

HOUSE

Help End Duchenne Muscular Dystrophy

By Supporting Research, Care Considerations, and Therapy Development

Sign the FY23 Duchenne MD Appropriations Letter

[Use this link to sign on](#) (*link will only work for hill staffers with Quill accounts*)

Dear Colleague –

Duchenne muscular dystrophy and the milder form, Becker muscular dystrophy, are rare X-linked recessive diseases belonging to a group of conditions known as dystrophinopathies. Duchenne and Becker are caused by mutations in the dystrophin gene leading to the absence or reduced production of the dystrophin protein, a protein that is key to stabilization of the muscle cell membranes. This lack of dystrophin in muscle leads to progressive muscle weakness and loss over time causing premature death. Affecting 1 of every 5,000 boys, Duchenne is typically diagnosed during the first few years of life. A muscle wasting disorder, Duchenne gradually robs children of their ability to walk by their teenage years. Over time, their muscles weaken further to the point of paralysis, with most patients living only into their late 20s.

Although there are now five FDA-approved therapies that may help slow its progression, there is currently no cure for Duchenne or Becker. However, there is reason for hope, due in large part to the support and research funding Congress has provided:

- More than 35 potential therapies are in various stages of clinical testing.
- The life expectancy of the average Duchenne patient has increased by about 10 years over the past 10 years, driven in large part by development and dissemination of Care Standards.

Now is the time to continue building upon these successes and move closer to achieving the goal of ending Duchenne and Becker by supporting research, public health, and therapy development initiatives. We invite you to help keep this momentum going by signing the FY23 Duchenne Muscular Dystrophy appropriations sign-on letter. This year, we are requesting language to:

- Maintain CDC's Muscular Dystrophy Program funding of \$8 million.
- Maintain funding for the Duchenne Muscular Dystrophy Research Program within DOD's Congressionally Directed Medical Research Programs (CDMRP) at \$12 million.
- Evaluate the impact of efforts such as the Care Considerations and Certified Duchenne Care Centers on outcomes and identify remaining gaps.
- Given recent therapy's success in extending lifespan for Duchenne patients, agencies should focus more on therapies and care considerations that have the potential to improve

how patients feel, function, and survive. This includes improvements to care related to the impact of progressive disability on the mental health of patients and their families as well as improvements in cardiac care and treatments.

- Support research at NIH on challenges related to gene therapies and ask FDA to enable the development pathway of genetic therapies across the spectrum, particularly those that lack the patient population to incentivize drug developers.

The full request is below. We urge you to co-sign this letter to advance these priorities and bring us closer to the day of ending Duchenne.

Please [use this link to sign on](#). Note, this link will only work for Hill staffers with Quill accounts. If you have any questions, please contact Christina McCauley (christina.mccauley@mail.house.gov) with Rep. Matsui or Davis Michols (davis.michols@mail.house.gov) with Rep. Balderson.

Sincerely,

DORIS MATSUI
Member of Congress

TROY BALDERSON
Member of Congress

DATE

The Honorable Rosa DeLauro
Chair
Labor, HHS, Education,
& Related Agencies Subcommittee
Committee on Appropriations
2358-B Rayburn House Office Building
Washington, DC 20515

The Honorable Tom Cole
Ranking Member
Labor, HHS, Education,
& Related Agencies Subcommittee
Committee on Appropriations
2358-B Rayburn House Office Building
Washington, DC 20515

The Honorable Sanford Bishop Jr.
Chair
Agriculture, Rural Development, FDA,
& Related Agencies Subcommittee
Committee on Appropriations
2362-A Rayburn House Office Building
Washington, DC 20515

The Honorable Andy Harris
Acting Ranking Member
Agriculture, Rural Development, FDA,
& Related Agencies Subcommittee
Committee on Appropriations
2362-A Rayburn House Office Building
Washington, DC 20515

The Honorable Betty McCollum
Chair
Defense Subcommittee
Committee on Appropriations
H405 U.S. Capitol Building
Washington, DC 20515

