The following is a summary of a live presentation offered through joint collaboration with UMDF, MitoAction and the Foundation for Mitochondrial Medicine to the mitochondrial disease patient and family community on August 11th, 2017. Stealth BioTherapeutics' CEO Reenie McCarthy and Chief Clinical Development Officer Jim Carr, Pharm.D., presented an update on Stealth's clinical trials in mitochondrial disorders and answered questions from the community.

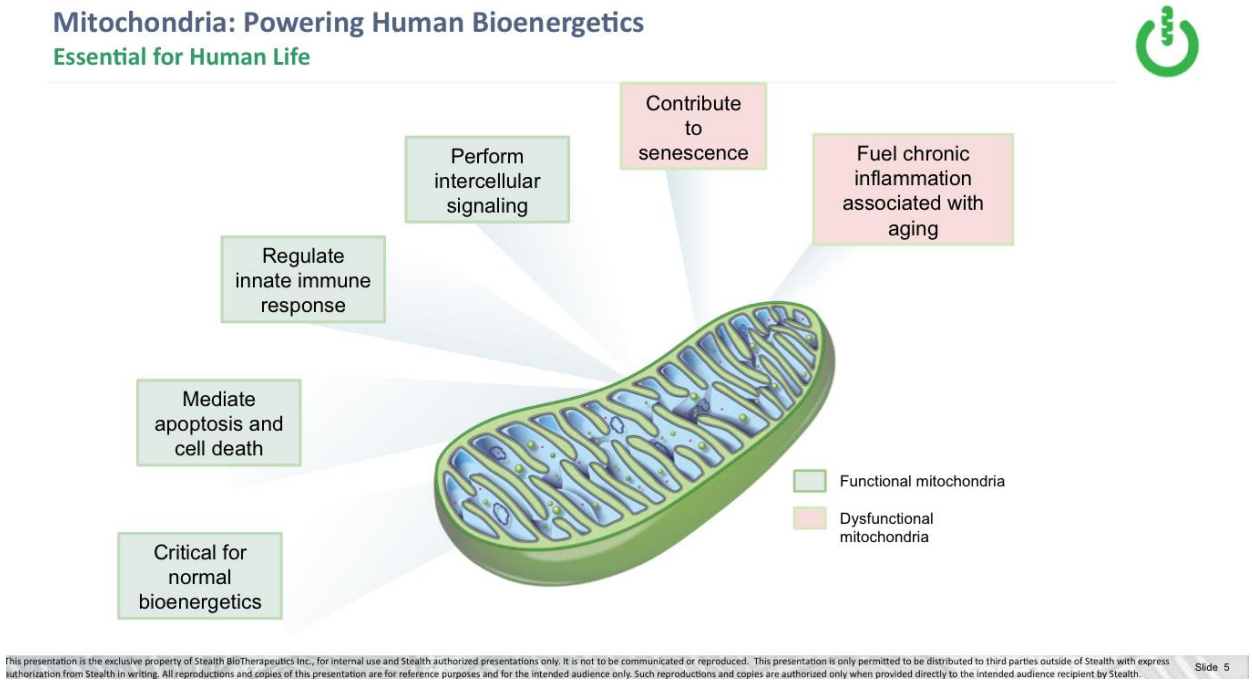
Stealth BioTherapeutics' Mission:

To establish mitochondrial medicine as a new and innovative approach to the treatment of human disease, and improve quality of life for patients with diseases involving mitochondrial dysfunction.

Key Points from this presentation:

- Elamipretide is a first-in-class investigational drug with a novel mechanism of action that appears to normalize mitochondrial function in multiple disease models.
- Elamipretide is being studied in primary mitochondrial diseases as well as in common aging-related diseases involving mitochondrial dysfunction.
- Stealth BioTherapeutics' lead program with elamipretide is in primary mitochondrial myopathy, where it already conducted two Phase 2 clinical trials (MMPOWER and MMPOWER-2) and has an ongoing observational study (RePOWER) to identify participants for its Phase 3 clinical trial, which will start enrolling patients in the US within the next few months.
    - MMPOWER-2 was a safety and efficacy study evaluating subcutaneously administered elamipretide in people with genetically-confirmed mitochondrial myopathy.
    - RePOWER is a worldwide, observational study currently recruiting patients ages 16-80 with primary mitochondrial myopathy. Patients must be enrolled in RePOWER in order to be considered for the Phase 3 interventional trial.
- Stealth BioTherapeutics is committed to developing mitochondrial therapeutics and to supporting and engaging the mitochondrial disease patient and family community.

