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A novel FDA approved drug

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Abstract:

Seizures can manifest with a wide range of symptoms, including temporary changes in muscle tone or movement, such as stiffness, twitching, or limpness, as well as alterations in behavior, sensation, or consciousness. These manifestations occur due to spontaneous and uncontrolled electrical impulses among neurons in the brain. This rare neurodevelopmental disorder, known as Cyclin-dependent kinase-like 5 deficiency disorder (CDKL5-DD), is characterized by severe epileptic encephalopathy, developmental delay, and intellectual disability. There is a lack of effective treatments for CDKL5-DD, despite significant research efforts. Ganaxolone, a synthetic analog of the neurosteroid allopregnanolone, is a potential therapeutic option with a mechanism of action that focuses on gamma-aminobutyric acid (GABA) receptors. These receptors are important for regulating neuronal excitability.

Keywords:

Seizures, Cyclin- dependent- kinase- like 5 deficiency disorder, ZTALMY (Ganaxolone).