Mitochondrial Disease Advocacy Coalition (MDAC) Advocacy Priorities

September 2026

Mitochondrial Disease – A Case for Congressional Priority

Mitochondria are cellular organelles that convert food and oxygen into energy. Primary mitochondrial disease occurs when mitochondrial function is impaired. These diseases can affect both children and adults. Mutations in many genes in the mitochondrial genome (which are maternally inherited) are associated with numerous rare mitochondrial diseases that affect a wide range of body systems and organs and exhibit diverse symptoms. Those that affect the neurological and musculoskeletal systems are among the most devastating.

In addition to primary mitochondrial disease, mitochondrial dysfunction is increasingly recognized as contributing to a variety of common diseases that impact millions of Americans, including Alzheimer's, Parkinson's, Huntington's, diabetes, heart disease, stroke, and a wide range of cancers. Autoimmune diseases such as multiple sclerosis, lupus, and rheumatoid arthritis may have a mitochondrial basis. Less than one-percent of the more than 300 known variants of mitochondrial disease have an approved therapy. Ongoing research to better understand mitochondrial physiology has helped grow clinical care standards and inform the development of precision therapies.

Join the Congressional Caucus on Mitochondrial Disease

The Mitochondrial Disease Advocacy Coalition (MDAC) is asking Members of the House of Representatives to join the bipartisan Congressional Mitochondrial Disease Caucus to raise awareness of mitochondrial disease and dysfunction. A robust Congressional Mitochondrial Disease Caucus provides a forum for members of Congress and their staff to learn about mitochondrial disease and related issues, including prevalence, connections to other illnesses, and ongoing research. The Caucus supports cutting-edge research into mitochondrial disease to develop therapies and cures for specific diseases and to generate new insights into neurological, autoimmune, and other related disorders through agencies such as the National Institutes of Health (NIH) and the Department of Defense (DoD).

The Caucus is co-chaired by Reps. Brian Fitzpatrick (R-PA) and Jim McGovern (D-MA) and includes a bipartisan group of members committed to advancing awareness and research.

Offices interested in joining the Caucus should contact:

- Bella Edo (Rep. McGovern): bella.edo@mail.house.gov
- Clare Dentner (Rep. Fitzpatrick): clare.dentner@mail.house.gov

Co-Sponsor the Medical Foods & Formulas Access Act (H.R. 5684/S. 3304)

The bipartisan *Medical Foods & Formulas Access Act* (previously titled the Medical Nutrition Equity Act) would provide coverage under Medicare, Medicaid, the Children's Health Insurance Program, and Federal Employee Health Benefits for a carefully defined group of nutritional interventions prescribed by a medical professional for a limited group of identified diseases, including inherited metabolic disorders. These nutritional interventions are critical in addressing the consequences of mitochondrial disease. Generally, a patient's physician determines

the specific nutritional interventions, which vary depending on the individual Mito patient's diagnosis, clinical symptoms, and weight. Out-of-pocket costs for these nutritional interventions can exceed $1,000 per month.

Support Continued DoD Investment in Mitochondrial Research

The Department of Defense, through the Peer-Reviewed Medical Research Program (PRMRP), has been a strong partner in support of mitochondrial disease and dysfunction research. There is a documented connection between mitochondrial function and issues of concern to the military, including its central role in traumatic brain injury (TBI), Persian Gulf Illness, and enhanced energetics in casualty care. Mitochondrial disease has been included in the PRMRP's list of eligible research topics since Fiscal Year 2015, with DoD awarding approximately $80 million for studies on topics such as the genetic determinants of mitochondrial disease, its effects on respiration and brain function, and the development of targeted therapies.

