MYOTONIC DYSTROPHY FOUNDATION

The Honorable Michael C. Burgess, MD
US House of Representatives
2336 Rayburn House Office Building
Washington, DC 20515

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

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