Mitochondria: Powering human bioenergetics, essential for human life

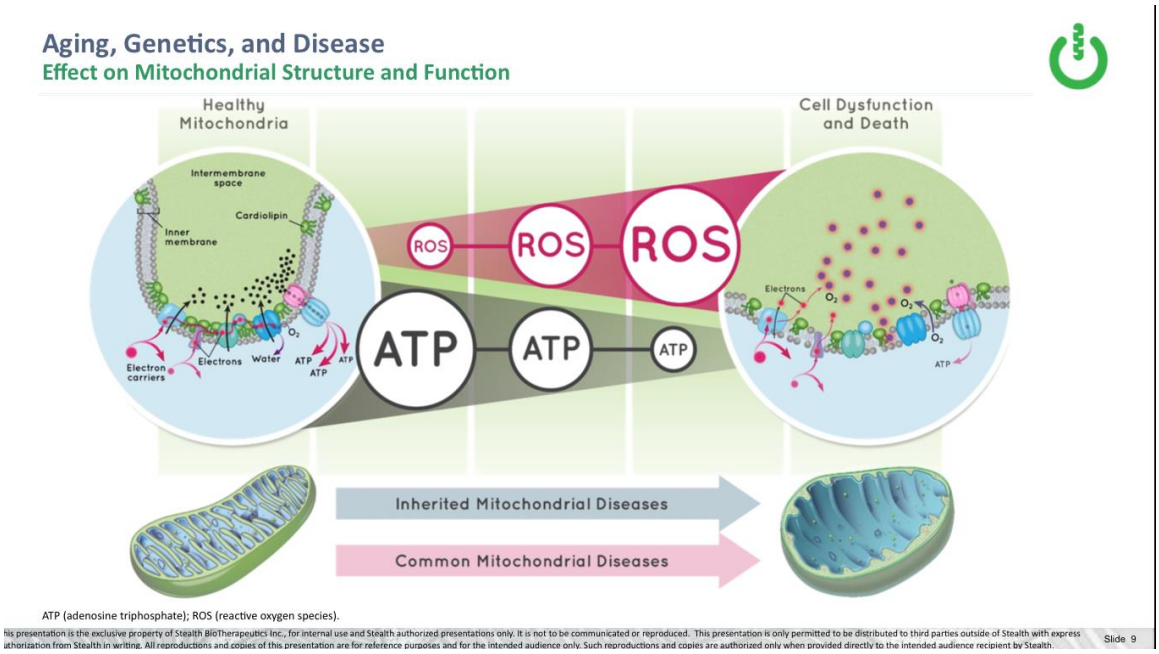


Mitochondria are found in nearly every cell in the body and produce about 90% of the energy (ATP) essential for human life. ATP is required for critical functions such as the contraction of skeletal muscle, cardiac and vascular muscle, maintenance of cell membrane potential, cellular transport and secretion of hormones and neurotransmitters. However, when mitochondria become dysfunctional due to genetic mutations, aging, disease or acquired toxicity, they can contribute to the pathogenesis of multiple diseases.

Reactive Oxygen Species, Mitochondrial Damage and Oxidative Stress

ATP is produced by the electron transport chain (ETC) located within the curves, or cristae, of the inner mitochondrial membrane. In healthy mitochondria, the ETC produces a low level of reactive oxygen species (ROS) as a normal by-product of mitochondrial ATP production. In dysfunctional mitochondria, ROS generation increases to unhealthy levels, which can damage cardiolipin, a phospholipid found only in the inner mitochondrial membrane. When cardiolipin is damaged by excess ROS, the normal structure and function of the ETC is disrupted, leading to increasing ROS production and oxidative stress.

