



MitoAction Advocates for Barth Syndrome Community in FDA Listening Session

FOR IMMEDIATE RELEASE

[Boston, MA, April 1, 2024] – MitoAction, a leading nonprofit organization dedicated to improving the lives of individuals and families affected by mitochondrial disease, recently convened a pivotal FDA listening session on March 22nd, 2024. This session served as a crucial platform for MitoAction to underscore the persistent unmet needs and ongoing challenges faced by the Barth syndrome community.

Barth syndrome is an ultra-rare, life-threatening genetic disorder characterized by cardiac and skeletal muscle weakness, as well as immune system dysfunction. Individuals living with Barth Syndrome and their families continue to confront significant obstacles in accessing appropriate care and therapies. There are currently no FDA approved therapies for Barth syndrome.

The FDA listening session organized by MitoAction provided an invaluable opportunity for stakeholders within the Barth syndrome community to engage directly with regulatory authorities. During the session, participants, who included Key Opinion Leaders and moms of boys diagnosed with this rare disorder, shared compelling insights into the lived experiences of individuals affected by Barth syndrome, highlighting the urgent need for accelerated research and expanded treatment options.

"MitoAction remains steadfast in our commitment to advocating for the Barth syndrome and the entire mitochondrial disease community," said Kira Mann, CEO at MitoAction. "Our recent FDA listening session was a pivotal moment for amplifying the voices of those affected by this devastating condition. By fostering dialogue and collaboration with the Barth Syndrome Foundation and the FDA, we are working tirelessly to drive positive change and improve outcomes for individuals living with Barth syndrome."

Through collaborative efforts with regulatory agencies like the FDA, MitoAction continues to champion initiatives aimed at advancing research, accelerating drug development, and enhancing access to care for individuals and families affected by mitochondrial diseases, including Barth syndrome.

For more information about MitoAction and its advocacy efforts, please visit www.mitoaction.org



About MitoAction: MitoAction is a nonprofit organization dedicated to improving the quality of life for individuals and families affected by mitochondrial disease through support, education, advocacy, and clinical research initiatives. Since its founding in 2005 MitoAction has been at the forefront of empowering the mitochondrial disease community and driving positive change in the field of mitochondrial medicine. MitoAction serves families from across the U.S. and the globe, working tirelessly to offer comprehensive, up-to-date, expert resources, information, and support at no cost to any patient, family, clinician, educator, or community member. Learn more at www.mitoaction.org.

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