Novel Therapy for Treatment of Chronic Degenerative Brain Diseases

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CONTACT

Ellen Kats
ellen.kats@ucsf.edu
tel: 415-758-1598.

OTHER INFORMATION

KEYWORDS

neurological disease, brain disease, therapeutic, orphan drug indication

CATEGORIZED AS

- **Medical**
  - Disease: Central Nervous System

RELATED CASES

2004-017-0

BACKGROUND

There are many types of neurological diseases that affect infants and children. Although the frequency of individual disorders is not high, together they are a significant group of disorders with a collective frequency of 1 in 18,000 live births. Unfortunately, neurologic disease is seldom curable. Thus, strategies for the treatment of these debilitating and often fatal diseases frequently focus primarily on palliative measures. Attempts at curing neurological disease have also been proposed. These treatments have included enzyme replacement therapy, gene therapy, and allogenic bone marrow transplantation. Sadly, however, these treatments typically do not improve the condition nor alter the ultimate outcome of the disease, leaving a desperate need to develop effective therapies.

TECHNOLOGY DESCRIPTION

UCSF scientist, Dr. Synthia Mellon, was first to determine that the use of neuroactive steroids is effective for the treatment of degenerative brain diseases and central nervous system disorders. The effectiveness of these neurosteroids has been proven in the Niemann-Pick Type C mouse model, as well as other mouse models of neurological disease such as Sandhoff, Sanfilippo, and ischemia.

Research into roles of neuroactive steroids in other neurological disorders is ongoing. UCSF is interested in potential licensing and sponsored research opportunities to further this research.

APPLICATIONS

This invention would provide effective therapy for the treatment of a wide range of neurological diseases, including congenital lipid storage diseases, inflammatory diseases, and chronic degenerative brain disease.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	9,597,336	03/21/2017	2004-017

RELATED MATERIALS

- Endogenous and synthetic neurosteroids in treatment of Niemann–Pick Type C disease. Brain research reviews, 2008. 57(2), 410-420.