



## [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)

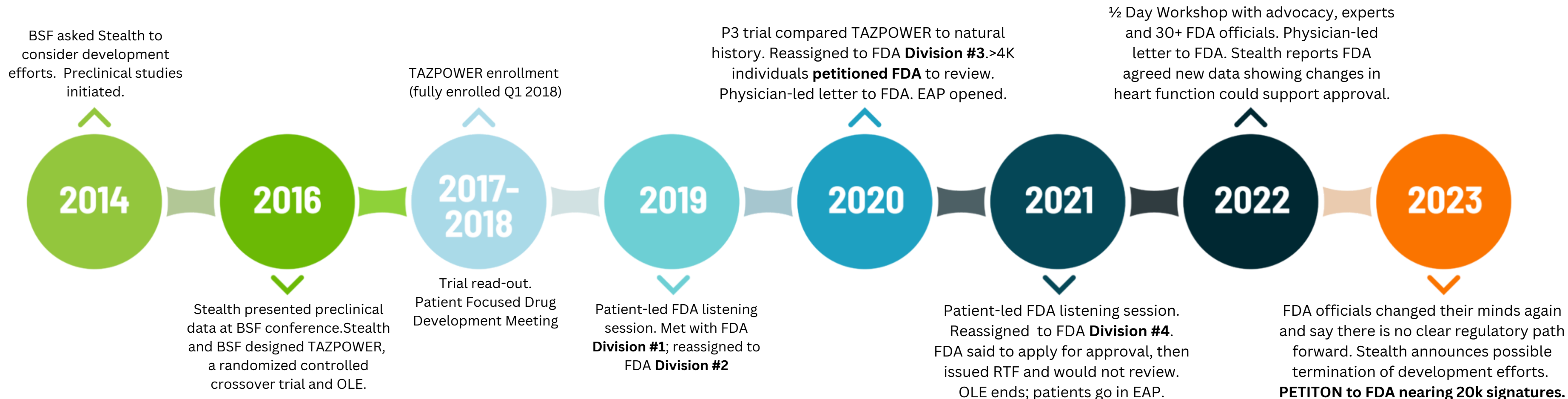
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# FDA Engagement Timeline



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.