Help End Duchenne Muscular Dystrophy
By Supporting Research, Patient Engagement, and Enhanced Disease Surveillance

Sign the FY22 Duchenne MD Appropriations Letter

Deadline to Sign: TBD

Dear Colleague –

Duchenne Muscular Dystrophy, one of 9 forms of muscular dystrophy, is the most common lethal genetic disorder diagnosed in childhood. 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. However, there is reason for hope, due in large part to the support Congress has provided for Duchenne:

• More than 35 potential therapies are in various stages of clinical testing.

• The life expectancy of the average 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 by supporting Duchenne research, public health, and therapy development initiatives. We invite you to help keep this momentum going by signing the FY22 Duchenne Muscular Dystrophy appropriations sign-on letter. This year, we are requesting language to:

• Increase funding for CDC’s Muscular Dystrophy Program from $6 million to $8 million.

• Increase funding for the Duchenne Muscular Dystrophy Research Program within DOD’s Congressionally Directed Medical Research Programs (CDMRP) from $10 million to $12 million.

• Evaluate the impact of the Care Considerations on patient outcomes, particularly in rural and underserved areas. Similarly, evaluate the effect of Certified Duchenne Care Centers on patient outcomes.

• Evaluate the availability of consistent and coordinated care for adults with Duchenne as they transition from pediatric care settings to adult care settings.
• Evaluate the impact of progressive disability on the mental health of patients and their caregivers.

• Establish an NIH framework for data-sharing across clinical studies and evaluate novel clinical trial designs at FDA.

• Support research at NIH on challenges related to gene therapies and ask FDA to update guidance on Duchenne treatments based on emerging gene therapies.

• Study the impacts of Duchenne on the brain and the heart. In light of improvements in care leading to patients living into their third decade, the leading cause of death in Duchenne Muscular Dystrophy patients is now heart failure.

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.

To sign or if you have any questions, please contact Sally Farrington (sally_farrington@wicker.senate.gov) with Senator Wicker or Alex Graf (alex_graf@stabenow.senate.gov) with Senator Stabenow.

Sincerely,

Roger F. Wicker
United States Senator

Debbie Stabenow
United States Senator
April XX, 2021

The Honorable Patty Murray  
Chair  
Labor, HHS, Education, & Related Agencies Subcommittee  
Room S-128, The Capitol  
Washington, DC 20510

The Honorable Roy Blunt  
Ranking Member  
Labor, HHS, Education, & Related Agencies Subcommittee  
Committee on Appropriations  
Room S-128, The Capitol  
Washington, DC 20510

The Honorable Tammy Baldwin  
Chair  
Agriculture, Rural Development, FDA, & Related Agencies Subcommittee  
Committee on Appropriations  
Room S-128, The Capitol  
Washington, DC 20510

The Honorable John Hoeven  
Ranking Member  
Agriculture, Rural Development, FDA, & Related Agencies Subcommittee  
Committee on Appropriations  
Room S-128, The Capitol  
Washington, DC 20510

The Honorable John Tester  
Chair  
Defense Subcommittee  
Committee on Appropriations  
Room S-128, The Capitol  
Washington, DC 20510

The Honorable Richard Shelby  
Ranking Member  
Defense Subcommittee  
Committee on Appropriations  
Room S-128, The Capitol  
Washington, DC 20510

Dear Chairmen Murray, Baldwin, and Tester and Ranking Members Blunt, Hoeven, and Shelby:

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 2022 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 2022 Duchenne MD appropriations request contains language and provisions to help continue and strengthen these and other ongoing initiatives. Specifically, the request would:

- Increase funding for Centers for Disease Control and Prevention (CDC)’s Muscular Dystrophy Program from $6 million to $8 million.

- Increase funding for the Duchenne Muscular Dystrophy Research Program (DMDRP) within the Department of Defense’s Congressionally Directed Medical Research Programs (CDMRP) from $10 million to $12 million.

- Direct the CDC to:
  - Evaluate the impact of the Care Considerations on patient outcomes, particularly in rural and underserved areas. Similarly, evaluate the effect of Certified Duchenne Care Centers on patient outcomes.
  - Evaluate the availability of consistent and coordinated care for adults with Duchenne as they transition from pediatric care settings to adult care settings.
  - Evaluate the impact of progressive disability on the mental health of patients and their caregivers.
  - Partner with stakeholder organizations to leverage additional knowledge and resources to advance this work.

