Therapeutics & Vaccines

Treating Absence Epilepsy with Ganaxolone

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The Wisconsin Alumni Research Foundation (WARF) is seeking commercial partners interested in using the drug ganaxolone to treat pediatric patients suffering from absence epilepsy.

Overview

Absence epilepsy (formerly known as 'petit mal epilepsy') afflicts thousands of people worldwide, many of them children. It is characterized by nonconvulsive seizures, loss of consciousness, glassy- eyed staring and 'spike and wave' EEG discharges. The seizures last only a few seconds but can occur up to 200 times per day. Standard drug therapies don't work for about a third of all patients.

A prime target for anti-epilepsy medications is the $GABA_A$ receptor – a family of protein complexes that mediates both synaptic and nonsynaptic (also called tonic) inhibition. The receptor is the primary target of nearly all general anesthetics and many sedatives. Mutations or poisoning of the receptor typically leads to seizures.

The Invention

UW–Madison researchers have developed a method for treating absence epilepsy with the drug ganaxolone, a synthetic neurosteroid analog that modulates $GABA_A$ receptors. The drug has shown promise for treating other forms of epilepsy but has not been recommended for absence epilepsy until now.

The researchers have found that in low doses the drug provides an optimal amount of tonic inhibition that restores function and reduces symptoms in a mouse model. The drug may be particularly useful for treating young patients whose condition is characterized by a reduction in tonic inhibition.

Applications

- Treating absence epilepsy

Key Benefits

- Offers a new treatment option for a defiant form of epilepsy
- Ganaxolone has been safety tested in clinical trials.
- Well-tolerated in adults and children
- Minimal side effects observed

Stage of Development

The researchers have shown that there are at least two distinct forms of absence epilepsy, characterized by too much or too little tonic inhibition, respectively. In mice with reduced tonic inhibition, very low concentrations of ganaxolone decreased the occurrence of spike

and wave EEG discharges and behavioral seizures by 66 percent.

Additional Information

For More Information About the Inventors

- Mathew Jones

Related Intellectual Property

- View Divisional Patent in PDF format.

Tech Fields

- Therapeutics & Vaccines : CNS

For current licensing status, please contact Rafael Diaz at rdiaz@warf.org or 608-960-9847