

**Congress of the United States**  
**Washington, DC 20515**

March 16, 2018

The Honorable Tom Cole  
Chairman  
Labor-HHS-Education Subcommittee  
Subcommittee  
Committee on Appropriations  
Washington, D.C. 20515

The Honorable Rosa DeLauro  
Ranking Member  
Labor-HHS-Education  
  
Committee on Appropriations  
Washington, D.C. 20515

The Honorable Robert Aderholt  
Chairman  
Agriculture, Rural Development, FDA  
FDA  
& Related Agencies Subcommittee  
Committee on Appropriations  
Washington, D.C. 20515

The Honorable Sanford Bishop  
Ranking Member  
Agriculture, Rural Development,  
& Related Agencies Subcommittee  
Committee on Appropriations  
Washington, D.C. 20515

Dear Chairmen Cole and Aderholt and Ranking Members DeLauro and Bishop:

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 (MD), the most common lethal genetic disorder diagnosed during childhood. We are writing to urge that as you prepare your Fiscal Year 2019 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 has 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. These care standards have helped markedly lengthen and improve the lifespan of the average Duchenne patient.

Our Fiscal Year 2019 Duchenne MD appropriations request contains language and provisions to help continue and strengthen these and other ongoing initiatives. Specifically, the request would:

- Maintain level funding for CDC's Muscular Dystrophy Program in order to support the advance of Duchenne newborn screening, continue supporting the dissemination of

Duchenne care standards, and to leverage the new ICD-10 code to expand surveillance of Duchenne and Becker muscular dystrophy.

- Request NIH to demonstrate its stated commitment to improved research data-sharing and to work with other agencies to create a plan to address development challenges of gene therapies.
- Encourage FDA to continue implementing policies to promote access to information about how patient experience data is used within reviews of newly approved products and asks FDA to consider ways to include patient experience information in relevant product labeling to inform patient and provider decisions and insurer coverage determinations.
- Request that the Social Security Administration (SSA) make available data on the rate at which persons with Duchenne and Becker utilize SSA programs.
- Seek a report from CMS on the use of the newly established ICD-10 code, as compared to the former broader code.

Much progress has been achieved in recent years, but much more work remains to be done. The FY 2019 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:

#### **National Institutes of Health (NIH)**

##### Office of the Director:

*Duchenne Muscular Dystrophy* – The Committee is aware that NIH-funded projects require a data-sharing plan to encourage transparency and leverage around the federal investments in this research. At the same time, project leaders and their institutions often cite barriers to implementing these plans in a timely and cost-effective manner. The Committee believes that a focused initiative to show commitment and to improve data-sharing performance in Duchenne research can create a model for the broader research community and recommends that the NIH develop this initiative in collaboration with patient, clinical, and research organizations.

##### National Center for Advancing Translations sciences:

*Duchenne Muscular Dystrophy* – The Committee recognizes the potential that virally mediated gene therapy applications pose for Duchenne and other rare diseases. As this science moves toward translational and clinical evaluation, the Committee recommends the NIH develop a comprehensive and integrated plan with the Food & Drug Administration and the Centers for Medicare and Medicaid, and other relevant entities, to address the complex, interrelated issues

such as manufacturing practices, dosing and redosing, and reimbursement models and related data requirements so that patients have timely access to these innovative therapies.

### **Food and Drug Administration (FDA)**

*Patient Experience* – The Committee is aware of FDA’s implementation of policies to promote public access to information about how patient experience information factored into the review of approved products. The Committee supports this step forward and encourages FDA to continue refining the instrument and ways to improve its visibility. The Committee also requests that FDA consider ways to include patient-experience information in relevant labeling and accompanying documentation to inform patient/provider decision making and payer determinations.