Oxidative stress can trigger cellular and extra-cellular cascades involving inflammation, fibrosis and cell death. These compromise organ function, particularly in organ systems such as the skeletal muscle, the heart, the kidney, the eye and the brain, which are high consumers of mitochondrial ATP.

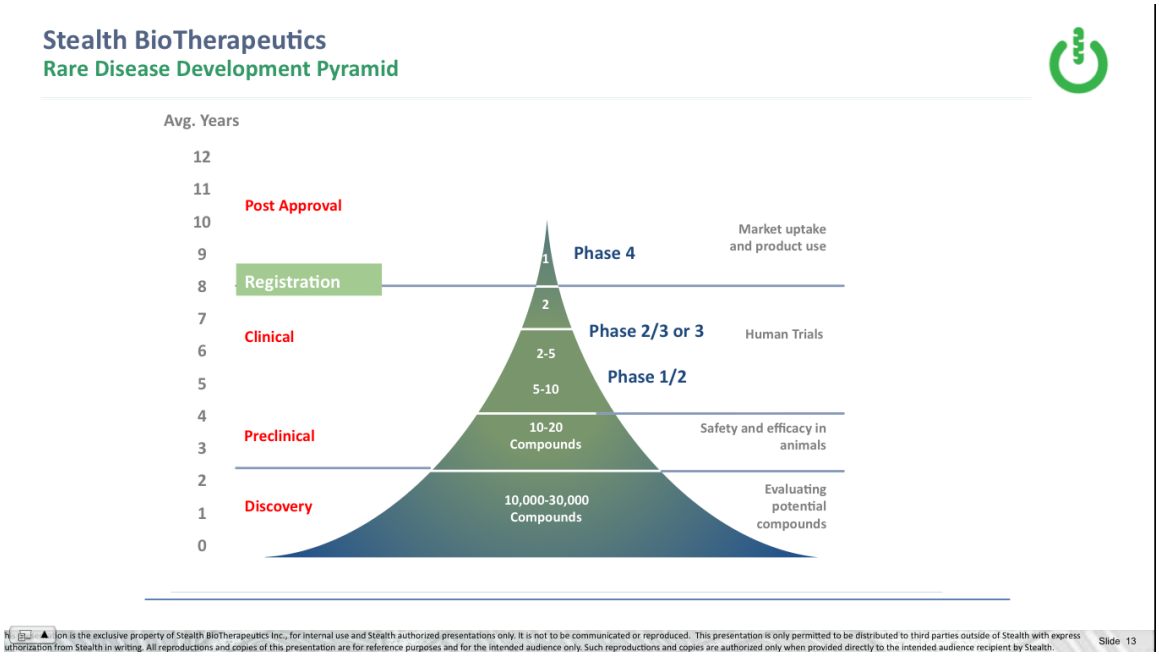


Elamipretide and the Inner Mitochondrial Membrane

Stealth's investigational product candidates target and bind reversibly to cardiolipin. They have been shown to stabilize the structure and function of the inner mitochondrial membrane and the ETC in the presence of elevated ROS. In various animal models, treatment with these compounds has resulted in normalization of mitochondrial function, including increased ATP production, decreased ROS generation, restored cardiolipin content, and decreased inflammation, fibrosis and cell death. Treatment with these compounds has also been found to improve organ function in animal models of aging skeletal muscle, heart failure, acute kidney injury, neurodegenerative diseases, and diseases of the eye.

The Journey of Rare Disease Drug Development

The process of developing therapeutics for rare diseases involves multiple stages, from discovering potentially therapeutic compounds, to testing them for safety and efficacy in cells, tissues and animals, to testing them for safety in healthy volunteers, and then to testing them for safety and efficacy in additional stages of human clinical trials. This process typically takes a decade or longer.