Earlier this year, MDAC and its members submitted numerous appropriations requests urging Congress to continue its longstanding support for the PRMRP and mitochondrial disease research. As Congress completes the FY2027 appropriations process, MDAC respectfully requests that Congress provide robust funding for the PRMRP—at not less than $370 million—and continue to include mitochondrial disease among the conditions and diseases eligible for competitive research funding. The FY2026 Senate Defense Appropriations Committee report provided $370 million for the PRMRP and specifically included mitochondrial disease as an eligible research topic. The Senate has not yet released its FY2027 Defense appropriations bill and report, so it is especially important to retain this designation and robust funding for FY2027.

Encourage the National Institutes of Health to Prioritize Mitochondrial Disease Research

The NIH has a long history of supporting mitochondrial disease research through numerous separate institutes and centers, including the National Institute of Child Health and Human Development, the National Institute of Diabetes and Digestive and Kidney Diseases, and many others. Various MDAC groups have worked with NIH to conduct workshops on mitochondrial disease and to create a cross-cutting mitochondrial disease working group.

As the science and understanding of mitochondrial disease and therapeutic interventions have advanced, MDAC believes NIH should establish at least one Center of Excellence in Primary Mitochondrial Disease. Earlier this year, MDAC and its members submitted numerous appropriations requests seeking continued congressional support for mitochondrial disease research at NIH, including report language calling for a dedicated primary mitochondrial disease research center award. The Senate FY2027 Labor/HHS/Education appropriations bill and accompanying report have not yet been released; accordingly, MDAC's immediate priority is ensuring that this important language is retained for FY2027 and supported in subsequent fiscal years.

Please support the inclusion and retention of the following language in the FY2027 Labor/HHS/Education appropriations bill committee report:

Mitochondrial Disease Research — The Committee recognizes the critical need for the NIH to accelerate and prioritize research on primary and secondary mitochondrial disease. A constellation of rare diseases linked to impaired mitochondrial function requires further research to drive promising interventions through the FDA approval process. Continued research is essential to better understand disease mechanisms and improve patient outcomes. Additionally, growing evidence underscores the significant role of mitochondrial dysfunction in a range of neurodegenerative and neuromuscular conditions, including Alzheimer's, Parkinson's, ALS, Muscular Dystrophy, and multiple sclerosis. The Committee urges the NIH to expand its commitment to mitochondrial disease research by fostering interdisciplinary collaborations and ensuring the issuance of at least one dedicated primary mitochondrial disease research center award. (National Institutes of Health; Office of the Director).

MDAC urges Congress to sustain robust federal investment in mitochondrial disease research at NIH and DoD and preserve mitochondrial disease as a priority in FY2027 and future appropriations.

Encourage Timely and Flexible FDA Review of Rare Disease Therapies

MDAC appreciates the FDA's recent efforts to bring greater speed, flexibility, and predictability to rare disease drug development, including greater consideration of the totality of evidence, innovative trial designs, and the unique challenges of developing therapies for very small patient populations.

We've seen this flexibility in action with the agency's work on the resubmission of SL1009 (sodium dichloroacetate, also known as DCA) for the treatment of Pyruvate Dehydrogenase Complex Deficiency (PDCD), a life-threatening mitochondrial disease with no approved therapy. After much back and forth, the FDA found a path forward that did not require an additional clinical trial, which would have meant further delays for a community in desperate need.

And while we applaud that decision, it's critical we see this flexibility applied to the actual resubmission of the New Drug Application for DCA, which was assigned a PDUFA date of December 30, 2026. The PDCD population has an urgent, unmet medical need – delay, or even worse, denial, would mean more suffering. By the time the PDUFA action date arrives, families will have waited over two full years since the therapy first came under FDA review.

Given this, MDAC respectfully asks Members of Congress to encourage FDA to:

- Complete its review of DCA promptly, applying the full regulatory flexibility available for rare disease therapies and appropriately considering the totality of available evidence; and
- Build on FDA's recent progress by applying these flexibilities consistently and predictably across ultra-rare mitochondrial diseases, particularly where diseases are life-threatening, and patients have no FDA-approved treatment options.