Testimony of Pat Furlong
Founding President & CEO
Parent Project Muscular Dystrophy

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Subcommittee on Health
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Good morning Chairman Pitts, Chairman Upton, Ranking Member Pallone, Ranking Member Waxman and Members of the Committee.

My name is Pat Furlong. Twenty years ago, I joined other parents to form Parent Project Muscular Dystrophy to end Duchenne, one of nine forms of muscular dystrophy and the most common, lethal genetic disorder diagnosed in childhood. Ten years earlier, in 1984, I received the horrific diagnosis that my two sons, Christopher and Patrick, had this disease. Though both my sons are gone, I wage this fight in their honor.

Today I am going to briefly describe a journey spanning two decades that shows what is possible when all sectors – public and private – come together to pursue breakthroughs in research and to advance into innovative treatments.

Before I begin, I want to commend Chairman Upton, Congresswoman DeGette, and the Committee for undertaking the 21st Century Cures Initiative. This committee has a long history of leadership on research, drug development and public health issues, and the Cures Initiative is much-needed.

Nearly 15 years ago, this Committee shepherded through Congress the Children's Health Act, which created or strengthened multiple programs focused on childhood disease and disability. This law contained a small but important provision that directed the National Institutes of Health to expand research into all forms of muscular dystrophy, including Duchenne. At the time of this enactment, the NIH commitment to muscular dystrophy overall was less than $10 million annually, and Duchenne research was almost non-existent.

The following year, recognizing that an even more comprehensive effort was needed, Congress enacted the Muscular Dystrophy Community Assistance, Research and Education or MD CARE Act, a law that also was marked up by this committee.

The MD CARE Act and its 2008 amendments have transformed research and clinical care for Duchenne and the many other forms of muscular dystrophy. And today, a modest, but essential, update to this law is now moving through Congress. I thank the members of the Health Subcommittee for marking up this legislation late last month, and we look forward to forthcoming action by the full committee.

Focused on all forms of muscular dystrophy, the MD CARE Act was in my view the single biggest advancement for the Duchenne community since Lou Kunkel and his team identified the gene that causes Duchenne back in 1986.

To give you a sense as to how far we have come since the law's enactment in 2001, consider that, at that time, most boys lived only into their late teens. Today, the average life span for some young men – while still far too short – is about 10 years longer.

Also, in 2001, the Duchenne therapeutic pipeline was barren with very few academic researchers and no industry players – big or small – involved in the field. Today, we have about a dozen Duchenne therapies in clinical testing, and many more candidates in earlier stages of development.

Despite the many scientific breakthroughs driven by the MD CARE Act, Duchenne remains a fatal disease without any disease-modifying therapies or approved treatments. Most boys end up in wheelchairs by their mid-teens and face a steady decline of muscle function as they age.

Our community needs therapies, and we need them fast.

To accelerate the development and delivery of effective therapies to patients, PPMD has led two projects over the past year to address major gaps in the process:

- The first-ever scientific survey of our community on benefit and risk preferences; and

- The first-ever, patient-initiated drafting of a guidance document for industry sponsors for consideration by FDA as a formal adopted process.

Because Duchenne is 100 percent fatal at a young age, many patients and families have long said they would be willing to accept higher levels of potential risk in return for the prospect of certain benefits. Last year, PPMD partnered with Johns Hopkins University to conduct the first-ever scientific survey of about 120 parents of children with Duchenne.

This survey validated our community's higher risk threshold, even when balanced against a non-curative treatment, such as prolonging the ability to walk, and absent an increase in lifespan. This data was shared with the FDA and was recently published in an academic journal. Now, FDA must ensure its reviewers apply this perspective to their decision-making process.

Another significant impediment to drug development, particularly for those working in rare disease, is the absence of clear guidance from FDA when it comes to designing clinical trials. Small patient populations, limited knowledge about the conditions and a lack of accepted or validated biomarkers that indicate a potential treatment is working are some of these challenges.

At the recommendation of the FDA, PPMD led a comprehensive, six-month effort to convene key stakeholders – patients, parents, clinicians, researchers and industry – to develop a draft guidance document that would address trial design and other issues. This document was submitted to FDA last month, marking the first time a patient group has led the development of such a product.

Now, FDA must step up to promptly review the draft, gather stakeholder input and issue a guidance document under the agency's name. A docket has been opened, a good first step, but we must see more activity this summer and fall.

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So what can Congress and FDA do moving ahead?

Beyond holding a stakeholder meeting and issuing a draft guidance document, FDA must make sure its reviewers recognize the benefit/risk perspectives of our community. The message from the survey is quite clear – given the alternative of death at a young age, our community is willing to accept a higher degree of risk.

One way to ensure that the patient perspective impacts the review process would be to establish a non-burdensome step wherein reviewers disclose how they did – or did not – take such information into account in making their decisions on a candidate therapy. This would shed light on what is for many a black-box process and could be done in a non-burdensome manner.

Beyond such transparency, I would urge an even greater focus on regulatory science so that the FDA keeps pace with the breakneck pace of industry drug development. In addition to bolstering support for regulatory science research, NIH must infuse a regulatory science component into all translational awards. By incorporating regulatory science considerations earlier in the pipeline, we can maximize the likelihood that candidate therapies will be ready for the rigor of the FDA process.

Finally, I would encourage greater flexibility in clinical trials, particularly for fatal rare diseases like Duchenne that have smaller populations. Business-as-usual trial design simply will not hit the mark when working with such populations. Specifically, we should be open to post-hoc or post-trial analysis to continue gathering important information on benefits and risks without making for unnecessarily lengthy and burdensome trials.

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The Duchenne community has traveled a great distance over the past 15 years, thanks in significant part to the leadership of this Committee. But for too many families, my own included, this journey has not been fast enough, and we think every day about those whom we have lost.

We need treatments and therapies, right now, so we can end Duchenne and address the thousands of other rare diseases in need of treatments and cures.

I applaud the Committee for focusing on this important issue, and look forward to contributing to this work going forward.