Stealth's lead programs for rare diseases are nearing the final stage of clinical development. Stealth is working to launch its Phase 3 trial in primary mitochondrial myopathy by the end of this year, and is currently enrolling patients in its Phase 2/3 clinical trial in Barth syndrome. Stealth's Phase 2 trial for Leber's hereditary optic neuropathy is fully enrolled, with data expected in 2018.

Trial	Indication	Phase
Elamipretide for Primary Mitochondrial Diseases		
MMPOWER OLE	Mitochondrial Myopathy	Open label extension
REPOWER	Mitochondrial Myopathy	Registry
TAZPOWER	Barth Syndrome	Phase 2/3
ReSIGHT	Leber's Hereditary Optic Neuropathy	Phase 2
SBT-20 for Neurodegenerative Disease		
CHALLENGE-HD	Huntington's	Phase 1/2

Primary Mitochondrial Disease and Primary Mitochondrial Myopathy

Over 250 genetic mutations are associated with primary mitochondrial disease. A patient with primary mitochondrial myopathy (PMM) has genetically-confirmed mitochondrial disease and the primary clinical manifestations of that disease are

myopathic symptoms including muscle weakness, easy fatigability, exercise intolerance and pain.

Primary mitochondrial disease can be highly variable with respect to age of onset, types of symptoms, and severity of symptoms, even as between those within the same family and/or within the same genetic diagnosis. This variability makes it challenging to develop clinical trials, which rely on identifying assessments, or endpoints, that can measure benefit across all trial participants. By studying PMM, which primarily affects skeletal muscle symptoms, Stealth has designed its trials with endpoints measuring changes in skeletal muscle function and fatigue.

Clinical Studies for Patients with PMM:
MMPOWER, MMPOWER-2, RePOWER and MMPOWER-3

MMPOWER, Stealth's first clinical trial enrolling patients with PMM, was a randomized, double-blind placebo-controlled trial testing the safety, tolerability and efficacy of three different doses of elamipretide administered once daily intravenously over five days to 36 patients (age 16-65) with PMM. Treatment with elamipretide appeared to be well tolerated, and no serious adverse events were observed. Patients receiving the highest dose of elamipretide demonstrated a 44-meter placebo-adjusted improvement in the six-minute-walk test (6MWT), an assessment measuring how far they could walk in six minutes. This reached nominal significance and supports further study of elamipretide in this patient population.

MMPOWER-2, Stealth's second clinical trial enrolling patients with PMM, was a randomized, double-blind, placebo-controlled trial to evaluate the safety, tolerability and efficacy of elamipretide administered once daily subcutaneously to 30 patients (age 16-65) with PMM. This was a 12-week crossover trial, meaning that patients were randomized to receive injections of either elamipretide or placebo for an initial 4-week period, after which they received no treatment during a 4-week "wash-out" period, before crossing over to receive the opposite injection during the last 4-week period.

An overall assessment of the top-line MMPOWER-2 results showed evidence of efficacy across multiple endpoints and supports a Phase 3 study in this patient population. Specifically:

- 6MWT: patients receiving elamipretide walked an average 20 meters further on the 6MWT than those receiving placebo
- PMMSA Total Fatigue: patients receiving elamipretide showed a statistically significant improvement in fatigue on the Primary Mitochondrial Myopathy Symptom Assessment (PMMSA), a questionnaire developed by Stealth in accordance with FDA guidance to assess fatigue, muscle weakness, and other symptoms in the PMM population

- NeuroQoL: patients receiving elamipretide showed a statistically significant improvement in fatigue measured by the NeuroQoL short form fatigue scale, an NIH-developed questionnaire to assess fatigue
- PMMSA Most Bothersome Symptom: patients receiving elamipretide showed a statistically significant improvement in their individual "most bothersome symptom" assessed by the PMMSA

