

# Breakthrough leads to a potential drug for a rare disease for a treatment for primary mitochondrial myopathy

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A drug candidate discovered by Peter W. Schiller, Director of the Chemical Biology and Peptide Research unit at the Institut de recherches cliniques de Montréal (IRCM) and Research Professor in the Department of Pharmacology of the Université de Montréal, has been granted Fast Track designation by the Food and Drug Administration (FDA) for the treatment of primary mitochondrial myopathy. The candidate, elamipretide (formerly known as Bendavia), is being developed by Stealth BioTherapeutics Inc.

Primary mitochondrial myopathy is a type of [rare genetic disorder](#) that impairs patient health and well-being. The muscle weakness and extraordinary fatigue experienced by people suffering from this disease make simple daily tasks very challenging. There is currently no FDA-approved treatment for these patients. Peter Schiller, in collaboration with Professor Hazel Szeto, of Cornell University, discovered a family of molecules, among which is elamipretide. "Elamipretide targets the inner mitochondrial membrane and helps preserve mitochondrial energetics," says Peter Schiller. "Due to this property, elamipretide could become the first treatment designed for primary mitochondrial myopathy. The FDA's grant of a Fast Track designation for this [treatment](#) is therefore excellent news."

The FDA's Fast Track program facilitates the development and review of drugs to treat serious conditions with unmet medical needs. In

addition, the Fast Track program increases the likelihood of eligibility for priority review and accelerated approval if relevant criteria are met. Stealth BioTherapeutics is presently studying elamipretide in rare diseases like mitochondrial myopathy and as a potential therapeutic for cardiovascular and ocular diseases.

**More information:** Information on the MMPOWER clinical trial [clinicaltrials.gov/ct2/show/NC ... ?term=mmpower&rank=1](https://clinicaltrials.gov/ct2/show/NC...?term=mmpower&rank=1)

Provided by University of Montreal

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