FDA NEWS

FDA grants orphan drug status to ganaxolone for pediatric female epilepsy

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The FDA recently granted orphan drug designation to ganaxolone for the treatment of protocadherin 19 gene female pediatric epilepsy, according to a press release from the drug's manufacturer.

PCDH19 female pediatric epilepsy is a rare epileptic syndrome affecting approximately 15,000 to 30,000 females in the United States. Caused by an inherited mutation of the protocadherin 19 (PCDH19) gene, the condition is characterized by early-onset cluster seizures, cognitive and sensory impairment of varying degrees and behavioral disturbances.

The most consistent feature of PCDH19 female pediatric epilepsy is the occurrence of seizures beginning before the age of 5 years, with typically focal clusters of seizures that last for days or weeks and do not respond well to available medications. Often, the syndrome is associated with a cognitive impairment of varying nature, and behavioral or social disorders with autistic traits.

Currently, there are no approved therapies for PCDH19 female pediatric epilepsy.

Ganaxolone (Marinus Pharmaceuticals) is a synthetic analog of allopregnanolone, an endogenous neurosteroid produced in the central nervous system that modulates the brain neurotransmitter GABA. Ganaxolone was designed to have the same modulation effects as allopregnanolone without the steroidal effects.

"This designation underscores the significant unmet medical need for girls suffering from this severe epileptic syndrome, associated with clusters and other types of seizures," **Christopher M. Cashman,** president and CEO of Marinus Pharmaceuticals, said in a press release. "We believe that the novel mechanism of ganaxolone, along with the established safety profile seen in pediatric epilepsy trials,

supports the potential for ganaxolone to control seizures in these young girls who currently have no approved treatment options."

Marinus Pharmaceuticals is conducting a phase 2 clinical trial to further evaluate the safety and efficacy of ganaxolone as adjunctive therapy for uncontrolled seizures in PCDH19 female pediatric epilepsy, with initial data expected later this year.

The FDA's Office of Orphan Products Development grants orphan drug designation to novel drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States.