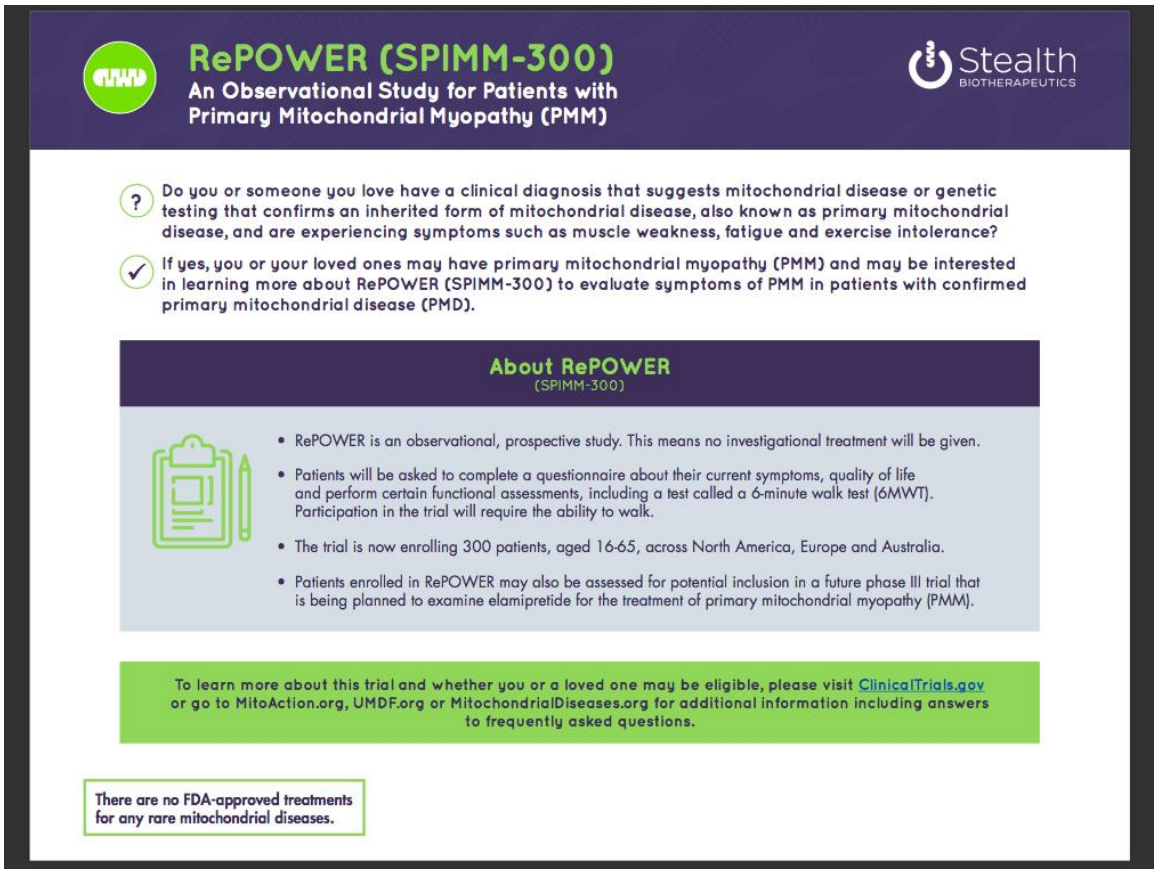
Treatment with elamipretide appeared to be well tolerated, with no serious adverse events. The most common side effect was injection-site reactions; most were mild redness or itching.

RePOWER (SPIMM-300) is a prospective, observational (meaning no treatment will be given) study enrolling approximately 300 patients across North America, Europe and Australia. The primary purpose is to assess the relationship between a patient's diagnosis and experience living with PMM, as well as local and regional differences in care and diagnosis. Patients are asked to complete questionnaires about their current symptoms and quality of life, and to perform certain functional assessments to measure strength and endurance.

Patients enrolled in RePOWER study must have confirmed or suspected PMD as well as signs and symptoms of myopathy, such as easy fatigability, exercise intolerance and muscle weakness. Patients must be between the ages of 16-65, be able to walk and attempt the 6MWT, and without prior exposure to elamipretide.

