Myotonic Dystrophy FOUNDATION

Myotonic Dystrophy Foundation Advocacy Day Talking Points:

General Suggestions:

1. Members of Congress and staff may be unfamiliar with myotonic dystrophy, please say "myotonic dystrophy" and avoid using "DM" in your congressional meetings.

2. Begin by thanking the staff person for meeting with you and your state delegation members.

3. Make sure to leave time in the meeting to make your request and for the congressional staff to ask questions.

4. Remember to write down the name and contact information of the Congressional staff you meet with on your feedback form.

Congressional Meeting Talking Points:

- My name is (JANE DOE), and I am a constituent who lives in (City, State). Ask your state delegation colleagues to quickly introduce themselves.

- We are here today with the Myotonic Dystrophy Foundation (MDF) as part of our 2023 annual conference. MDF is leading efforts to improve care for individuals with myotonic dystrophy and find treatments and a cure.

- Myotonic Dystrophy is a multi-systemic inherited disease that affects as many as 1 in 2,100 people or over 150,000 people in the U.S. It is caused by a mutation in a gene required for normal muscle function, which prevents the gene from functioning properly.

  The disease is inherited, and individuals affected by it may have skeletal muscle problems, heart function abnormalities, breathing difficulties, cataracts, issues with speech and swallowing, cognitive impairment, excessive daytime sleepiness, or diabetic symptoms. It can reduce life expectancy. There are currently no FDA-approved treatments, and federal funding has lagged behind other similar genetic disorders.

- If you are living with myotonic dystrophy, briefly tell your story, including:
  - Year of diagnosis, how long did it take
  - Impact on daily life, including top three symptoms
  - Significant medical life events
  - Impact on employment and family life

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- Today, we are here to ask for your support on two critical issues:

1. **Maintain Myotonic Dystrophy Eligibility as Part of the Senate-Led Department of Defense (DOD) Peer Reviewed Medical Research Program.**

    - We are grateful the Senate, for the 7th year in a row, included myotonic dystrophy as an eligible condition for research funding under the Peer-Reviewed Medical Research Program (PRMRP) as part of the recently passed fiscal year 2024 Department of Defense funding bill.

    - Since its inclusion in 2018, PRMRP has funded over $16 million in new cutting-edge myotonic dystrophy research, which has helped advance our understanding of this rare genetic disorder and bring us closer to the first-ever FDA-approved treatments and, eventually, a cure for this rare genetic disorder.

    - We urge you to **PRESERVE** this program and the funding level included in the Senate DOD Appropriations Committee report in the final FY2024 appropriation legislation.

2. **Support Senate Appropriations Committee's Top-Line funding for the National Institutes of Health in the Fiscal Year 2023 Labor, Health and Human Services Appropriations Legislation.**

    - There are no Food and Drug Administration (FDA) approved treatments for myotonic dystrophy, and NIH funding for this disease has lagged behind other similar disorders.

    - Top-line funding is necessary to make additional critical investments in myotonic dystrophy research to advance our scientific understanding of this rare genetic disorder and accelerate efforts to finally bring to market FDA-approved treatments.

    - This funding is necessary to support recent Congressional report language encouraging NIH to explore the most effective approaches to support trans-NIH research on repeat expansion disorders, including myotonic dystrophy, and consider new funding mechanisms to support scientific discoveries that will lead to treatments and cures for myotonic dystrophy and related genetic disorders.

Myotonic Dystrophy Foundation Applauds Senate for Including Myotonic Dystrophy in Peer Reviewed Medical Research Program for 7th Year in a Row

As part of the Myotonic Dystrophy Foundation's 2023 Annual Conference in Washington D.C., advocates from across the country are visiting our Senators on September 7th to express our gratitude for including myotonic dystrophy as an eligible condition for research funding under the Peer-Reviewed Medical Research Program (PRMRP) for the 7th year in a row as part of the recently passed fiscal year 2024 (FY24) Department of Defense (DoD) funding bill. We urge Congress to preserve this program and the funding level included in the committee report in the final FY24 appropriation legislation. Since its inclusion in 2018, PRMRP has funded over $16 million in new cutting-edge myotonic dystrophy research, which has helped advance our understanding of this rare genetic disorder and brings us closer to the first-ever FDA-approved treatments and, eventually, a cure.

