

Backgrounder on appropriations and Duchenne report language

Why we advocate in DC every year

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 law and subsequent amendments, federal commitments to research has expanded, spurring 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 to capture important data about the incidence and prevalence of Duchenne and to develop care standards for Duchenne which have been implemented globally. These care standards have helped markedly lengthen and improve the average lifespan of individuals with Duchenne.

In short, The MD-CARE Act provides a legislative framework for federal focus and investment in the muscular dystrophies including Duchenne, but it does not PROVIDE the funding needed to achieve the goals of the framework. Because of this, every year our community heads to Washington, DC to communicate to Congress the need for continued funding for the agencies that impact Duchenne. Along with the funding, we also request that Congress include Duchenne specific “report language” which include specific priorities and guidance for Duchenne related spending.

About ‘Appropriations’

Every year Congress passes a budget for funding the federal government. The budget is passed through annual “appropriations” bills because congress *appropriates* the money (your tax dollars) to programs and projects for all the federal agencies. The budget includes funding for federal agencies that impact Duchenne muscular dystrophy, all are under the Department of Health and Human Services (HHS). Every year we target these agencies in our request for Congress because they each fall within the MD-CARE Act framework and all impact Duchenne.

Agencies we target because they impact Duchenne:



Centers for Disease Control (CDC)

CDC conducts and supports health promotion, prevention and preparedness activities in the United States, with the goal of improving overall public health



National Institutes of Health (NIH)

NIH is the primary agency of the United States government responsible for biomedical and public health research.



Centers for Medicare/Medicaid (CMS)

CMS is the federal agency that administers the Medicare program and works in partnership with state governments to administer Medicaid.



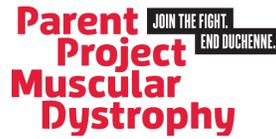
Food and Drug Administration (FDA)

FDA is responsible for protecting and promoting public health through the control and supervision of food and drugs.



Social Security Administration (SSA)

SSA administers social programs covering disability, retirement and survivors' benefits.



About 'Report Language'

Along with the budget, Congress writes a report that provides direction to the agencies on how to spend the money. With that, Duchenne advocates speak with their members of Congress about including specific "report language" in the report regarding the current priorities for the Duchenne community. This way, when the agencies receive their money they can refer to the report and see what the priorities are for any given disease. In our case, for Duchenne. This is why we are on the Hill every year! We need to ensure that the spending bills for the upcoming year (in this case, 2020) includes specific language in the 'funding legislation' directing the federal agencies to spend funds in a way that reflect the current needs of our Duchenne community.

How we get our report language included in appropriations bills

Thanks to PPMD's Congressional champions, letters (also known as "Dear Colleague Letters") in both the House and the Senate are crafted and sent to the Committees that oversee funding (Appropriations Committees). **This is where you come in!** While on the Hill on Tuesday, we will be asking members of Congress to **SIGN this letter supporting the request.**

So what are we asking Congress to do for the Fiscal Year 2020 bill (FY20)?

The request for Congress is very simple. ASK: Sign the FY 2020 Duchenne Muscular Dystrophy Appropriations Request letter (also known as the Duchenne "Dear Colleague" letters)

What is the specific language we are including in the request?

The request is the **SAME** for both the House and the Senate. This letter:

CDC	<p>Funding: Asks Congress to maintain <u>level funding of \$6 million</u> for CDC's Muscular Dystrophy Program.</p> <p>Updates on Activities: Requests updates from CDC on Duchenne newborn screening efforts, implementation of the new Duchenne ICD-10 code, and health economic activities (burden of disease and healthcare utilization)</p>
NIH	<p>Adaptive Trials: Requests NIH expand its support for research on Duchenne, particularly accelerating and improving the clinical trial process through new adaptive trial designs like platform studies which might serve as a model for other rare diseases communities.</p> <p>Gene Therapy: Urges NIH to support research on challenges with gene therapy, such as redosing, manufacturing supply, and potential immune responses of gene therapies.</p>
FDA	<p>Patient Experience Data: Supports the FDA's policy to make information available about how patient experience data is used within FDA reviews of newly approved products, encourages continued refinement of this process and requests a status on such efforts.</p> <p>Labeling: Requests information on FDA efforts to include patient-experience data in relevant product labeling and accompanying documentation to inform patient/provider decision-making and payer determinations.</p>

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SSA	SSA Programs: Requests that the Social Security Administration (SSA) make available data on the rate at which persons with Duchenne and Becker utilize SSA programs.
CMS	ICD-10 Code: Seeks a report from CMS (Medicare and Medicaid) on the use of the newly established ICD-10 code.