Barth Syndrome Foundation®

*"Saving lives through education, advances in treatment,
and finding a cure for Barth syndrome."*

October 20, 2021

Stealth BioTherapeutics Inc.
140 Kendrick Street, Bldg.C-West
Needham, MA 02494

Dear Reenie,

I know you share in our extreme disappointment to learn of the FDA's refusal to file the NDA for elamipretide. The FDA's decision represents a failure to consider the body of evidence, in its totality, that Stealth amassed and to which the TAZPOWER clinical trial participants contributed. It's further clear that regulators did not actively consider the perspectives of Barth syndrome patients and families represented in the reams of letters, testimonies, reports, and petitions from our community. The FDA's decision is nothing short of a tragedy for a potential treatment that has been shown to be both safe and very beneficial to a majority of individuals with Barth syndrome who have taken the drug. I am frankly appalled and aggrieved.

There is no mistaking that today constitutes a huge setback for our community. Indeed, I've come to know many of these types of moments, unfortunately, through my role at BSF. They are, however, usually the tragic grief of human loss. Working for and with members of the ultra-rare disease community is not for the faint of heart. And, as you've likely come to learn over the years in working with us, challenges tend to bolster our resilience. This community has taught me that personally. We at BSF are steadfast in our value that **we never, ever give up**. We encourage you, your team, and board of directors to not lose heart…just as we cannot, and will not!

We urge you to remain committed in Stealth's pursuit of an NDA for elamipretide in Barth syndrome. If you have not already done so, we ask that you request a Type A meeting with the FDA to understand what steps can be taken to provide a path forward for elamipretide in Barth syndrome. If that meeting does not yield clarity, we ask that you file over protest. We are demanding parity with drugs that were approved in other indications using the same criteria and considerations and ask that you not waiver in your pursuit to see through advancing elamipretide as a potential therapy into the hands of Barth syndrome patients.

In addition, while we recognize the strain this puts on your company, we implore you to continue to provide access, for as long as the drug is manufactured, to any individual with Barth syndrome who is currently taking elamipretide and desires to remain on drug. Many patients attest to the transformative clinical impact elamipretide has had on their lives. With no other therapeutic options, individuals living with Barth syndrome are in dire need of options for care, and it would be a terrible, if not unethical, scenario should they be denied access to elamipretide. We ask for your firm commitment to ensure patient access to elamipretide is preserved, without economic or access barriers.

In recognition of each child and adult who has lived with or is living with Barth syndrome, stay #BarthStrong. In the BSF community, we commonly say that together, we are stronger. That unity carries us through the darkest days and gives us the promise of a better tomorrow. We hope you and your board will fully consider our appeal as outlined in this letter and await your response.

With hope and gratitude,

Emily