



## **Huntington disease directly linked to perturbation of mitochondria function**

**The Challenge:** Huntington's Disease (HD) is a neurodegenerative disorder that is characterized clinically by progressive memory loss and neuropathologically by extensive neuronal cell death and accumulation of misfolded proteins in the brain. Genetically, this disease is caused by abnormal expansion of a CPG trinucleotide repeats found in the first exon of huntingtin gene that is translated into a huge 350kD protein. This form of protein has expanded polyglutamine rich regions. Pathogenesis of HD induced by polyglutamine expanded protein is likely to be mediated through an oxidative stress-induced mitochondrial fragmentation, fission reaction. Thus, it would be of great benefit to develop new methods that can inhibit mitochondrial fragmentation and subsequently decrease/reverse toxicity induced by polyglutamine repeats.

**UMBI Solution:** UMBI scientists have shown that over expression of Mfn2 and Drp-1 genes can reduce oxidative stress-induced mitochondrial fragmentation and cell death. Therefore, methods that modulate mitochondrial fragmentation might have utility in finding new ways to treat HD. Mitochondrial fragmentation can also be used for developing a diagnostic kit to measure the stage of HD disease and to check the potency of a given treatment.

### **Commercial Applications:**

- **Drug Development:** targets for development of therapeutics to treat neurodegenerative disorders and other diseases characterized by expanded polyglutamine proteins.

**Stage of Development:** This technology has been tested in *vitro* and in nematode model systems.

**Patent Status:** Pending provisional patent application.

**Licensing Potential:** UMBI is seeking exclusive or non-exclusive licensees to all or part of this technology portfolio. The UMBI Inventor would welcome the opportunity to collaborate with any licensee to further refine the invention or extend its capabilities.

**Lead Inventor & UMBI References:** Monteiro, 08-009

### **Contact Information:**

Jonathan Gottlieb, PhD, MBA  
Director, Technology Transfer and Commercialization  
Office of Research, Innovation & Commercialization

University of Maryland Biotechnology Institute  
9600 Gudelsky Drive, Suite 2105L  
Rockville MD 20850

Phone: (240) 314-6506  
Mobile: (443) 468-9875  
Email: [gottlieb@umbi.umd.edu](mailto:gottlieb@umbi.umd.edu)  
<http://www.umbi.org>