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20+ Barth Syndrome Advocates Urged Congress to Hold FDA Accountable to Conduct Fair & Appropriate Review of Potential Treatment

Advocates participate in 109 meetings during Rare Disease Week

WASHINGTON, DC - The Barth Syndrome Foundation (BSF), the only patient advocacy organization dedicated to Barth syndrome and saving lives around the world through education, advances in treatment and finding a cure, led a delegation of 21 Barth syndrome advocates from 16 states to Washington, DC, to meet with congressional leaders during Rare Disease Week (Feb. 26-29, 2024). Advocates participated in 109 meetings with Congressional leaders urging them to hold the U.S. Food and Drug Administration (FDA) accountable to review the New Drug Application (NDA) for elamipretide, the only potential treatment for Barth syndrome, in a fair, equitable, and appropriate manner. Families and individuals affected by Barth syndrome shared personal stories and advocated for a fair review process to ensure access to potentially life-saving treatment.

Darryl Byrd, a 42-year old male that lives with Barth syndrome and who has lost a brother and a nephew to this devastating illness, said**:** "During one of our meetings, Senator Casey recognized the importance of advocates like myself being in the room. “It's validating to know that our voices are not only heard but truly valued in shaping policy and advancing research and FDA approvals for rare diseases like Barth syndrome. Rare Disease Week was a profound experience, reminding me of the strength of our community and the impact we can have when we come together to advocate for better treatment options and support."

Despite compelling evidence of the clinical benefits of elamipretide in Barth syndrome and numerous prior meetings of the Barth syndrome community with regulators and policymakers,

the FDA has thus far refused to provide a fair and appropriate review. The Barth Syndrome Foundation's petition last Fall 2023, which garnered nearly 20,000 signatures from individuals across the nation, underscored the urgent need for FDA action.

Barth syndrome is an ultra-rare, life-threatening, progressive disease primarily affecting males, with no FDA-approved treatments currently available. Individuals with Barth syndrome face significant health challenges, including cardiac abnormalities, muscle weakness, and reduced life expectancy. Elamipretide has shown promising results in improving heart function, exercise tolerance, and muscle function in patients with Barth syndrome.

About Barth Syndrome Foundation: The Barth Syndrome Foundation is the leading organization dedicated to advancing research, education, and support for individuals and families affected by Barth syndrome. Through advocacy efforts, the foundation works to ensure access to treatments and improve outcomes for those living with Barth syndrome.

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For more information about Barth syndrome and the Barth Syndrome Foundation's advocacy efforts, visit www.barthsyndrome.org and www.nottooraretocare.org