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

Sign the FY21 Duchenne MD Appropriations Letter

Deadline to Sign: March 20, 2020

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 three 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 30 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 decade, driven in large part by the 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 FY21 Duchenne Muscular Dystrophy appropriations sign-on letter. This year, we are requesting language to:

- Ask Congress to increase funding for CDC’s Muscular Dystrophy Program to $8 million.

- Evaluate the impact of the Care Considerations on patient outcomes, particularly in rural and underserved areas.

- Evaluate the impact of the presence of a Certified Duchenne Care Center on patient outcomes.

- Assess diagnostic odyssey and provider resource needs before and after implementation of recommendations made by the National Task Force for Early Identification of Childhood Neuromuscular Disorders following the Mississippi Pilot of 2005-2008.

- Convene a multi-stakeholder workshop to evaluate pre-clinical models us to consider whether alternative models or strategies may improve therapy development outcomes.
• Encourage the National Institute of Neurological Disorders and Stroke (NINDS) to develop a centralized resource to store and access de-identified data for further research.

• Encourage the FDA to expand on the implementation of 21st Century Cures and to include in the benefit-risk framework a description of how patient experience data was considered.

• Support including the Duchenne Muscular Dystrophy Research Program (DMDRP) among the Department of Defense’s (DoD) Congressionally Directed Medical Research Programs in the Senate DoD appropriations bill, at a funding level of $10 million (equal to the FY20 enacted amount).

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 Kirby Miller (kirby_miller@wicker.senate.gov) with Senator Wicker or Amy Brown (amy_brown@stabenow.senate.gov) with Senator Stabenow.

Sincerely,

Roger F. Wicker
United States Senator

Debbie Stabenow
United States Senator
Dear Chairmen Blunt, Hoeven, and Shelby and Ranking Members Murray, Merkley, and Durbin:

Thanks in large part to the leadership of Congress, significant progress has been made in recent years in the fight to end Duchenne Muscular Dystrophy (DMD), the most common lethal genetic disorder diagnosed during childhood. We are writing to urge that, as you prepare your Fiscal Year 2021 Appropriations bill, you include provisions to help further these pursuits, particularly to advance scientific breakthroughs, to accelerate therapy development, and to help improve life for those currently affected by this disease.

In 2001 Congress enacted the Muscular Dystrophy Community Assistance, Research and Education (MD CARE) Act, which dramatically transformed efforts to combat Duchenne and other forms of Muscular Dystrophy. As a result of this 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 2021 Duchenne MD appropriations request contains language and provisions to help continue and strengthen these and other ongoing initiatives. Specifically, the request would:
• Ask Congress to increase funding for CDC’s Muscular Dystrophy Program to $8 million.

• Evaluate the impact of the Care Considerations on patient outcomes, particularly in rural and underserved areas.

• Evaluate the impact of the presence of a Certified Duchenne Care Center on patient outcomes.

• Assess diagnostic odyssey and provider resource needs before and after implementation of recommendations made by the National Task Force for Early Identification of Childhood Neuromuscular Disorders following the Mississippi Pilot of 2005-2008.

• Convene a multi-stakeholder workshop to evaluate pre-clinical models us to consider whether alternative models or strategies may improve therapy development outcomes.

• Encourage the National Institute of Neurological Disorders and Stroke (NINDS) to develop a centralized resource to store and access de-identified data for further research.

• Encourage the FDA expand on the implementation of 21st Century Cures to include in the benefit-risk framework a description of how patient experience data was considered.

• Support including the Duchenne Muscular Dystrophy Research Program (DMDRP), within the Department of Defense’s (DoD) Congressionally Directed Medical Research Programs, in the Senate DoD appropriations bill at a funding level of $10 million (equal to the FY20 enacted amount).

Much progress has been achieved in recent years, but much more work remains to be done. The FY 2021 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 FY20)

*Duchenne Muscular Dystrophy.* The Committee is aware of the development and dissemination of the Duchenne Muscular Dystrophy Care Considerations. In order to understand their impact, the Committee has provided resources for the CDC to prepare an evaluation of the Care Considerations. This study should consider how widely the Care Considerations have been adopted across the country and whether there has been an improvement in patient outcomes, particularly in rural and underserved areas. In addition, the Committee requests the CDC consider the possible relationship between patient outcomes and the presence of a Certified Duchenne Care Center (CDCC). Finally, the Committee requests the CDC conduct an assessment of diagnostic odyssey and provider resource needs before and after implementation of recommendations made by the National Task Force for Early Identification of Childhood Neuromuscular Disorders following the Mississippi Pilot of 2005-2008. This assessment
should also focus on underserved areas and include the relationship between outcomes and the presence of a CDCC.

**National Institutes of Health**  
*Office of the Director*

*Duchenne Muscular Dystrophy Research Models.* – In recent years, more Duchenne drug trials have failed than succeeded despite promising results from pre-clinical animal models. These results lead to years of inefficient drug development and few approved treatments. The Committee urges NIH to convene a multi-stakeholder workshop to evaluate pre-clinical animal models used frequently in Duchenne treatment research and to consider whether alternative models or strategies may improve therapy development outcomes.

**National Institute of Neurological Disorders and Stroke**

*Data Resource Optimization.* – The Committee is aware of the National Institute of Child Health and Human Development Data and Specimen Hub (DASH) project to create a centralized resource to store and access de-identified data from NICHD-supported studies. The Committee encourages NINDS to develop a similar system to leverage its research work product.

**Food and Drug Administration**

*Patient Experience in Drug Reviews.* — The Committee is aware FDA is implementing policies to encourage the collection and utilization of patient experience data, such as patient preferences and patient-reported outcomes, under the patient-focused drug development framework. The Committee encourages FDA expand on the implementation of Sec. 3001-3004 of P.L. 114-255 to include a description of how patient experience data was considered, if at all, in the benefit risk framework completion and review process.

Sincerely,

Roger F. Wicker  
United States Senator

Debbie Stabenow  
United States Senator