Myotonic Dystrophy FOUNDATION

February 28, 2025

The Honorable Mike Johnson
Speaker of the House of Representatives
Washington, D.C. 20015

The Honorable Hakeem Jeffries
House Democratic Leader
Washington, D.C. 20015

The Honorable John Thune
Senate Majority Leader
Washington, D.C. 20510

The Honorable Chuck Schumer
Senate Democratic Leader
Washington, D.C. 20510

Dear Speaker Johnson, Leader Jeffries, Majority Leader Thune, and Leader Schumer,

On behalf of the Myotonic Dystrophy Foundation (MDF), the over 150,000 Americans living with myotonic dystrophy, our clinical and research community, and our families, I write to express our deep concern regarding the arbitrary termination of federal employees, the suspension of essential operations, and the reduction and postponement of federal grants across the U.S. Department of Health and Human Services (DHHS).

Myotonic dystrophy is a multi-systemic inherited genetic disease that affects as many as 1 in 2,100 people in the U.S. There is no cure nor FDA approved treatments for this progressive disease, which affects both adults and children. The disease is caused by a mutation in a gene required for normal muscle function, which prevents the gene from functioning properly. Individuals affected by myotonic dystrophy may have skeletal muscle problems, heart function abnormalities, breathing difficulties, early cataracts, issues with speech and swallowing (dysarthria and dysphagia), cognitive impairment, excessive daytime sleepiness, diabetic symptoms, and more.

The federal government has made modest investments in myotonic dystrophy research, and we are closer than ever to having the first FDA approved treatment for the disease. We are deeply concerned that the recent DHHS decisions will cripple critical scientific advances, delay the development and approval of new drugs and gene therapies, devastate efforts to protect public health, and degrade the operations of Medicare and Medicaid, all of which are vital to the **30 million individuals** living with myotonic dystrophy and other rare diseases in the US.

Every day there is a delay in research for myotonic dystrophy extends the length of time it will take to develop successful treatments to alleviate symptoms and find a cure. The nation's top myotonic dystrophy scientists have been clear that even a delay of a few months in the federal grant announcement, review, and funding distribution process will

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have a lasting and devastating impact across all areas of research, especially those that focus on rare diseases.

Attracting and retaining new researchers within the myotonic dystrophy research community is essential to ensuring continued progress and breakthroughs for patients and families. These DHHS policy changes will severely affect the research community's ability to foster a strong and growing network of researchers, especially early career scientists that include graduate students, post-doctoral fellows, and junior investigators, who need funding and infrastructure to continue their research.

Drug developers are now working on several late stage clinical trials with promising therapeutic candidates, which may become the first FDA approved treatments for myotonic dystrophy. Decisions to reduce drug review staff at the FDA could delay the approval of these treatments and deny thousands of Americans living with myotonic dystrophy access to safe and effective treatments.

Today, on Rare Disease Day, we urge you to take action to protect the health and well-being of individuals with myotonic dystrophy by suspending these moves, holding public hearings, and working with the public to ensure these vital federal health agencies can continue to perform their essential missions. Our families cannot afford these setbacks – every minute counts.

We thank you for your kind attention to these public comments and for eliminating these barriers to finding a cure for myotonic dystrophy.

Sincerely,

Tanya Stevenson, EdD, MPH
Chief Executive Officer
Myotonic Dystrophy Foundation

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