TAZPOWER
Barth Syndrome

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.

Stealth BIOTHERAPEUTICS