

Primary Mitochondrial Myopathy Community Webinar, offered in partnership by Stealth BioTherapeutics with MitoAction, UMDF, Foundation for Mitochondrial Medicine and MitoCanada on October 24, 2018

The following is a summary of a live webinar presented by Reenie McCarthy, CEO and Jim Carr, PharmD, Chief Clinical Development Officer of Stealth BioTherapeutics (Stealth BT). All investigational product candidates mentioned in this summary are investigational new drugs. They are not approved by the FDA for the treatment of any disease, condition or ailment. As always, please consult with your doctor when considering treatment options or clinical trials available to you or your loved one.

About Stealth BioTherapeutics & elamipretide

Stealth BioTherapeutics is dedicated to meeting the unmet medical needs of patients suffering from diseases associated with mitochondrial dysfunction. Stealth BT's lead investigational product candidate, elamipretide, is currently being studied in several mitochondrial disorders including primary mitochondrial myopathy (PMM), Barth syndrome, Leber's hereditary optic neuropathy and dry age-related macular degeneration.

Stealth BioTherapeutics is patient-focused, relying on partnerships with patient advocacy organizations and patients to inform our development of therapies for mitochondrial diseases. Our relationships with advocacy organizations, including MitoAction, UMDF, the Foundation for Mitochondrial Medicine and MitoCanada, help inform our understanding of the patient journey, disease burden, the accessibility and usability of our investigational product candidates and devices, and the clinical outcomes deemed most important by patients. We incorporate these learnings into our development programs, and use them to educate doctors, payors, and regulators about the diseases we are studying.

MMPOWER-3: the current PMM clinical trial

MMPOWER-3 is a Phase 3 double-blind, placebo-controlled trial enrolling 202 patients with PMM at sites in North America and Europe. During the first six months of the trial, patients are randomized 1:1 to receive once daily subcutaneous injections of either elamipretide or placebo, meaning there is a ~50% chance of receiving elamipretide in this first period. Following the first six-month period, all patients are eligible to participate in an open-label extension during which all participants will receive elamipretide. The open-label extension continues for a period of up to three years. Stealth will continue to collect data on the safety and effectiveness of elamipretide in patients with PMM during this trial and its open-label extension.

All trial sites in the US and Canada are currently recruiting patients ages 16-80 with genetically-confirmed primary mitochondrial disease who have myopathic symptoms of muscle weakness, exercise intolerance and fatigue. Patients who are interested and feel that they may qualify should directly contact the study site closest to them.

There are 16 MMPOWER-3 trial sites in the United States and Canada, and there are additional trial sites in Europe. For a full listing of study centers including contact information and inclusion and exclusion criteria, please visit <https://mitotrials.com/> or <https://clinicaltrials.gov/ct2/show/NCT03323749?term=NCT03323749&rank=1>

MMPOWER-3: where?



United States

Northeast

- Children's Hospital of Philadelphia, Philadelphia, PA
- Columbia Presbyterian, NY, NY
- Massachusetts General Hospital, Boston, MA
- Children's Hospital of Pittsburgh, Pittsburgh, PA

Midwest

- Akron Children's Hospital, Akron, OH
- Cleveland Clinic, Cleveland, OH

Southeast

- Rare Disease Research Center, Atlanta, GA

Southwest

- Baylor University, Houston, TX
- University of Texas, Houston, TX

United States (cont'd)

West

- Children's Hospital of Colorado, Aurora, CO
- Seattle Children's Hospital, Seattle, WA
- Stanford University, Stanford, CA
- University of California San Diego, La Jolla, CA
- University of Utah, Salt Lake City, Utah

Canada

- Adult Metabolic Disease Clinic at Vancouver General Hospital, Vancouver, British Columbia
- McMaster University Medical Center, Hamilton, Ontario

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Community Questions

How can I be selected to participate in the MMPOWER-3 trial?

If you would like to be considered, please reach out directly to the clinical trial sites mentioned above. As always, seek guidance from your physician about whether or not the clinical trial is right for you. All of the 16 US and Canada trial sites are accepting additional patients and are open for direct enrollment in the trial. Patients are not restricted by geography, although it may be more convenient to go to a site closer to home. Compensation for travel costs is provided.

After the completion of the trial, can the patient remain on the investigational drug? After completion of the 6 month double-blind placebo-controlled portion of the MMPOWER-3 study, patients are eligible to continue in an open-label extension in which all patients will receive elamipretide. The open-label extension will continue for 3 years or until elamipretide is approved for PMM or until the trial is discontinued, whichever comes first.

When will the phase 3 trial start and how long does the trial last?

Phase 3 has been enrolling since fall 2017. The duration of the placebo-controlled portion of the trial is 6 months. Patients can then elect to participate in an open-label extension during which they will receive elamipretide for up to 3 years.

What's the mechanism of action of elamipretide?

Elamipretide enters the inner mitochondrial membrane where it targets cardiolipin, an essential component of the inner mitochondrial membrane. By associating with cardiolipin, elamipretide appears to stabilize the structure and function of the inner mitochondrial membrane during oxidative stress.

Do patients need to be hospitalized to participate in MMP3?

No. Participation in the MMPOWER-3 trial does not require hospitalization.

How often are site visits needed and how much do patients have to pay for travel?

During MMPOWER-3, elamipretide is self-administered by patients or their caregivers once daily at home.

Site visits are more frequent during the first 6 months of the trial, including an initial screening visit, an initial baseline site visit, a one-month follow-up visit, a 3-month follow-up visit, a 6-month visit and then a 7-month safety follow-up visit. Following the 7-month visit, patients are eligible to participate in an open-label extension during which all patients receive elamipretide for up to three years. During the open-label extension, patients are typically seen at the trial site every 3-6 months.

Travel reimbursement is available.

Am I required to stop taking supplements or other medications prior to enrolling?

Patients are not required to stop taking supplements, including the mito cocktail, or other medications prior to or during the trial. However, patients are not eligible to participate in other trials of investigational new drugs during the trial.

Can my primary care doctor or another specialist register me for MMPOWER-3?

Patients must go directly to one of the clinical trial sites in the US, Canada or Europe for registration. Patients are encouraged to discuss their questions with their physician, but patients can also contact the trial sites directly.

What is a randomized trial?

Randomized means that when a clinical trial participant meets the inclusion and exclusion criteria, the investigator uses an automated system that randomly assigns the participant to one of the prespecified study arms.

What is the timeline for new drug applications and drug availability?

Once the MMPOWER-3 trial is fully enrolled and after the last enrolled patient has their last clinical trial visit, the data can begin to be analyzed. After evaluation of the data, assuming it is positive, Stealth will prepare a new drug application (NDA) to submit to FDA. Stealth cannot make any predictions as to the timing of the FDA decision.

To find out more, please visit one of the following websites:

www.clinicaltrials.gov

www.mitotrials.com

The following advocacy resources may also be helpful:

www.mitoaction.org

www.umdf.org

www.mitocanada.org

www.hopeflies.org