Background

Myotonic dystrophy is a multi-systemic inherited genetic disease that affects as many as 1 in 2,100 people or over 150,000 individuals in the United States. The disease is caused by a mutation in a gene required for normal muscle function, which prevents the gene from functioning correctly. Individuals affected by myotonic dystrophy may have skeletal muscle problems, heart function abnormalities, breathing difficulties, cataracts, issues with speech and swallowing (dysarthria and dysphagia), cognitive impairment, excessive daytime sleepiness, or diabetic symptoms. It causes disability and can reduce life expectancy. There are currently no Food and Drug Administration (FDA) approved treatments for myotonic dystrophy, and federal funding for myotonic dystrophy has lagged other similar genetic disorders. PRMRP funding is vital to advancing science in this neglected field and improving the quality of life for American military personnel and civilians living with myotonic dystrophy.

Examples of Recent Department of Defense/PRMRP Funded Myotonic Dystrophy Research:

- **Massachusetts General Hospital:** Extracellular Vesicles as Therapeutic Vehicles for Myotonic Dystrophy
- **University of Illinois, Champaign/Urbana:** Role of Neuron-Specific Giant Ankyrins Isoform in Developing Cardiac Arrhythmia for DM Type 1
- **Scripps Research Institute/University of Florida:** Design and Study of Small Molecules That Cleave the RNA That Causes DM Type 1

About The Myotonic Dystrophy Foundation

The Myotonic Dystrophy Foundation (MDF) was founded in 2007 by families seeking answers and support. The mission of MDF is "Community, Care and a Cure." MDF supports and connects the myotonic dystrophy community, provides resources and advocates for care, and accelerates research toward treatments and a cure. MDF is the leading global advocate for helping individuals and families navigate the myotonic dystrophy disease process. It is often the first resource contacted by newly diagnosed patients, their families, their social workers, and their physicians worldwide. To learn more, visit: www.myotonic.org

Myotonic Dystrophy FOUNDATION

Myotonic Dystrophy Foundation Urges Congress to Support $47.8 Billion for the National Institutes of Health as Part of Final FY24 Labor-HHS Appropriations Bill

Funding Will Help Fund New Myotonic Dystrophy Research

The Myotonic Dystrophy Foundation urges Congress to support the Senate Committee on Appropriations passed $47.8 billion funding level for the National Institutes of Health as part of the final fiscal year 2024 Labor, Health, and Human Services Appropriations legislation. There are no Food and Drug Administration (FDA) approved treatments for myotonic dystrophy, and NIH funding for myotonic dystrophy has lagged other similar genetic disorders. This funding level is necessary to make additional critical investments in myotonic dystrophy research to advance our scientific understanding of this rare genetic disorder and accelerate efforts to finally bring to market FDA-approved treatments. Further, this funding is necessary to support recent congressional report language encouraging NIH to explore the most effective approaches to support trans-NIH research on repeat expansion disorders, including myotonic dystrophy, and consider new funding mechanisms to support scientific discoveries that will lead to treatments and cures for myotonic dystrophy and related genetic disorders.

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Researchers at the **University of Florida, University of Rochester, Stanford University, Emory University, Baylor College of Medicine**, and others are making progress in studying how repeat expansions disrupt healthy gene regulation and cause disease symptoms; however, federal funding and coordination have been limited. Recognizing an opportunity to advance science to accelerate the discovery of new treatments and cures for these genetic disorders and related conditions, the House Appropriations Committee included report language in the fiscal year 2023 Labor, Health and Human Services Appropriations legislation to establish a trans-NIH Repeat Expansion Disease Initiative (REDI) within the Office of the Director to increase federal funding for research on repeat expansions utilizing new funding mechanisms across multiple institutes. Increased NIH funding is critical to funding this research, which holds the potential to advance new groundbreaking treatments for myotonic dystrophy and related genetic disorders caused by repeat instability and toxic RNA.

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