### **Centers for Disease Control and Prevention (CDC)**

#### National Center for Birth Defects and Developmental Disabilities (NCBDDD)

*Duchenne Muscular Dystrophy.*—The Committee recommends maintaining level funding of \$6,000,000 for the Muscular Dystrophy program within the National Center for Birth Defects and Developmental Disabilities. The Committee expects NCBDDD to continue its work to disseminate the revised Duchenne/Becker Muscular Dystrophy care standards, to expand surveillance of Duchenne/Becker via the MD STARnet, and support Duchenne newborn screening efforts. In addition, within the amount provided for the Muscular Dystrophy program, the Committee has provided sufficient funds to support a coordinated Duchenne newborn screening initiative through the National Center for Environmental Health’s Newborn Screening Quality Assurance Program. The Committee directs the CDC to develop a comprehensive plan to foster development and use of disease-specific validation test panels to expeditiously develop protocols that meet required standards, include capacity building support for state laboratory implementation, and support development of care standards and diagnostic algorithms for newborns with Duchenne.

*Duchenne Newborn Screening* – The Committee continues to be encouraged by efforts to develop a newborn screening test for Duchenne Muscular Dystrophy. The Committee is aware of the successful pilot project in Ohio and its support of existing plans to conduct an additional state pilot. The Committee seeks to advance Duchenne and other newborn screening efforts and directs the agency to develop a comprehensive plan to foster development and use of disease-specific validation test panels to expeditiously develop testing protocols that meet all required standards. The plan should also include capacity building support for state laboratory staff to be able to accurately interpret results and minimize false positives as well as a system to monitor performance of labs in this work. To support this effort, we recommend that the agency develop working groups involving all stakeholders, including the newborn screening community, to address these and future concerns.

*Duchenne Muscular Dystrophy* – The Committee is pleased by the publication of updates of the care standards for Duchenne Muscular Dystrophy and encourages the agency to continue supporting widespread dissemination of these standards to all appropriate provider audiences. The Committee is also aware of CDC’s efforts to develop an ICD-10 code for Duchenne and

requests that CDC develop a plan to leverage the recently established ICD-10 code to shift the Muscular Dystrophy Surveillance, Tracking and Research Network (MD STARnet) toward a more passive surveillance effort enabling an expansion of MD STARnet to additional sites/states.

**Social Security Administration (SSA)**

*Muscular Dystrophy* - The Committee is aware that the Social Security Administration is included in the Muscular Dystrophy Coordinating Committee under the Muscular Dystrophy CARE Act Amendments enacted in September 2014. The Committee expects the agency to make data available on the rate at which persons with Duchenne and Becker Muscular Dystrophy utilize SSA programs, particularly those focused on promoting employment and community independence such as the Ticket to Work Program.

**Centers for Medicare and Medicaid Services (CMS)**

*Muscular Dystrophy* – The Committee is aware of the addition of the new ICD-10 code for Duchenne/Becker to the FY 2019 CMS Addenda. The Committee requests a report on utilization for the newly established ICD-10 code, as compared to the former broader ICD-10 code.

Thank you for considering including the attached programmatic request and report language.

Sincerely,



PETER T. KING  
Member of Congress



DORIS MATSUI  
Member of Congress



LEE ZELDIN  
Member of Congress



GLENN "GT" THOMPSON  
Member of Congress



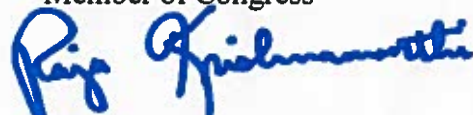
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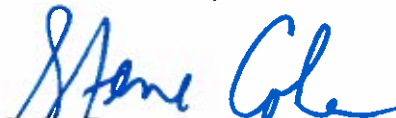
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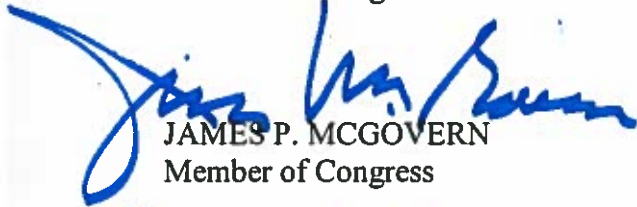




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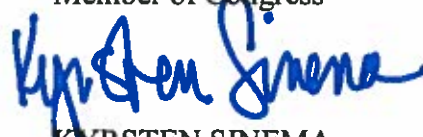
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