Congress of the United States
Washington, DC 20515

May 14, 2025

The Honorable Martin A. Makary M.D., M.P.H.
Commissioner of Food and Drugs
U.S. Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993

Dear Commissioner Makary:

On behalf of our constituents impacted by Barth syndrome, we write to request the prompt attention of the Food and Drug Administration (FDA) to the review of elamipretide. As you know, elamipretide is the first and only treatment in clinical development for those with Barth syndrome, an ultra-rare, lethal genetic disorder with no known cure or FDA-approved therapies. Symptoms of Barth syndrome include severe and debilitating muscle weakness, exercise intolerance and fatigue, cardiac dysfunction commonly leading to premature death, recurrent infections, feeding issues, and delayed growth. Those affected – mostly males – have a shortened life expectancy, with 85% of early deaths occurring by age 5 and most survivors of early childhood passing away before their 40s.

While Barth syndrome is extremely rare, affecting 1 in 1 million male births, we are aware of at least seven individuals in Arizona living with Barth syndrome. Multiple Arizonans receive elamipretide through an expanded access program (EAP), which we understand currently provides access to the investigational medicine for approximately 20 percent of the known U.S. affected population. Declan Comerford, a three-year old from Rimrock, Arizona, has been on EAP since January 2023. Declan was diagnosed just before his first birthday following an acute cardiac crisis requiring resuscitation, life support, placement of a left ventricular assist device (LVAD) and listing for heart transplant. Declan received access to elamipretide through compassionate use and experienced profound benefits, as reported locally by his parents, Jamie Dubuque and Jesse Comerford.[1] Declan's doctor reports that Declan's recovery enabled explanation of the LVAD with his native heart intact, allowing him to avoid transplant—something never previously seen in the Barth syndrome community. Today, Declan's mom reports that he is a strong and healthy three-year old who is hitting his developmental milestones. Declan was able to join his parents in Washington, DC, in October 2024 for the Cardiovascular and Renal Drugs Advisory Committee (CRDAC) meeting, where experts concluded that elamipretide is effective for the treatment of Barth syndrome. While one patient's experience cannot and does not prove safety or effectiveness, we continue to be encouraged by his progress and delighted that his family is able to experience his growth. Declan's family has been actively advocating for elamipretide's approval since his diagnosis and remains very invested in any developments related to the regulatory process.

[1] https://www.azfamily.com/2023/12/19/family-rimrock-toddler-with-rare-disorder-fears-access-life-saving-therapy-could-end/

Our constituents have raised extreme concern about the recent announcement that the FDA did not meet its already extended Prescription Drug User Fee Act (PDUFA) action date of April 29, 2025.[2] The FDA's further delay in what has already been a 15-month "priority" review is inexplicable given the expert advisory committee feedback and our constituents' vocal and persistent feedback to us and the FDA regarding the urgency of the situation. Arizona families are anxious that the prospect of further regulatory delays and inaction may jeopardize their continued access to elamipretide, and dismayed that those not currently on EAP will likely face further delays in gaining access. We echo these concerns.

We respectfully request FDA provide our offices with clarity around elamipretide's updated PDUFA timeline. Delays, like decisions, have consequences, and in this instance, we are gravely concerned that those consequences may impact the lives of some of our most vulnerable constituents.

Given the lethality and early-onset nature of this disease, the widespread medical demand for access to therapy, your expert advisors' recommendation, your ongoing approval of compassionate use requests, and your lack of safety concerns, we hope timely completion of your review of this application is a top priority. Patient advocacy has said that over 15 percent of the Barth syndrome in the United States population has passed away since development efforts began in 2014. Time is an essential factor in the lives of our constituents.

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

Sincerely,

Mark Kelly
United States Senator

Ruben Gallego
United States Senator

[2] https://stealthbt.com/stealth-biotherapeutics-announces-delay-in-fda-action-date-for-barth-syndrome-application/

Yassamin Ansari
Member of Congress

Greg Stanton
Member of Congress

Juan Ciscomani
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