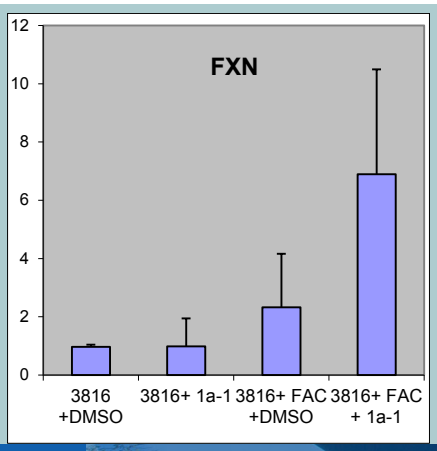

therapeutic; pharmaceutical; small molecule; rare disease, neurodegenerative disease, cardiodegenerative disease

PENN CENTER FOR INNOVATION
Empowering Ideas

Novel compounds for treatment for Friedreich Ataxia

Brief Description

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

Docket # V5006

STATE OF DEVELOPMENT

- Lead candidates identified and validated in in vitro models
- Lead optimization is underway

INTELLECTUAL PROPERTY

Issued US patent (4/17/2015) 9,000,009 and pending Divisional application

REFERENCE MEDIA

Cotticelli, G.M., et.al., 2011. J. Biomol Screen. 17(3). 303-13.

DESIRED PARTNERSHIPS

- Exclusive License
- Sponsored research
- Co-development

LEARN MORE

Linara Axanova
axanova@upenn.edu
215-573-3445 (O)

Technology Overview

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 (see Inventor's Bio)

Inventor

Robert Wilson M.D., Ph.D. is a world-renowned expert studying Friedreich ataxia for over 18 years, both in clinical and lab research settings. Dr. Wilson is co-Director of the CHOP-UPenn Center of Excellence for Friedreich's ataxia Research which involves 13 centers in 5 countries. The Center sees more FA patients than any other center in the world and is actively developing biomarkers, clinical protocols, and ataxia scales for use as Phase III endpoints. Dr. Wilson was the Scientific Director of Friedreich's Ataxia Research Alliance for 7 years and currently serves on the Medical Research Advisory Board for the National Ataxia Foundation. The Wilson lab has developed, and/or utilizes, most of the models of the disorder, has the reagents and expertise to measure all aspects of FA biochemistry, and is actively collaborating with FA researchers throughout the world.

http://www.med.upenn.edu/apps/faculty/index.php/g5455356/p17112