- Urge the National Institutes of Health (NIH) to:
  - Establish a framework for data-sharing across clinical studies.
  - Support methodological research on challenges related to gene therapies.
  - Within the National Institute of Neurological Disorders and Stroke (NINDS), support research to study the impacts of Duchenne on the brain.
  - Within the National Heart, Lung, and Blood Institute (NHLBI), support research that characterizes cardiac disease in Duchenne patients. In light of improvements in care leading to patients living into their third decade, the leading cause of death in Duchenne Muscular Dystrophy patients is now heart failure.

- Urge the Food and Drug Administration (FDA) to:
  - Consider whether its 2018 guidance on developing therapies for Duchenne should be updated to reflect new developments in gene therapies.
  - Convene a multi-stakeholder meeting to evaluate novel clinical trial designs.
Much progress has been achieved in recent years, but much more work remains to be done. The FY 2022 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 (increase of $2M over FY21)

*Duchenne 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 the CDC to prepare a report describing how the Muscular Dystrophy Program funding is allocated for specific activities in Fiscal Years 2020 and 2021. The report also should, to the extent practicable, identify program priorities for Fiscal Years 2022 and 2023, including an evaluation of the impact of the Duchenne Muscular Dystrophy Care Considerations across the country on patient outcomes and any remaining gaps, particularly in rural and underserved areas. The Committee also expects the CDC to address the following three areas of unmet need; evaluating the differences in care and outcomes between Certified Duchenne Care Centers and non-CDCC MD-STARnet data and outcomes, the availability of consistent and coordinated care for adults with Duchenne as they transition from pediatric care settings to adult care settings, and the impact of progressive disability on the mental health of patients and their caregivers. The Committee encourages the CDC to partner with stakeholder organizations to leverage additional knowledge and resources to advance this work.

**National Institutes of Health**

*Office of the Director*

*Duchenne Muscular Dystrophy.*—Duchenne muscular dystrophy is a severe form of muscular dystrophy for which there is no cure and for which life expectancy is in the second or third decade. The Committee urges the NIH to establish a framework for data-sharing and sharing of specimens generated or collected within six months of completion of any NIH-funded clinical study. The Committee also urges NIH to support methodological research on challenges related to gene therapies, such as enabling delivery to individuals with neutralizing antibodies to viral vectors, manufacturing supply to ensure all patients can receive treatment, and minimizing potential life-threatening immune response to high viral doses.

*National Institute of Neurological Disorders and Stroke*

*Duchenne Muscular Dystrophy.*—While the expression of the dystrophin protein in the brain is recognized, our understanding of the link between the absence of dystrophin and related neurobehavioral/cognitive diagnosis is not well understood. The Committee urges NINDS to support research to characterize the role of dystrophin in the brain and to further define the relationships between mutation and neurobehavioral and cognitive diagnosis.
National Heart, Lung, and Blood Institute

Duchenne Muscular Dystrophy.—In light of improvements in care leading to patients living into their third decade, the leading cause of death in Duchenne Muscular Dystrophy patients is heart failure. The Committee urges NHLBI to support research that characterizes cardiomyopathy in Duchenne and Becker Muscular Dystrophy. There is a gap in the ability to develop novel cardiac therapeutics for Duchenne Muscular Dystrophy due to a lack of accepted cardiac outcome measures. The Committee encourages NHLBI to convene a workshop with research, clinical, and patient organization leaders to work towards establishing viable cardiac outcome measures for the development of therapeutic agents to combat cardiomyopathy.

Food and Drug Administration

Center for Biologics Evaluation and Research

Duchenne Muscular Dystrophy.—The Committee is aware of the 2018 Guidance on developing therapies for Duchenne Muscular Dystrophy and Related Dystrophinopathies. Given the potential that gene therapies may hold to treat these devastating diseases, the Committee urges FDA to consider whether the 2018 Guidance should be modified to reflect these developments. Any such effort should involve the relevant experts at both CBER and CDER.

Center for Drugs Evaluation and Research

Duchenne Muscular Dystrophy.—Several clinical trials for potential Duchenne Muscular Dystrophy therapies have failed due to a small effect over a one-year period. In order to increase study power and minimize time on placebo in trials longer than one year, the Committee urges CDER to convene a multi-stakeholder meeting to evaluate the use of external controls. The Committee encourages FDA to explore novel trial design to incorporate exploratory external control arms as a necessary first step to enabling their effective use.

Department of Defense (DOD)

Congressionally Directed Medical Research Program (CDMRP) Duchenne Muscular Dystrophy Research Funding: Increase from $10,000,000 to $12,000,000.

Sincerely,

Roger F. Wicker  Debbie Stabenow
United States Senator  United States Senator