

United States Senate  
WASHINGTON, DC 20510

June 5, 2024

Robert Califf, MD  
Commissioner of Food and Drugs  
U.S. Food and Drug Administration

Patrizia Cavazzoni, MD  
Director  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

Dear Commissioner Califf and Director Cavazzoni,

On behalf of our constituents impacted by Barth Syndrome, we write encouraged that the Food and Drug Administration (FDA) has agreed to file the New Drug Application (NDA) for elamipretide. As you know, elamipretide is a biologic treatment for those with Barth Syndrome, an ultra-rare genetic disorder with no known cure. Symptoms of Barth Syndrome include an enlarged and weakened heart, muscle weakness, recurrent infections and delayed growth. Those affected – mostly males – also have shortened life expectancy into their 40s, however most early deaths occur by age 5.

While Barth Syndrome impacts 1 in 300,000 people worldwide, we are aware of multiple Arizonans who rely on elamipretide through the Expanded Access Program. This includes Declan Comerford, a two-year-old from Rimrock, Arizona. After starting elamipretide, Declan's mother noticed her son's increased energy and strength. A few months ago, Declan was strong enough to join his mother in Washington, DC, where they met with us and members of our staffs. While one patient's experience cannot and does not prove safety or effectiveness, we are encouraged by his progress and delighted that his family is able to experience his growth.

Stories like Declan's are why we appreciate the FDA's decision to consider elamipretide's application and utilize an advisory committee. As Barth Syndrome is a condition that affects many bodily functions, we hope the advisory committee will include multi-disciplinary experts that can offer a holistic view of the drug's potential impact.

In accordance with all existing rules, regulations, and ethical guidelines, we appreciate your consideration and your continued engagement with our staffs.

Sincerely,



Mark Kelly  
United States Senator



Kyrsten Sinema  
United States Senator