Methods and Compounds for Treating Mitochondrial Diseases

Tech ID: 25722 / UC Case 2016-198-0

ABSTRACT

Researchers at the University of California, Davis have developed a treatment for mitochondrial disease through a repurposing approach whereby a library of FDA-approved drugs was screened for previously unknown therapeutic effectiveness in these diseases.

FULL DESCRIPTION

Mitochondrial diseases are a clinically heterogeneous group of disorders of mitochondrial metabolism that can arise from a genetic mutation in nuclear or mitochondrial DNA. There are several inherited mitochondrial diseases, including Leber's hereditary optic neuropathy (LHON), MERRF, MELAS, NARP, and Leigh's syndrome, which affect about 50,000 people in the US. Additionally, Parkinson's disease, which affects about 1 million people in the US, is thought to result from mitochondrial defects. In many of these diseases there is visual function loss, which is particularly severe in patients with LHON. Inherited forms of mitochondrial diseases have high rates of mortality and morbidity. Currently, there are no FDA-approved therapies for mitochondrial disease, and there remains a need for pharmaceutically effective drugs to treat these conditions.

Researchers at the University of California, Davis have developed a treatment for these mitochondrial diseases through a repurposing approach to screen existing libraries of FDA-approved drugs for previously unrecognized therapeutic effectiveness. Using a mouse model, identified drugs have been shown to protect mice from mitochondrial blindness, and have the potential of slowing down or arresting the progression of mitochondrial disease. These molecules represent drugs that potentially go beyond treating symptoms to be the first curative therapy for mitochondrial disease.

APPLICATIONS

- Treatment for mitochondrial mitochondrial blindness (e.g., LHON)
- Treatment of orphan mitochondrial disease (e.g., MELAS, MERRF, NARP, Leigh’s Syndrome, and others)
- Treatment for Parkinson’s disease

FEATURES/BENEFITS

- Potential curative therapy as opposed to treating disease symptoms

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	10,792,287	10/06/2020	2016-198

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Agents Useful for Treating Obesity, Diabetes and Related Disorders

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OTHER INFORMATION

KEYWORDS

mitochondrial disease, mitochondrial blindness, Leber's hereditary optic neuropathy (LHON), Parkinson’s disease

CATEGORIZED AS

- **Medical**
  - Disease: Genetic Diseases and Dysmorphic Syndromes
  - Therapeutics

RELATED CASES

2016-198-0

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