Myotonic Dystrophy FOUNDATION

The Impact of Funding Cuts & Policy Changes on Myotonic Dystrophy Research

Delays in myotonic dystrophy research mean longer waits for families hoping for answers, care, and treatment options.

Understanding Myotonic Dystrophy

Myotonic dystrophy (DM) is a rare inherited genetic disease that affects as many as 1 in 2,100 people, or over 150,000 individuals in the U.S. There are no FDA-approved treatments for myotonic dystrophy, which impacts both adults and children.

Individuals affected by DM may experience skeletal muscle problems, heart function abnormalities, breathing difficulties, cataracts, issues with speech and swallowing, cognitive impairment, excessive daytime sleepiness, or diabetic symptoms.

Why DM Research Funding Matters

Federal funding cuts and staffing reductions are stalling myotonic dystrophy (DM) research. Scientists report grant delays, hiring freezes, and project shutdowns—slowing progress toward life-changing treatments. To better understand these challenges, the Myotonic Dystrophy Foundation (MDF) surveyed myotonic dystrophy researchers from academia, industry, and clinical research in March 2025.

1. Delayed Research Funding Has Already Impacted Progress Toward DM Treatments

CHALLENGES FACED BY DM RESEARCH

- Funding delays have prevented hiring and launching new studies, slowing DM treatment progress.
- Disruptions to research projects and stalled collaborations delay discoveries.
- Funding shortages and hiring freezes are limiting training for future DM scientists.

What Research Funding Delays Mean for Families with DM?

- Fewer scientists in DM → Fewer breakthroughs & discoveries.
- Longer treatment delays → Families left waiting with no options.
- Fewer clinical trials → Slower progress toward a cure.

55% (11 / 20 respondents) report that policy changes have already impacted their work.

"The delay of so many study sections, grant reviews, funding councils, and funding announcements for new research grants, even for a few months, will have a lasting and devastating impact across all areas of research, but especially those that focus on rare diseases."

- DM RESEARCHER

2. Recruitment and Retention Challenges Are Reducing the DM Research Workforce

DM RESEARCH STAFFING CHALLENGES

1. Reduced opportunities for early-career researchers who become the next generation of DM experts
2. Staff retention difficulties due to funding uncertainty
3. Hiring challenges for new DM researchers or staff

82% (14 / 17 respondents) say policy changes are making it harder to recruit and retain researchers.

"The global disruptions of NIH function have set back the progress of medical research. Most importantly, the long-term effect will be dissuading the best and the brightest from pursuing careers in research."

- DM RESEARCHER

3. Policy Changes Will Impact Myotonic Dystrophy Clinical Trials and Treatments

Shift in funding priorities away from DM	→	More focus on common diseases, diverting resources & slowing momentum for DM research.
Less federal grant funding available	→	Increased competition for fewer grants, making it harder for DM researchers to sustain their work.
Delays in research funding approvals	→	Hiring freezes & project delays, preventing scientists from launching critical studies.
Staffing cuts at the FDA	→	Fewer personnel to review and approve treatments, leading to delays in drug approvals and longer waits for DM therapies.
Clinical trial sites struggling with funding and staffing	→	Fewer resources mean fewer trials can be conducted, limiting treatment options for DM patients.
Fewer clinical trials	→	Reduced opportunities to test new treatments, slowing progress toward a cure.

90% (18 / 20 respondents) anticipate that policy changes will impact DM clinical trials.

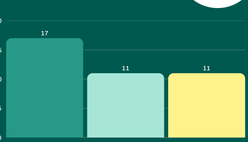
"Postponing NIH council and study section meetings [which convene to review and approve research grants] will have a ripple effect on DM funding and likely have long-term impacts on research and clinical trials."

- DM RESEARCHER

4. Funding Constraints Are Forcing Changes in DM Research Priorities

Myotonic dystrophy researchers are being forced to adjust their approach by:

- **Looking for Alternative Funding**
  → Takes away valuable time from conducting research, slowing progress toward treatments.
- **Adjusting Study Designs**
  → Budget constraints force modifications that may not reflect the best scientific approaches, potentially compromising the quality of research outcomes.
- **Shifting Research Focus**
  → Redirecting resources away from DM research reduces momentum toward better understanding and developing treatments for the disease.



"These policy changes will ultimately disrupt the DM research community immediately and for many years. The uncertainty over funding streams is likely to impact DM research and the speed at which therapeutics enter the clinic."

- DM RESEARCHER

Learn More and Support the Future of Myotonic Dystrophy Research

Funding cuts are only one of the challenges facing the DM research community—but there's more you can do to help!

- Visit the MDF website at www.myotonic.org to:
  - Learn more about DM and its impact on individuals and families
  - Explore current research initiatives and clinical trials
  - Access resources for patients, families, and healthcare providers
  - Discover ways to support advocacy, awareness, and research efforts

Myotonic Dystrophy FOUNDATION

Connect with the Myotonic Dystrophy Foundation

The Myotonic Dystrophy Foundation (MDF) is the leading global advocacy organization advancing community, care, and a cure for individuals living with myotonic dystrophy.

Learn more at www.myotonic.org.