**[Your Name]**
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| **Dr. Martin Makary**<br>Commissioner<br>U.S. Food and Drug Administration<br>10903 New Hampshire Avenue<br>Silver Spring, MD 20993 | **Dr. George Tidmarsh**<br>Director of the Center for Drug Evaluation and Research<br>U.S. Food and Drug Administration<br>10903 New Hampshire Avenue<br>Silver Spring, MD 20993 |

**Re: Urgent Request for Reconsideration of FDA Decision on Elamipretide for Barth Syndrome**

Dear Commissioner Makary and Dr. Tidmarsh,

I am writing with deep urgency on behalf of individuals living with Barth syndrome and the greater mitochondrial disease community who have no approved treatments. Elamipretide is a promising small molecule therapy whose FDA review process has been fraught with challenges and delays that have pushed the drug's sponsor to the brink of bankruptcy. As a mitochondrial disease advocate, I have urgent concerns that access to elamipretide will end as soon as next month, abruptly withdrawing affected individuals from treatment.

We were encouraged by your public commitment to *Conditional Approval Based on Plausible Mechanism*, which gave the rare disease community hope that FDA would apply the regulatory flexibility Congress intended for ultra-rare diseases. Our communities have advocated diligently for the right to try this medication but have been faced with repeated roadblocks to access: multiple NDA resubmissions, 4 division changes, and two PDUFA delays. Despite the AdComm's positive vote in favor of elamipretide's efficacy for the treatment of Barth syndrome, the recent refusal to reconsider the complete response letter coupled with the imposition of a longer 6-month approval review period timeline, is functionally a denial of timely access. In rare disease populations like ours, *time is not a luxury—it is the difference between life and death*.

**We respectfully and urgently request that you:**

1. **Reconsider the August 1 decision** and approve elamipretide now based on the existing data, using the accelerated approval pathway 2-month review period, with the flexibility provided in 21 CFR Part 314, Subpart H and FDA's December 2023 Rare Disease Guidance.
2. **Ensure continued access** for current Expanded Access patients and broad labeling to include infants, for whom conducting separate trials is infeasible.

This is a critical moment, and we are eager to be your partners in this process. This is an opportunity to demonstrate that FDA will act decisively for ultra-rare disease patients when compelling evidence exists and lives are at stake. Delayed access now will result in deaths that could have been preventable.

Thank you for your attention to this urgent matter and for your leadership in safeguarding the health of all Americans, especially those with the rarest and most vulnerable conditions.

Sincerely,
[Your Name]
[Your Title/Organization, if applicable]