Novel drugs for the treatment of dystonia

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Value Proposition

Dystonia is a classification of rare and debilitating movement disorders, for which there exists few therapeutic options. Treatments include therapy with botulinum neurotoxin to induce paralysis or deep brain stimulation. Not only are these modalities invasive, they do not target the root cause of the disease and can only help reduce the symptoms. Thus, there is an urgent demand for disease-modifying treatments for dystonia; however, this has historically been a challenge, given that these mechanisms remain poorly understood.

Technology

Dr. Nicole Calakos, a neurobiologist at Duke University, and colleagues have recently implicated the eIF2-alpha pathway in dystonic cells carrying the common GAG deletion within the TOR1A gene. Dr. Calakos et al. have demonstrated that therapeutically modulating the eIF2-alpha pathway helps restore several aberrancies associated with dystonic cells and improves survival in a dystonia mouse model. Importantly, these therapies can be administered in a minimally-invasive manner, making them an appealing alternative to most current therapies for dystonia. These findings having encouraging implications for the treatment of dystonia and may help target the underlying mechanisms involved in this pathology.

Other Applications

The current invention has also elucidated multiple biomarkers that may help gauge the severity of disease, as well as tailor treatment to an individual patient.

Advantages

Current therapeutic options for dystonia treat symptoms but not the underlying cause of the disease. The current invention is thought to exert disease-modifying effects.

Publications

- US patent app 15/764,315
- US patent app 15/764,320
- Functional Genomic Analyses of Mendelian and Sporadic Disease Identify Impaired eIF2α Signaling as a Generalizable Mechanism for Dystonia (Neuron, 2016)

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Patents

Patent Number: 10,857,145

Title: COMPOSITIONS AND METHODS FOR IDENTIFYING AND TREATING DYSTONIA DISORDERS

Country: United States of America

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Title: COMPOSITIONS AND METHODS FOR IDENTIFYING AND TREATING DYSTONIA DISORDERS

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