Congress of the United States

Washington, DC 20515

June 30, 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:
We are writing to you today to respectfully request your prompt attention to the review and final approval process for elamipretide-a first of its kind, experimental medication aimed at treating Barth Syndrome.

As you may be aware, elamipretide is the first and only treatment in clinical development for Barth syndrome, an ultra-rare, lethal, progressive genetic disorder with no known cure or FDA-approved treatment options. Elamipretide is also provided to patients with rare mitochondrial disorders, such as Microthalmia with Linear Skin Defects syndrome (MLS syndrome), under the Expanded Access Program (EAP). Barth syndrome is characterized by symptoms including severe and debilitating muscle weakness, exercise intolerance and fatigue, cardiomyopathy and 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.

Despite the exceedingly rare nature of Barth syndrome, which impacts 150 known individuals in the United States, we are aware of at least six individuals in the state of Georgia living with Barth syndrome. Beyond those impacted by Barth syndrome, there are a number of individuals affected by rare mitochondrial disorders in Georgia. We'd like to highlight two exceptional cases involving individuals who have had the ability to access elamipretide. Walker Burger, age 36, from Atlanta, Georgia, and his nephew, Jackson Greene, age 15, are both living with Barth syndrome. Walker lived for 19 years, many of those without a diagnosis, until the birth and diagnosis of his nephew, Jackson – who received a heart transplant at just a few months old.[1] In 2017, Walker chose to enroll as one of twelve individuals in the first ever trial for a potential therapy for Barth syndrome, the TAZPOWER study. Since then, Walker has received daily subcutaneous injections of elamipretide for over seven years. In his own words: "This is bigger than me," he says. "I've had the privilege of access. Now it's time to make sure others do too. I hope one day all boys and their families living with Barth syndrome can live the life I'm living now… because it's truly the life I could only dream about before." Walker is terrified at the prospect of losing access to elamipretide and that other Barth affected individuals,[2] like his nephew Jackson, will never get the chance to experience what he has. Sally, his mother and Jackson's grandmother, said the following: "Walker is terrified he'll lose access. I don't know what would happen to him without it, I do not want to know " she says. "And Jackson—he

[1] https://www.regulations.gov/comment/FDA-2024-N-3969-0066
[2] https://www.statnews.com/pharmalot/2022/07/26/ultra-rare-disease-drugs-fda-clinical-trials/

hasn't even had the chance yet. It's inhumane to withhold treatment that is safe, effective, and life changing."[3]

The EAP also provides access to the investigational drug for individuals like four-year-old Hope Filchak from Gainesville, Georgia. Hope lives with an exceedingly rare mitochondrial disease known as MLS syndrome, which caused her to be born with deafness, blindness, agenesis of the corpus callosum, Wolff-Parkinson White syndrome, and cardiomyopathy. Before taking elamipretide, Hope was sleeping up to 17 hours a day and missing out on learning opportunities. Hope started the drug through EAP in February 2024 and her parents, Caroline and Ben Filchak, report profound benefits since she began elamipretide – including maintenance of an ejection fraction of 46% and a decrease in BNP level from 92 to 70.1.[4] Today, Hope's mom and dad share that she is a happy, energetic four year-old who is participating in music class, playing with her friends, and learning her ABCs.[5]

We respectfully request the FDA provide our offices with clarity around elamipretide's review pathway moving forward. These stories and other reports across the country demonstrate profound and meaningful benefits in the lives of some of our state and country's most vulnerable individuals, those impacted by ultra-rare diseases with no treatment options. Given the early-onset nature of this disease, as well as the successful research conducted on the effectiveness of this treatment, approval by the FDA for elamipretide could be transformative and life-saving. We strongly urge your fair and prompt consideration of elamipretide at the earliest opportunity that aligns with the safety guidelines and processes of the FDA.

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

Sincerely,

Earl L. "Buddy" Carter
Member of Congress

Austin Scott
Member of Congress

Rick W. Allen
Member of Congress

Henry C. "Hank" Johnson, Jr.
Member of Congress

[3] https://www.barthsyndrome.org/article/2025/05/20/two-generations-one-drug-and-a-race-against-time
[4] https://www.atlantanewsfirst.com/2025/06/05/gainesville-family-fights-regain-access-life-changing-drug-4-year-old-daughter/ https://www.wsbtv.com/video/local-video/parents-want-fda-reconsider-experimental-drug/0cdb7b1e-408e-4652-9185-5dc5a643f7ae/
[5] https://www.regulations.gov/comment/FDA-2024-N-3969-0049

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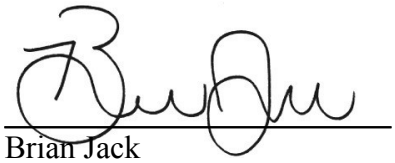

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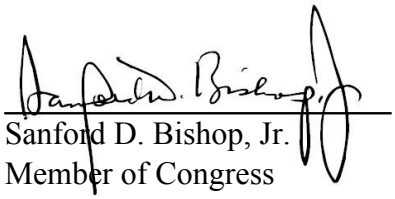

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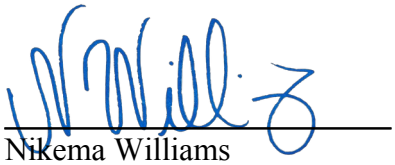

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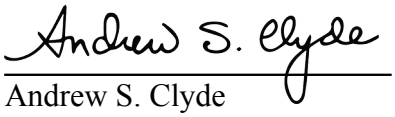

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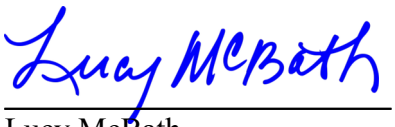

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