RePOWER is active and currently recruiting (as of October, 2017) at 21 sites in the US, with additional sites in Australia, Canada, and Europe (details can be found on clinicaltrials.gov or StealthBT.com).

*In order to be eligible for **MMPOWER-3,** Stealth's Phase 3 interventional trial in which patients will be randomized to receive elamipretide or placebo, patients must have been enrolled in RePOWER.*

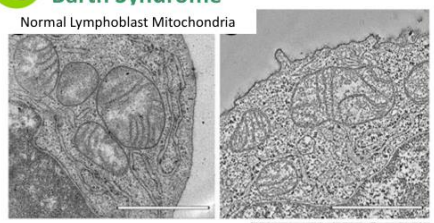


Barth Syndrome, LHON and Huntington's

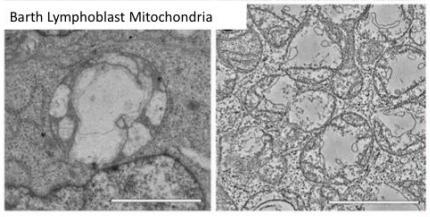
Elamipretide is also being studied in ***TAZPOWER***, a Phase 2/3 clinical trial in Barth syndrome, which is currently enrolling patients at Johns Hopkins, and in ***ReSIGHT,*** a Phase 2 clinical trial in patients with LHON, which is fully enrolled at Doheny Eye Institute. A second clinical-stage compound, SBT-20, is currently being studied in a Phase 1/2 clinical trial in patients with Huntington's disease.

Genetic Mitochondrial Disease
Barth Syndrome

Normal Lymphoblast Mitochondria



Barth Lymphoblast Mitochondria



Due to genetic mutation causing abnormal cardiolipin composition. Elamipretide binds to Barth "MLCL" cardiolipin in similar ratios as to normal cardiolipin.

Saric, A., et al. (2015), Front Genet. Acehan, Xu, Stokes, Schlame, Lab Invest, 2007

TAZPOWER Clinical Plan – Phase 2/3

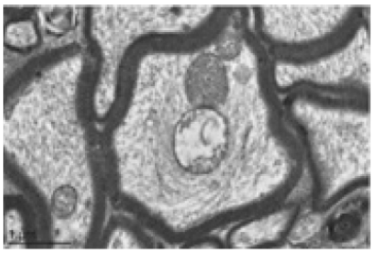
≥12 years with confirmed Barth Syndrome

- Randomized, double-blind placebo-controlled crossover design
- Two 12-week treatment arms with 1 month interim wash-out
- Once daily SC administration
- Primary efficacy endpoint:
  - Change in 6-Minute walk test
- Secondary efficacy endpoints:
  - 5X sit-to-stand
  - Handheld dynamometry
  - Accelerometry
  - ECHO Biomarkers of functional cardiac improvement
  - Quality of life
- Safety Assessments

Barth syndrome

- Caused by mutations in tafazzin gene, which codes for an enzyme involved in the synthesis of cardiolipin.
- Affects between an estimated one in 200,000 and one in 400,000 births
  - Johns Hopkins' Kennedy Krieger Institute hosts North America's only multidisciplinary Barth syndrome clinic
- Cardiomyopathies, skeletal muscle weakness, and neutropenia

Genetic Mitochondrial Disease
Leber's Hereditary Optic Neuropathy



Retinal ganglion cells from LHON mouse model

ReSIGHT Clinical Trial

12 subjects
- 3 arms
- Eye drop formulation

- Randomized, double-masked, vehicle-controlled trial for 52 weeks
- Elamipretide drops with fellow eye control in two arms, third arm is active drop in both eyes
- Efficacy endpoints
  - Photopic negative Response ERG
  - Visual field
  - Visual acuity and function
  - Retinal biomarkers measured by spectral domain optical coherence tomography
- Safety and tolerability

LHON

- An estimated 10,000 U.S. individuals diagnosed with LHON; G11778A mutation (inclusion criteria) comprising an estimated >70%
- Central blindness.

