Elamipretide Petition FAQs

What is the deadline to respond?
The deadline to sign the petition is September 30, 2020.

What is an NDA? Does Stealth submit the NDA or does BSF?
NDA stands for new drug application. Following a clinical trial, the sponsor (the company developing the drug) can choose to submit an NDA to the U.S. Food and Drug Administration (FDA). In this case, given that Barth syndrome is a very rare disease, Stealth has been granted Fast Track and Orphan Drug designations for elamipretide, allowing Stealth to have input from FDA and a quicker review period than what is typical for other diseases. Since Stealth is the drug developer, they have the responsibility of submitting an NDA.

Why does it matter what BSF says if Stealth is responsible for submitting the application for approval?
Under the 21st Century Cures Act, FDA and sponsors must consider the patient perspective when making decisions about approval of new drugs. It is our job as a global Barth syndrome community to provide that patient perspective in a clear and compelling way. We began this endeavor in 2018 at the PFDD (Patient-focused-drug-development) meeting and have shared the Voice of the Patient report with FDA. However, FDA must also appreciate that our community has an overwhelming unmet need and a desire to have access to elamipretide. We must demonstrate that affected individuals have a lower quality and length of life due to Barth syndrome, and that, given the safety profile and potential benefit of elamipretide as demonstrated in the clinical trial and open-label extension, those individuals want the opportunity to use this drug. We also are able to appeal to FDA that we understand that the drug may not help everyone with Barth syndrome, but that it is worth approval because of the potential benefit to some people in our community.

Can I provide incentives like gift cards so that more people will sign the petition?
While you can and should share your personal story and share the petition asking your family, friends and community to sign, providing incentives is not allowed and would be viewed unfavorably by FDA. We want to demonstrate that there is overwhelming support for access to elamipretide, and we can show this by having both a large number of signatures as well as compelling comments from our families and affected individuals.

Why is this called a petition?
A petition is the mechanism by which opinions of the public are collected and shared with the people who make legislative or regulatory decisions. Typically, the petition serves as a way to inform people before signing and ensures that there is overwhelming support for the request as stated in the petition.

Why did this drug work for some people and not others?
Under privacy laws that protect patient confidentiality, the identity of the individuals and details of their particular responses during the TAZPOWER clinical trial are unknown. It is not unusual for drugs to help some people more than others. Dr. Hilary Vernon, the principal investigator of the study, has presented information about the results of the trial at the American Heart Association meeting in 2019 and at the BSF SciMed Virtual Symposium in 2020, and will be discussing those results again on Friday September 25th at the BSF Roundtable.

Does BSF give the petition to the FDA or Stealth?
In parallel with this advocacy initiative, Stealth has expressed their intention to submit an NDA in the very near future to FDA. BSF has requested a meeting with FDA in order to be able to share compelling evidence from the community that we want access to elamipretide in order to offer a potentially beneficial therapy to people with Barth syndrome. If granted the meeting, the petition would be shared at that time directly by BSF. It is possible that FDA will advise BSF to provide the supporting materials and the petition responses to Stealth, and Stealth will then submit the petition with other documentation.

Would elamipretide be allowed for people who are under the age of 12 or who had transplant if approved?
Dr. Hilary Vernon, principal investigator of TAZPOWER, has stated that the inclusion and exclusion criteria that were necessary during the clinical trial were that the affected individual must be over the age of 12, medically stable and have not had a heart transplant.

How many signatures are needed?
There is not a set number or goal for the number of signatures needed; however, the petition provides us an opportunity to showcase the patient voice and demonstrate a clear and compelling case that affected individuals, parents and caregivers, and treating healthcare providers understand the safety profile and potential benefit of elamipretide and want access to the drug as a treatment choice. Asking friends and family to sign on demonstrates there is broad community support as well. Both the number of signatures and representation from our Barth syndrome family are important.

What happens next?
The petition will close on September 30th. At that time, BSF will work with Stealth and FDA to share the evidence of support that was collected.

What should I say in the comments section? Do you have a suggested response for me to use?
It is very, very important that each person provides an authentic response about how the disease has compromised his or her quality of life and how access to a drug, specifically elamipretide, could be life-changing (or could have been life-changing in the case of a deceased individual).

Do we have testimonies from others who stayed on the open label extension? Also, have they all signed the petition?
The names and identities of those who participated in the trial are confidential and are known only by the Principal Investigator and the drug company unless the individuals have voluntarily disclosed that they were involved. You can help ensure that everyone in our community is aware of this petition effort by sharing the petition with the other Barth syndrome families and affected individuals that you know. We are asking everyone to please support this appeal. As individuals sign the petition, we are able to monitor the comments and follow-up with people who voluntarily share that they participated in the trial.

Is there a suggested minimum age for affected individuals to sign it themselves?
We are suggesting that every affected individual over the age of 12 sign themselves and provide a comment, but in truth, any individual who is able to understand the petition and answer for themselves should have an opportunity to sign.

If approved in the US, what does that mean for other countries?
Each country has its own regulatory agency or governing body that approves new drugs. Historically, when a drug for a rare disease is approved in one country, approval in other countries is easier. If approved, we will work together as a global community to address these issues, so these current efforts might well help everyone.

SIGN THE PETITION