein, or TARP- $\gamma$ 8 (high density expression in hippocampus, a region of importance in partial epilepsies), while sparing AMPA receptors thought to be associated with TARP- $\gamma$ 2 (high density expression in cerebellum regulating the ataxia and falling associated with broad spectrum AMPA receptor antagonists). CERC-611 has been observed to have positive preclinical activity in multiple models of epilepsy, neuropathic pain, and depression.

## **About Cerecor**

Cerecor is a clinical-stage biopharmaceutical company developing innovative drug candidates to make a difference in the lives of patients with neurological and psychiatric disorders. We are committed to the development of drugs that improve lives by applying our extensive knowledge and experience in central nervous system disorders. Cerecor is currently pursuing the development of two clinical Phase 2-stage product candidates: CERC-301 and CERC-501.

CERC-301 is an oral, NR2B specific N-methyl-D-aspartate receptor antagonist that is currently in a Phase 2 clinical trial as an oral, rapidly acting adjunctive treatment for patients with severe major depressive disorder ("MDD") who are failing to achieve an adequate response to their current antidepressant treatment. We expect top-line data from this trial in November 2016. Cerecor received fast track designation by the United States Food and Drug Administration in 2013 for CERC-301 for the treatment of MDD. We believe CERC-301 has the potential to be a first-in-class medication that may significantly reduce depressive symptoms in a matter of days.

CERC-501 is a potent and selective kappa opioid receptor antagonist that is currently in a Phase 2 clinical trial for smoking cessation that is expected to provide top-line data in December 2016. In addition to Cerecor's Phase 2 trial, three externally-funded clinical trials are being conducted to evaluate the use of CERC-501 in treating depressive symptoms, stress related smoking relapse and cocaine addiction. One study is being conducted under the auspices of the National Institute of Mental Health, the second is a collaboration between Cerecor and Yale investigators with funding from the National Institutes of Health and the third is being conducted at Rockefeller University Hospital with funding from a private foundation.

Cerecor has one preclinical stage asset, CERC-406, a brain penetrant catechol-O-methyltransferase inhibitor with potential procognitive activity.

For more information about the Company and its products, please visit <a href="www.cerecor.com">www.cerecor.com</a> or contact Mariam E. Morris, Chief Financial Officer, at (443) 304-8002.

## **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 include, without limitation, statements about the potential efficacy, safety and tolerability of Cerecor's product candidates as well as their potential therapeutic benefits, the timing of the expected IND submission and commencement of clinical development for CERC-611, the timing of the availability of data from clinical trials, and other 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, potential benefits of product candidates, the expected timing of data from clinical trials, technology enhancements 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 those 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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