

HGA-0984 for the Treatment of Neurological disorders

Overview

| Drug Name | HGA-0984 |
|---------------------|--|
| Description | HGA-0984 is a GABA aminotransferase inhibitor in preclinical development for the |
| | treatment of neurological disorders, such as infantile spasm. |
| Target | 4-aminobutyrate aminotransferase (ABAT) |
| Drug Modality | Small molecule |
| Indication | Neurological disorders |
| Product Category | New molecular entity |
| Mechanism of Action | GABAergic Neurotransmission Enhancers |
| Status | Phase I |
| Patent | Granted |
| | |

Seeking Global Cooperation

Protheragen Inc. is actively seeking partnership for HGA-0984. Potential collaboration can be strategic alliance, licensing, or marketing agreement.

We look forward to hearing from you.

Target

4-aminobutyrate aminotransferase

4-aminobutyrate aminotransferase (ABAT) is responsible for catabolism of gamma-aminobutyric acid (GABA), an important, mostly inhibitory neurotransmitter in the central nervous system, into succinic semialdehyde. The active enzyme is a homodimer of 50-kD subunits complexed to pyridoxal-5-phosphate. The protein sequence is over 95% similar to the pig protein. GABA is estimated to be present in nearly one-third of human synapses. ABAT in liver and brain is controlled by 2 codominant alleles with a frequency in a Caucasian population of 0.56

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and 0.44. The ABAT deficiency phenotype includes psychomotor retardation, hypotonia, hyperreflexia, lethargy, refractory seizures, and EEG abnormalities. Multiple alternatively spliced transcript variants encoding the same protein isoform have been found for this gene.

Indication

Neurological Disorders

A neurological disorder is any disorder of the nervous system. Structural, biochemical or electrical abnormalities in the brain, spinal cord or other nerves can result in a range of symptoms. Examples of symptoms include paralysis, muscle weakness, poor coordination, loss of sensation, seizures, confusion, pain and altered levels of consciousness.

Hundreds of millions of people worldwide are affected by neurological disorders. For example, more than 6 million people die because of stroke each year; over 80% of these deaths take place in low- and middle-income countries. More than 50 million people have epilepsy worldwide. It is estimated that there are globally 47.5 million people with dementia with 7.7 million new cases every year, in which Alzheimer's disease is the most common cause of dementia and may contribute to 60–70% of cases. The prevalence of migraine is more than 10% worldwide.

Interventions for neurological disorders include preventive measures, lifestyle changes, physiotherapy or other therapy, neurorehabilitation, pain management, medication, operations performed by neurosurgeons or a specific diet.

Mechanism of Action

GABAergic Neurotransmission Enhancers

Molecular Mechanism

GABA Aminotransferase Inhibitors

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Status

The Status of HGA-0984

The international patent applications under the PCT have been granted.

| | Discovery/Optimization | Preclinical | Clinical |
|----------|------------------------|-------------|----------|
| HGA-0984 | | | |