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Families and Advocates Rally in Washington, DC Urging FDA to Conduct Fair & Appropriate Review of Potential Treatment for Barth Syndrome

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, will be leading a delegation of families and individuals affected by Barth syndrome to Washington, DC, to meet with congressional leaders. The purpose of the advocacy effort is to urge the U.S. Food and Drug Administration (FDA) to review the New Drug Application (NDA) for elamipretide, the only potential treatment for Barth syndrome, in a fair, equitable, and appropriate manner. The FDA declined to review the treatment in 2021, and despite numerous meetings of the Barth syndrome community with regulators and policymakers, the FDA has continued to deny this community access to a potentially life-saving treatment for this rare, progressive cardiac disease.

What: Advocacy Meeting with Congressional Leaders

When: February 26, 2024 - February 29, 2024 (“Rare Disease Week”)

Where: Capitol Hill, Washington, DC

Who: Representatives from the Barth Syndrome Foundation, families affected by Barth syndrome, Congressional leaders. Congressional meetings have been requested with over 60 member offices from 16 states.

Why: Despite compelling evidence of the clinical benefits of elamipretide in Barth syndrome, 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. Families and advocates will share personal stories and advocate for a fair review process to ensure access to potentially life-saving treatment for those affected by Barth syndrome.

Background: Barth syndrome is an ultra-rare, life-threatening genetic 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. However, regulatory hurdles have hindered its progress toward approval.

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.

Media Contact: For media inquiries or to schedule interviews, please contact Anna Stallmann at media@annacomms.com or 708-476-1258.

Note: Members of the media are invited to speak with families and advocates affected by Barth syndrome. Media will not be permitted inside advocacy meetings with congressional leaders.

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