A randomized, double-blind, placebo-controlled phase 2/3 crossover study to evaluate the safety, tolerability and efficacy of 12 weeks' treatment with daily subcutaneous injections of elamipretide in individuals with genetically confirmed Barth syndrome followed by an open-label treatment extension.

Barth syndrome is estimated to affect ~1 in 200-400k individuals worldwide at birth.

The trial will assess approximately 12 male individuals

AGES 12+

Trial duration:

PART I: PIVOTAL
PART II: OPEN-LABEL TREATMENT EXTENSION
UP TO 36 WEEKS

TRIAL BEGAN
Q1 2017

TRIAL SITES

1 trial site
Baltimore, Maryland
HILARY VERNON, MD, PHD

ENDPOINTS

PRIMARY
Distance walked (meters) during the 6-Minute Walk Test (6MWT)
Total fatigue score on the BTHS-SA

SECONDARY
Functional assessments
Patient-reported outcomes
Safety and tolerability

For more information, please visit ClinicalTrials.gov.