The Honorable Ken Calvert
Ranking Member
Defense Subcommittee
Committee on Appropriations
H405 U.S. Capitol Building
Washington, DC 20515

Dear Chairmen DeLauro, Bishop, and McCollum and Ranking Members Cole, Harris, and Calvert:

Thanks in large part to the leadership of Congress starting with the passage of the Muscular Dystrophy Community Assistance, Research and Education (MD CARE) Act in 2001, significant progress has been made over the past 20 years in the fight to end Duchenne Muscular Dystrophy (Duchenne MD), the most common lethal genetic disorder diagnosed during childhood. We are writing to urge that, as you prepare your Fiscal Year 2023 Appropriations bill, you include provisions to help further these pursuits, particularly to advance scientific breakthroughs, to accelerate therapy development, to ensure consistent high-quality care across the country, and to help improve life for patients and caregivers affected by this disease.

As a result of the MD CARE Act and subsequent amendments, federal commitments to research have expanded, helping spur scientific breakthroughs to develop potential therapies. These commitments have also leveraged significant non-federal funding from academic institutions, industry, and venture investors in a true public-private partnership model. In addition to research breakthroughs, the MD CARE Act has helped capture important epidemiological data, information that has helped standardize and improve patient care and to inform payer decision making.

Our Fiscal Year 2023 Duchenne MD appropriations request contains language and provisions to help continue and strengthen these and other ongoing initiatives. Specifically, the request would:

- Maintain funding for CDC’s Muscular Dystrophy Program of \$8 million.
- Maintain funding for the Duchenne Muscular Dystrophy Research Program within DOD’s Congressionally Directed Medical Research Programs (CDMRP) of \$12 million.
- Direct the CDC to:
 - Update the Care Considerations to take into account that now that life expectancy has increased, more attention needs to be paid to clinical care that improves quality of life, including clinical best practices around cardiac care and the impact of progressive disability on the mental health of patients and their caregivers.
 - Consider including information about current approved therapies in the Care Considerations.
 - Provide the previously requested report describing how the Muscular Dystrophy Program funding is allocated, including evaluation of the impact of the Care Considerations as well as differences in care and outcomes between Certified Duchenne Care Centers and non-certified centers with the MD-STARnet network.
 - Work with stakeholders to extract and evaluate the utility of common data elements in electronic health records (EHR) to improve care, understand disease outcomes, and model disease progression.
- Urge the National Institutes of Health (NIH) to:
 - Support research aimed at gene therapy safety utilizing viral vectors and support the development of less immunogenetic non-viral delivery systems.
 - Invest in the development of more sensitive outcome measures and biomarkers for both Duchenne and Becker.
 - In addition to research that extends lifespan, research should be targeted toward clinical care that improves how patients feel, function and survive.
 - Work across agencies, specifically the National Health, Lung, and Blood Institute (NHLBI) with the National Institute of Neurological Disorders and Stroke (NINDS), to establish a research network to follow patients throughout the lifespan to fully clinically characterize cardiac muscle function and better establish the relationship between cardiac muscle function and the impact of its progressive deterioration on both lifespan and quality of life.

- Direct the Food and Drug Administration (FDA) to:
 - Conduct a stakeholder meeting to discuss enabling the development pathway of genetic therapies across the spectrum, particularly those that lack the patient population to incentivize drug developers.
 - Consider the appropriate use of the accelerated approval pathway to provide timely access to treatments that may materially improve how patients feel, function and survive.

Much progress has been achieved in recent years, but much more work remains to be done. The FY 2023 Duchenne MD request will focus federal energies toward the highest priority needs to hopefully accelerate the development of therapies and treatments and to improve life for all patients impacted by this disease.