Lin *et al.* 10.0173 pnas.1217113109 ERG, electroretinogram ; LHON, Leber's hereditary optic neuropathy; PhNR, photopic negative response.

The Patient Journey

Stealth is committed to helping patients and families living with mitochondrial disease and to making a broad impact on the landscape of mitochondrial therapeutics by helping clinicians and researchers to better understand mitochondrial disease. As part of

this initiative, we hope to characterize a patient's journey with primary mitochondrial disease and gather insights from interviews with mitochondrial disease experts, patients, their families, healthcare providers and insurers.

Community Q&A

Q: When will Stealth begin trials in pediatric patients?

RePOWER is enrolling patients as young as age 16, and TAZPOWER is enrolling patients as young as age 12. Stealth is keenly aware of the disease burden primary mitochondrial disease presents to younger children, and is committed to developing mitochondrial therapeutics for these patients. Historically, the FDA has encouraged sponsors to demonstrate the safety and efficacy of new investigational products such as elamipretide first in adults, before allowing access to investigational drugs to pediatric patients. With MMPOWER and MMPOWER2 data available to inform it, Stealth is now conducting pre-clinical work (i.e. animal toxicology studies) to enable additional pediatric trials.

Q: What were some of the most bothersome symptoms reported that showed improvement?

Some of the PMMSA symptoms patients noted as "most bothersome" included muscle pain and muscle weakness at rest or with activity, tiredness at rest, tiredness during activity, problems with balance, vision problems, and abdominal problems.

Q: Do you know which diagnoses had the most improvements in the MMPOWER-2 trial, such as those with mtDNA mutations or large scale mitochondrial deletions?

The study was small (30 patients), and without sufficient numbers of subjects to determine whether any subsets of patients derived more benefit than others.

Q: Why was a 6MWT used as a measurement in this trial for Mito patients instead of a quality of life score like other trials in mitochondrial disease have used?

Functional and subjective measurements are both important when conducting clinical trials. The MMPOWER trials include both functional tests such as the 6MWT and subjective tests such as the NeuroQoL, the PMMSA, and other quality of life scores.

Q: Why are patients who cannot walk excluded from the PMM studies? Many patients are wheelchair bound or have other mobility issues.

Clinical trials must have endpoints that allow investigators to measure benefit in a consistent way across all patients. This is to provide regulators with confidence as to whether improvements in the clinical outcomes under evaluation – which are fatigue and skeletal muscle function in the case of Stealth's MMPOWER program - are being observed in a statistically significant manner. The 6MWT, which measures how far a

patient can walk in 6 minutes, is an endpoint measuring skeletal muscle function that is well-recognized by regulators in the US and in Europe. In order to show improvement on this outcome, Stealth needs to enroll patients who are able to walk so that they can perform this test. It may be acceptable to use walkers, canes, or other assistive devices to complete the 6MWT; further information can be found at clinicaltrials.gov.

Q: What does it mean that patients with the lowest baseline showed the greatest improvement?

Patients complete assessments such as the 6MWT at the start of the trial, before they have received any drug or placebo (baseline), and at the end of each treatment period. Patients who walked further at baseline (examples included patients who walked 500 meters at baseline, which may be considered close to normal), had smaller increases in 6MWT distance when treated with elamipretide. Patients with lower performance on the 6MWT at baseline (greater impairment relative to normal) had greater increases when treated with elamipretide. Stealth believes these observations are consistent with findings from its animal studies of skeletal muscle dysfunction, in which elamipretide was shown to improve dysfunctional (but not normal) muscle function.

Q: How is elamipretide administered?

Elamipretide is administered by subcutaneous injection for the PMM and Barth syndrome trials; a multi-use cartridge and pen, similar to an insulin pen, is under development**.**

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Special thanks to the leaders of the United Mitochondrial Disease Foundation, the Foundation for Mitochondrial Medicine and MitoAction for support and collaboration in order to make this presentation possible.
For more information visit:

www.stealthbt.com

www.umdf.org

www.mitochondrialdiseases.org

www.mitoaction.org