The Muscular Dystrophy Community Assistance, Research & Education Act

History & Impact

Few federal laws have achieved the same level of impact as the Muscular Dystrophy Community Assistance, Research and Education (MD CARE) Act. Originally enacted in late 2001 – just 11 years ago – this law has driven national and international efforts that have: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 these improved health outcomes; and

- Transformed a barren potential therapeutics landscape into one that today counts nearly 20 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 its subsequent reauthorization in 2008, much of this progress likely would not have occurred. As a result:

- The average lifespan of patients with Duchenne – the most common form of muscular dystrophy – would be the late teens or early 20s;

- Extreme variations in care standards and care quality, including inaccurate and late diagnoses in many areas of the nation, would be the norm; and

- Treatments and potential treatments would be limited largely to steroids and their myriad side effects.

The MD CARE Act epitomizes what is possible when Congress commits itself to advancing sound, evidence-based biomedical research and public health policy. Discoveries are accelerated, clinical care improves and – ultimately – patients live longer, healthier and more productive lives. Today patients with Duchenne and other forms of Muscular Dystrophy attend college and graduate school, hold jobs and live lives that were beyond imagination just a decade ago. While much progress has occurred, more work remains ahead. To maximize the sizeable 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, Congress must reauthorize the MD CARE Act.

A True Public Private Partnership Leveraging Billions of Dollars in Non-Federal Funding

Before the MD CARE Act was enacted, NIH invested less than $3 million total in Duchene research. Today, NIH supports about $32 million of Duchenne research annually, and since the MD CARE Act was enacted in 2001, more than $200 million in federal resources have been committed to Duchenne research and related activities. Driven by the Senator Paul D. Wellstone Muscular

Dystrophy Cooperative Research Centers, selected from rigorous peer-review competitions, this research has yielded multiple scientific breakthroughs that have driven improvements in clinical care and sparked the discovery of many of the potential therapies currently under investigation.

In terms of real dollars, the federal commitment from the MD CARE Act has spurred a non-federal investment of billions of dollars. Over the past 12 years, billions of dollars have been invested by academic research organizations, biopharmaceutical companies, venture capital and philanthropic organizations into research and drug discovery and development efforts focused on Duchenne alone. This non-federal financial support has expanded research programs and accelerated efforts to rapidly move laboratory breakthroughs into potential therapies, ultimately moving us closer to the quest for safe and effective treatments.

MD CARE Act Reauthorization – Continuing the Progress

The MD CARE Act is a shining legislative success, exemplifying what can be achieved through genuine public-private partnerships to transform the biomedical research and drug discovery landscape. But while much has been accomplished, more remains undone and in need of federal support to continue driving and leveraging non-federal funding. Some of the major challenges today include:

- Expanding and sustaining research efforts across the muscular dystrophies including an expanded focus into cardiac and pulmonary functioning and into the health care needs of adults with muscular dystrophies;

- Updating existing Duchenne-Becker care standards, developing for the first time care standards for adults living with Duchenne and developing and disseminating care standards for those with other forms of muscular dystrophy;

- Intensifying surveillance of tracking of all the muscular dystrophies and ensuring that this valuable data informs the biomedical research agenda; and

- Ensuring that when potential therapies are submitted for evaluation they are reviewed as quickly as possible.

The Muscular Dystrophy CARE Amendments Act of 2013 will address these issues to ensure the law continues driving toward improved outcomes for all Americans impacted by the muscular dystrophies and to achieving the scientific discoveries and breakthroughs necessary to develop treatments and therapies to increase patient lifespan and to enhance quality of life. By enacting the reauthorization bill, Congress will ensure this landmark law retains its focus on the most pressing issues, drives greater levels of coordination and collaboration, and encourages continued public-private partnership.