Below is the specific language we are requesting:

Centers for Disease Control and Prevention

BIRTH DEFECTS, DEVELOPMENTAL DISABILITIES, DISABILITIES, AND HEALTH
 \$8M for Muscular Dystrophy (level funding from FY22)

Duchenne and Becker Muscular Dystrophy. – The Committee supports the NCBDDD Muscular Dystrophy Program research and disease surveillance initiatives, including the Duchenne Muscular Dystrophy Care Considerations. The Committee directs CDC to update the Care Considerations to take into account that now that life expectancy has increased, more attention needs to be paid to clinical care that improves quality of life, including clinical best practices around cardiac care and the impact of progressive disability on the mental health of patients and their caregivers. Further, the Committee recommends that CDC consider including information about current approved therapies in the Care Considerations. The Committee looks forward to CDC’s report describing how the Muscular Dystrophy Program funding is allocated, including evaluation of the impact of the Care Considerations as well as differences in care and outcomes between Certified Duchenne Care Centers and non-certified centers with the MD-STARnet network. Finally, the Committee encourages the CDC to work with stakeholders to extract and evaluate the utility of common data elements in electronic health records (EHR) to improve care, understand disease outcomes, and model disease progression.

National Institutes of Health

Office of the Director

Duchenne and Becker Muscular Dystrophy – Developing gene therapies to treat Duchenne Muscular Dystrophy is a complex and multifaceted process. The Committee encourages the NIH to support research aimed at gene therapy safety utilizing viral vectors and support the development of less immunogenetic non viral delivery systems. The Committee also supports further NIH investment in the development of more sensitive outcome measures and biomarkers for both Duchenne and Becker.

National Heart, Lung, and Blood Institute

Duchenne and Becker Muscular Dystrophy – Now that life expectancy for Duchenne patients has increased, more attention needs to be paid to clinical care that improves how patients feel, function and survive. Furthermore, there is a paucity data on Becker patients in order to understand cardiac implications long term. NHLBI should work with the National Institute of Neurological Disorders and Stroke (NINDS) to establish a research network to follow patients throughout the lifespan to fully clinically characterize cardiac muscle function and better establish the relationship between cardiac muscle function and the impact of its progressive deterioration on both lifespan and quality of life.

Food and Drug Administration

Center for Drugs Evaluation and Research

Duchenne and Becker Muscular Dystrophy.— Some of the most promising therapies under development or approved are only viable for specific genetic mutations and impact a subset of the population. The Committee understands a treatment gap may be developing between Duchenne patients with a common mutation and those with less common mutations who may only be served by utilization of trial designs with very small sample sizes or n=1 trials. The Committee directs CDER, in coordination with the Center for Biologics Evaluation and Research (CBER), to conduct a stakeholder meeting to discuss enabling the development pathway of genetic therapies across the spectrum, particularly those that lack the patient population to incentivize drug developers.

Center for Biologics Evaluation and Research

Duchenne and Becker Muscular Dystrophy.— The Committee recognizes CBER and CDER’s efforts to collaborate across the agency and with stakeholders on community-led guidance for sponsors and FDA to incorporate the considerable, evolving body of scientific, clinical and regulatory information that has become available since the release of FDA’s February 2018 Guidance for developing drugs for treatment of Duchenne and related Dystrophinopathies. Given the progressive loss of function in multiple organ systems and ultimate loss of life caused by Duchenne muscular dystrophy, the Committee encourages the FDA to consider the appropriate use of the accelerated approval pathway to provide timely access to treatments that may materially improve how patients feel, function and survive.

Department of Defense (DOD)

Congressionally Directed Medical Research Program (CDMRP) Duchenne Muscular Dystrophy Research Funding: \$12M (level funding from FY22)

Sincerely,

DORIS MATSUI
Member of Congress

TROY BALDERSON
Member of Congress

SENATE

Senators Wicker and Stabenow will be leading the Duchenne Muscular Dystrophy FY23 Appropriations letter.

Dear colleague and language TBD.

With questions or to support, please reach out to Kirby Miller (Kirby_miller@wicker.senate.gov) or Sarah Jamgotch (Sarah_Jamgotch@stabenow.senate.gov)