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U.S. FDA grants RPD to Cerecin's investigational drug Tricaprilin

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Cerecin, a biopharmaceutical company focused on discovering and developing brain therapeutics, announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation to*tricaprilin,* an investigational drug under development for the treatment of infantile spasms (also known as West's syndrome), a rare form of childhood epilepsy.

The FDA defines RPD as a serious or life-threatening disease, with the serious or life-threatening manifestations primarily affecting individuals from birth to eighteen, and affecting fewer than 200,000 people in the U.S. Under the FDA's RPD program, a sponsor who receives approval of a drug for a condition that has been granted RPD designation, may also qualify for a Priority Review Voucher (PRV). This voucher can be redeemed to receive a priority review of a subsequent marketing application for a different product or can be sold or transferred to another entity.

"*Tricaprilin* has the potential to treat a broad range of neurological conditions including a number of rare childhood diseases. The grant of this Rare Pediatric Disease Designation by the FDA is an important milestone for Cerecin," commented Dr Charles Stacey, President and CEO at Cerecin. "Infantile spasms is a devastating condition. The plight of these children and their families is made worse because currently available treatments are limited, and many children may not respond or be able to tolerate these drugs. For decades, the epilepsy community has used the ketogenic diet for managing this condition. We believe *tricaprilin,* a ketogenic compound, will build on this emerging science for children with this condition."

Tricaprilin, is an investigational oral drug version of a medium chain triglyceride, which has been designed to induce ketosis and thereby improve mitochondrial metabolism. Cerecin has recently conducted non-clinical studies to examine the effect of *tricaprilin* in a model of infantile spasms. The results from these non-clinical studies were positive, and demonstrated that *tricaprilin* elevated ketones above control levels and reduced spasm counts.

Cerecin plans to meet with the FDA prior to submitting an Investigational New Drug application to advance*tricaprilin* into clinical studies for infantile spasms in 2021.