[Meet Abe, living with Barth syndrome]

There are no approved therapies for Barth syndrome. We are asking FDA to fairly and equitably review the only investigational product in clinical development for our ultra-rare disease. We have been asking for 5 years.

10% of our global Barth syndrome population has died while we have been waiting.

Read on to learn more about our regulatory odyssey.

Barth Syndrome Foundation®

Barth syndrome is an ultra-rare genetic disease affecting **<130** American boys and young men. It's characterized by:

- Reduced lifespan primarily (>90%) due to heart dysfunction with 85% of early deaths by age 5.
- Those who survive early childhood struggle with the following symptoms, and many more:
  - **Exercise intolerance:** During trips to the grocery store, my "*13-year-old must sit in the shopping cart as he doesn't have the stamina to make it throughout the store.*"
  - **Muscle weakness:** My son "*is not strong enough to open up a bag of chips. He cannot pop off the top of a ketchup bottle and the list goes on and on.*"
  - **Fatigue:** "*As I have gotten older, my fatigue continues to get worse. Some days, I'm too tired to leave home. I need to plan ahead and pace myself for how many things I can do in one day or even in the same week. After activities, I feel worse and must rest, sometimes for the rest of the day. Sometimes, I feel so exhausted that I am sick.*" (36-year-old living with Barth syndrome)

[Visit us here]

[Learn more about our FDA advocacy efforts @ not too rare to care]

FDA Engagement Timeline

2014

BSF asked Stealth to consider development efforts. Preclinical studies initiated.

2016

Stealth presented preclinical data at BSF conference.Stealth and BSF designed TAZPOWER, a randomized controlled crossover trial and OLE.

2017-2018

TAZPOWER enrollment (fully enrolled Q1 2018)

Trial read-out. Patient Focused Drug Development Meeting

2019

Patient-led FDA listening session. Met with FDA **Division #1**; reassigned to FDA **Division #2**

2020

P3 trial compared TAZPOWER to natural history. Reassigned to FDA **Division #3**.>4K individuals **petitioned FDA** to review. Physician-led letter to FDA. EAP opened.

2021

Patient-led FDA listening session. Reassigned to FDA **Division #4**. FDA said to apply for approval, then issued RTF and would not review. OLE ends; patients go in EAP.

2022

½ Day Workshop with advocacy, experts and 30+ FDA officials. Physician-led letter to FDA. Stealth reports FDA agreed new data showing changes in heart function could support approval.

2023

FDA officials changed their minds again and say there is no clear regulatory path forward. Stealth announces possible termination of development efforts. **PETITON to FDA nearing 20k signatures.**

OLE: open-label extension | Division #1: Neurology | Division #2: Gastroenterology and Inborn Errors of Metabolism | Division #3: Rare Disease and Medical Genetics | Division #4: Cardiology and Nephrology | EAP: expanded access program | RTF: refusal to file

Meet some members of our Barth syndrome community

Declan, in EAP

Jacob, in TAZPOWER, NOT in EAP

Walker, in TAZPOWER, now in EAP

Bob, among our oldest survivors, now deceased

Media coverage of our FDA advocacy journey includes Boston Business Journal, Global Genes RareCast, Pink Sheet , STAT and more.

Peer-reviewed scientific data published in Future Medicine, Journal of Cardiac Failure, Nature, Orphanet, and more.