



The Honorable Michael C. Burgess, M.D.  
U.S. House of Representatives  
2336 Rayburn House Office Building  
Washington, DC 20515

The Honorable Eliot L. Engel  
U.S. House of Representatives  
2161 Rayburn House Office Building  
Washington, DC 20515

Dear Representatives Burgess and Engel:

On behalf of the thousands of Americans with Congenital Muscular Dystrophy (CMD), we strongly support The Paul D. Wellstone Muscular Dystrophy Community Assistance, Research and Education (MD CARE) Amendments Act of 2013 (H.R. 594).

Cure CMD is a nonprofit organization dedicated to finding treatments and, eventually, a cure for the devastating ravages of the congenital muscular dystrophies. CMD is a group of diseases causing muscle weakness at birth or within the first two years of childhood. Several defined genetic mutations cause muscles to break down faster than they can repair or grow. A child with CMD may have various neurological or physical impairments and may never gain the ability to walk. Cure CMD represents the network of family, friends, caretakers and affected individuals tirelessly battling this devastating disease every day.

CMD is a progressive disease without treatment. The future is bleak. It is a life that ends after years of non-stop caregiving, dependency, hospitalizations and loss...loss of ambulation, loss of integration within society, loss of ability to sign and communicate, loss of an ability to breathe on one's own and loss of life.

Cure CMD is deeply appreciative of the critical support the MD CARE Act has provided to muscular dystrophy research and the steadfast pathway toward clinical trials. The pulmonary and cardiac research supported is making significant advances in better understanding and treating all muscular dystrophies including CMD. Without this vital research, the CMD community will be setback years in making progress and improving the quality of life of those suffering with this devastating disorder. It is critical to our communities that the strong momentum of scientific progress is able to be maintained.

Sincerely,

Patrick May, Chairman, Cure CMD  
[pat.may@curecmd.org](mailto:pat.may@curecmd.org)



The Honorable Fred Upton  
Chairman  
Committee on Energy and Commerce  
United States House of Representatives  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Joe Pitts  
Chairman  
Energy and Commerce Health Subcommittee  
U.S. House of Representatives  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Henry A. Waxman  
Ranking Member  
Committee on Energy and Commerce  
United States House of Representatives  
2322A Rayburn House Office Building  
Washington, DC 20515

The Honorable Frank Pallone, Jr.  
Ranking Member  
Energy and Commerce Health Subcommittee  
U.S. House of Representatives  
2322A Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton, Ranking Member Waxman, Chairman Pitts, and Ranking Member Pallone:

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June 12, 2014

The Honorable Michael C. Burgess, M.D.  
U.S. House of Representatives  
2336 Rayburn House Office Building  
Washington, DC 20515

The Honorable Eliot L. Engel  
U.S. House of Representatives  
2161 Rayburn House Office Building  
Washington, DC 20515

Dear Representatives Burgess and Engel:

On behalf of the Muscular Dystrophy Association, a voluntary health organization which funds services, treatments, and research for cures for the estimated 150,000 people living in the United States with one of the nine forms of muscular dystrophy, thank you for sponsoring H.R. 594, legislation to update the Muscular Dystrophy Community Assistance, Research, and Education Amendments (MD-CARE Act).

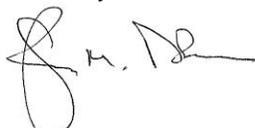
The MD-CARE Act, originally enacted by Congress in 2001 and updated in 2008, has yielded great advances in understanding the specific causes of the various forms of muscular dystrophies, the mechanisms of these diseases, identification of therapeutic targets, and now even clinical trial development. The Paul D. Wellstone Centers of Muscular Dystrophy Excellence are a model of how best to incentivize therapy development and ensure that research is not conducted in silos. In 2001, not 1 clinical drug trial had been conducted for muscular dystrophy. Today there are more than 67 clinical trials of drugs or therapies for muscular dystrophy, and currently more than 40 clinical trials are under way.

The MD-CARE Act has also served to standardize and optimize clinical care throughout the United States by developing care standards for each form of muscular dystrophy and then disseminating them to clinicians and patients throughout the United States. The inter-agency Muscular Dystrophy Coordinating Committee developed the MD Action Plan in 2005 and has worked to eliminate duplication and maximize opportunities for collaboration with government and private partners.

