Novel compounds for treatment for Friedreich Ataxia

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Technology description

Brief Description

New compounds identified through high throughput screening which improve mitochondrial function of Friedreich ataxia cells.

Background

Friedreich ataxia (FA) is an autosomal recessive, inherited neuro- and cardio-degenerative disorder characterized by progressive ataxia of all four limbs, skeletal deformities, and hypertrophic cardiomyopathy. FA is the most prevalent inherited ataxia, affecting about 1 in 50,000 people in the United States. Most patients are confined to a wheelchair by their late 20s with myocardial failure and/or arrhythmias being the most common cause of premature death. FA is caused by mitochondrial dysfunction secondary to decreased expression of the protein Frataxin.

Problem

Currently there are no approved drugs to treat FA and the resultant disability, prolong the life of a FA patient, or cure the disorder.

Invention

Dr. Wilson and his team at UPenn developed a novel in vitro high throughput screening (HTS) platform for screening drug candidates for treatment of FA. Using such systems the team has screened 342,000 compounds and identified lead candidate compounds that increase the expression of Frataxin protein and support the survival of primary FA fibroblasts. These compounds adhere to Lipinski rules, are highly specific to FA, and are active in the low nanomolar range. Several optimized modifications of the lead compounds have been generated.

Advantages

- Regulatory fast-track: FA is a FDA designated orphan disease with no approved treatment
- Access to the expertise and resources of Wilson’ s lab

Institution

University of Pennsylvania

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