

February 8, 2013

The Honorable Amy Klobuchar
302 Hart Senate Office Building
Washington, D.C. 20510

The Honorable Michael C. Burgess, MD
2336 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Roger Wicker
555 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Eliot Engel
2161 Rayburn House Office Building
Washington, D.C. 20515

Dear Senator Klobuchar, Senator Wicker, Congressman Burgess, and Congressman Engel,

We, the undersigned organizations, applaud you for introducing legislation to reauthorize the Muscular Dystrophy Community Assistance, Research and Education Amendments (MD CARE Act), to build upon the tremendous success achieved in coordinating and focusing federal research on all nine forms of muscular dystrophy, developing epidemiologic data, and developing and disseminating patient care guidelines -- all of which have made a significant impact on the quality of life and life expectancy of children and adults diagnosed with muscular dystrophy.

Congress enacted the original MD CARE Act in 2001 and reauthorized the law in 2008. In particular, NIH's research through the Senator Paul Wellstone Muscular Dystrophy Cooperative Research Centers, have been responsible for catalyzing many of the scientific breakthroughs across the muscular dystrophies, have led to the expansion and intensification of MD research, including the leveraging of significant non-federal sources of funding. Since 2001, there have been 67 clinical trials of drugs or therapies for muscular dystrophy and there are currently 37 clinical trials underway. A number of the potential therapies now in clinical investigation can be traced to the basic research efforts sponsored by the Centers.

These reauthorization bills would build upon the success of this law and update the MD CARE Act to:

- Expand the eligible fields of research by the Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers to include cardiac and pulmonary function, and requires that the program support no fewer than 8 centers of excellence.
- Require that the MDCC meet no less than 2 times per year and also requires the Coordinating Committee to update the plan for conducting and supporting research and education on muscular dystrophy, including a new emphasis on studying and developing optimal clinical care interventions and justifying independent living resources support for adults with various forms of muscular dystrophy.

- Direct the Coordinating Committee to develop a plan for expedited approval of emerging therapies and personalized medicines with potential to treat patients with muscular dystrophy.
- Give the Coordinating Committee authority to evaluate the potential need to enhance the clinical research infrastructure required to test emerging therapies, including expanding the use of regulatory science, cooperation and communication across federal agencies with a goal of facilitating the development and approval of therapies for the various forms of muscular dystrophy.
- Direct the CDC to update and widely disseminate existing Duchenne-Becker muscular dystrophy care considerations for pediatric patients as well as develop and widely disseminate care considerations for adults with the disease.
- Direct the CDC to develop and disseminate acute care considerations for all muscular dystrophy populations.

We thank you for your leadership on this issue, and we look forward to working with you to support this important legislation in the 113th Congress.

Sincerely,

Charley's Fund
Coalition Duchenne
Cure CMD
CureDuchenne
Defeat Duchenne, Inc.
Duchenne San Diego
Facioscapulohumeral Muscular Dystrophy Society (FSH Society)
Foundation to Eradicate Duchenne (FED)
Friends of FSH Research
Hope for Javier
Jain Foundation
JB's Keys to DMD
John Owen's Adventure, Inc
Liam Hiatt Foundation
Muscular Dystrophy Association (MDA)
Parent Project Muscular Dystrophy (PPMD)
Rally for Ryan, Inc.
Save Our Boy Foundation
Team Joseph
Two Smiles One Hope Foundation
Zack Heger Foundation