Thanks to the MD-CARE Act, we have made a significant impact on the quality of life and life expectancy of children and adults diagnosed with muscular dystrophy. However, the muscular dystrophies continue to be among the nation's most devastating health conditions, and most forms of muscular dystrophy are still considered to be 100% fatal.

Accordingly, we commend you for leadership and continued focus on providing updates to the current law, both to ensure that we build upon its successes as well as to ensure that the law continues to keep pace with the advances and investment that have been made to date. Muscular dystrophy affects Americans of all ages, ethnicities, and economic strata. Today – on behalf of each of these babies, children, teens, adults, and their families – thank you for continuing your important work on this legislation.

Sincerely,



Steven M. Derks  
President & CEO

[sderks@mdausa.org](mailto:sderks@mdausa.org)

office / 312-260-5901  
222 South Riverside Plaza  
Suite 1500  
Chicago, IL 60606-6000

June 12, 2014

The Honorable Fred Upton  
Chairman  
Committee on Energy and Commerce  
United States House of Representatives  
2125 Rayburn House Office Building  
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The Honorable Henry A. Waxman  
Ranking Member  
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Energy and Commerce Health Subcommittee  
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2322A Rayburn House Office Building  
Washington, DC 20515

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On behalf of the Muscular Dystrophy Association, a voluntary health organization which funds services, treatments, and research for cures for the estimated 150,000 people living in the United States with one of the nine forms of muscular dystrophy, we urge you to mark-up and approve H.R. 594, legislation to update the Muscular Dystrophy Community Assistance, Research, and Education Amendments (MD-CARE Act).

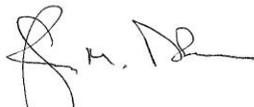
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Accordingly, we urge your approval of H.R. 594, which will build upon its successes of the MD-CARE Act as well as ensure that the law keeps continues to keep pace with the advances and investment that have been made to date. Muscular dystrophy affects Americans of all ages, ethnicities, and economic strata. Today – on behalf of each of these babies, children, teens, adults, and their families – thank you for your important work on this legislation.

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office / 312-260-5901  
222 South Riverside Plaza  
Suite 1500  
Chicago, IL 60606-6000

June 12, 2014

The Honorable Michael Burgess  
Vice Chairman  
Subcommittee on Health  
Committee on Energy and Commerce  
2336 Rayburn House Office Building  
Washington, DC 20515

The Honorable Eliot Engel  
Subcommittee on Health  
Committee on Energy and Commerce  
2161 Rayburn HOB  
Washington, DC 20515

Dear Vice Chairman Burgess and Representative Engel:

Parent Project Muscular Dystrophy, the leading advocacy organization striving to end Duchenne Muscular Dystrophy and to improve the quality of life for those with the disease and their families, urges you to advance H.R. 594 to help ensure continued progress against all forms of muscular dystrophy. First enacted in 2001 and updated in 2008, the Muscular Dystrophy Community Assistance, Research & Education Act – or MD CARE Act – is a shining success story. Since its enactment this law has leveraged limited federal resources to catalyze efforts that have:

- ✓ Increased by about 10 years over the same period of time the average lifespan of patients with the most common form of the disease;
- ✓ Dramatically improved and standardized clinical care helping drive improved health outcomes; and
- ✓ Transformed a barren potential therapeutics landscape into one that today counts 32 potential therapies in various stages of clinical investigation.

Had Congress not taken the bold step of enacting the initial MD CARE Act in 2001 and 2008 update, much of this progress likely would not have occurred. The MD CARE Act epitomizes what is possible when Congress commits itself to advancing sound, evidence-based biomedical research and public health policy.

While much progress has occurred, more work remains. To maximize the federal commitment made over the years and to achieve the end goal of safe and effective treatments and therapies for all forms of muscular dystrophy, **we urge you to support the MD CARE Amendments Act of 2013.**

This legislation recognizes the challenging fiscal climate by providing no new authorization of appropriations and proposes a small set of targeted improvements intended to ensure the law remains as effective as possible by focusing on the most critical areas. For example, the amendments would:

- ✓ Enhance existing research efforts to include a focus on cardiac, pulmonary, and other issues of importance to adults with muscular dystrophy;
- ✓ Update existing care standards and filling gaps that remain, particularly in properly caring for adults with Duchenne and other forms of muscular dystrophy;
- ✓ Intensify surveillance and tracking of all the muscular dystrophies including capturing more diverse populations; and
- ✓ Support adults with Duchenne so they can live independent, productive and rewarding lives.

