

# LEADING THE FIGHT TO END DUCHENNE

May 8, 2015

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

The Honorable Diana DeGette  
Ranking Member  
Subcommittee on Oversight & Investigations  
Committee on Energy & Commerce  
2322A Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton, Representative DeGette and Members of the Committee:

On behalf of Parent Project Muscular Dystrophy (PPMD) and the Duchenne muscular dystrophy community, I want express our continued support of the 21st Century Cures legislation. This comprehensive, thoughtful and bipartisan initiative to examine and update our national research and development infrastructure comes at such an important time to our community as life-altering new therapies for Duchenne are now closer than ever before.

For more than 20 years, Parent Project Muscular Dystrophy (PPMD) has led the fight to end Duchenne muscular dystrophy. Duchenne is the most common fatal genetic disorder diagnosed in childhood, affecting about 1 in every 4000 live male births, with about 20,000 new cases each year. As an X-linked disease, Duchenne primarily impacts boys and results in the absence of the dystrophin protein. This absence causes muscles to weaken and deteriorate. As people with Duchenne age, muscle atrophy leaves them unable to walk, to move their arms and, ultimately, to breathe, maintain heart function, and live.

Not one single disease-modifying therapy has yet been approved to treat Duchenne in the U.S. However, thanks to a decade of more robust and strategic public and private sector support for Duchenne drug discovery and development, nearly a dozen candidate therapies are in various stages of clinical evaluation today. And in just the last four months, two rolling New Drug Applications (NDAs) for Duchenne candidate therapies have been submitted to the Food and Drug Administration (FDA), with others expected over the coming months.

## Putting Patients First

PPMD remains strongly supportive of the provisions in the draft legislation on patient-focused drug development (PFDD) that seek to strengthen the patient's role in the medical product development process. However, we remain concerned that this section continues to lack a means of capturing and publicly reporting how the FDA is – or is not – using PFDD tools and authorities provided to the agency. While PPMD has been pleased to lead the Duchenne community in developing draft guidance to industry on Duchenne drug development, we want and need to see some level of assurance that such tools – which take tremendous amounts of time and resources to produce – will play a role in the FDA review process. To achieve this goal, we urge you to amend the **April 29<sup>th</sup> discussion draft with a provision we have entitled the Patient-Focused Impact Assessment or PFIA.**

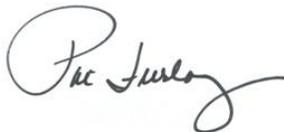
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At its core, the PFIA calls for creating a brief checklist that the FDA reviewers would complete at the time of review to say what PFDD authorities or tools they did – or did not – use in considering an application. Such a provision would create an essential feedback loop, helping shine some much-needed light on what is largely an opaque process. It also would help inform efforts by patient advocacy organizations and other stakeholders to develop PFDD tools with the necessary rigor for use by the agency.

PFIA topics could include benefit/risk data for the indicated populations, draft or final guidances, patient-preference data, patient-reported outcomes data, and the views of patients and other external experts on the application. The draft PFIA provision would require the FDA to compile an annual report summarizing the agency's use of PFDD tools and authorities within applications reviewed during the preceding year. We believe this piece is an important step in ensuring the FDA is utilizing patient focused drug development tools and urge the committee to include such a provision in the final legislation. Furthermore, more than 20 patient advocacy and related stakeholders including National Organization for Rare Disorders (NORD), The Genetic Alliance and the Everylife Foundation for Rare Diseases have endorsed this concept, demonstrating its application far beyond the Duchenne community. The PFIA endorsement letter from earlier this year and the latest draft legislative provision, informed by stakeholder comments, are attached for reference.

Thank you again for undertaking this important initiative and considering the Patient Focused Impact Assessment.

Sincerely,



Pat Furlong  
Founding President  
Parent Project Muscular Dystrophy