The Muscular Dystrophy CARE Amendments Act of 2013 will enable us to continue achieving the research and clinical care breakthroughs that have transformed lives for all Americans impacted by these conditions. We urge you to join us in recognizing the success of this work by supporting the MD CARE Amendments Act today.

Sincerely,

A handwritten signature in black ink, appearing to read "Pat Furlong". The signature is fluid and cursive, with a large initial "P" and a long, sweeping tail.

Pat Furlong  
President/CEO  
Parent Project Muscular Dystrophy

June 12, 2014

The Honorable Fred Upton  
Chairman  
Committee of Energy and Commerce  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Joe Pitts  
Chairman  
Subcommittee on Health  
Committee on Energy and Commerce  
420 Cannon House Office Building  
Washington, DC 20515

Dear Chairman Upton and Chairman Pitts:

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**Parent Project  
Muscular Dystrophy**

LEADING THE FIGHT TO END DUCHENNE

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President/CEO  
Parent Project Muscular Dystrophy

June 12, 2014

The Honorable Henry Waxman  
Ranking Member  
Committee of Energy and Commerce  
2322A Rayburn House Office Building  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
Subcommittee on Health  
Committee on Energy and Commerce  
237 Cannon House Office Building  
Washington, DC 20515

Dear Ranking Member Waxman and Ranking Member Pallone:

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Pat Furlong  
President/CEO  
Parent Project Muscular Dystrophy

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 update 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 amendments 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 robust research momentum through the 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 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 113<sup>th</sup> 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



**MYOTONIC  
DYSTROPHY**  
FOUNDATION

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London, England

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Richmond, VA

**Richard Weston**

Encinitas, CA

SCIENTIFIC ADVISORS

**Tetsuo Ashizawa, MD**

University of Florida

**John W. Day, MD, PhD**

Stanford University

**Richard W. Lymn, PhD**

NIH, Retired

**Darren Monckton, PhD**

University of Glasgow

**Richard Moxley III, MD**

University of Rochester

**Charles Thornton, MD**

University of Rochester

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The Honorable Eliot L. Engel  
US House of Representatives  
2161 Rayburn House Office Building  
Washington, DC 20515

June 13, 2014

Dear Representatives Burgess and Engel:

On behalf of the myotonic dystrophy community, the Myotonic Dystrophy Foundation urges you to support the Paul D. Wellstone Muscular Dystrophy Community Assistance Research and Education (MD-CARE) Amendments (H.R. 594).

Since its initial passage in 2001, the MD-CARE Act has transformed life for Americans impacted by many different forms of muscular dystrophy. The proposed expansion focuses on cardiac and pulmonary research, and supporting adult and transitioning populations. These updates are crucial for those living with myotonic dystrophy (DM), which is the most common form of adult-onset muscular dystrophy. DM is the only form that has impacts on cognition and brain function, in addition to impacts on the heart, lungs, muscles, gastrointestinal system, and many other body systems. Because DM is multi-systemic and variable, symptoms can vary widely and it is significantly under-diagnosed. The congenital and juvenile-onset forms of this disease are particularly devastating. As a genetic disease, it impacts entire families and becomes more severe with each successive generation.

Despite advances made in muscular dystrophy research, total funding for DM research – both academic- and industry-based – still lags behind. Because there are currently no treatments or a cure for DM, there is a real need to continue investment in this disease arena. DM is considered a breakthrough disease in terms of its ability to advance science around triplet repeat, toxic RNA diseases, and other neuromuscular disorders. Researchers are close to finding the answers to restore cell function in the body systems that are attacked by DM – in fact, scientists have already reversed the condition in laboratory mice, and the first human clinical trials launched earlier this month. There is an enormous research opportunity that extends far beyond this disease alone, which is why we must ensure that the MD-CARE Act continues to reflect the pace of scientific progress.

Few people ever have the opportunity in their lifetime to help cure or reverse a form of muscular dystrophy, but you are in a unique position to help advance DM research and influence the development of treatments for this disease. We hope we can count on your support.

Sincerely,

Molly White  
Executive Director



**MYOTONIC  
DYSTROPHY**  
FOUNDATION

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June 13, 2014

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Molly White  
Executive Director