

LEADING THE FIGHT TO END DUCHENNE

June 9, 2015

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

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

Dear Chairman Upton, Ranking Member Pallone, Chairman Pitts, Ranking Member Green and Rep. DeGette:

On behalf of Parent Project Muscular Dystrophy (PPMD), the leading organization fighting to end Duchenne muscular dystrophy, I am writing to enthusiastically endorse the **H.R. 6: The 21st Century Cures Act**. Over the past year-plus, you and your colleagues have undertaken an impressive process to develop and refine a piece of legislation that, if enacted into law, will supercharge our national commitment to biomedical research and will accelerate the process by which we deliver safe and effective therapies to patients in need, particularly those with profound unmet medical need.

PPMD has been privileged to have been an active participant in this process including serving as a witness before a hearing last summer and by participating in numerous regional events. We have also engaged one-on-one with many offices and committee staff to inform and refine multiple provisions of this legislation. We are particularly pleased that this legislation, informed by PPMD's own experiences in leading the development of patient-focused drug development tools, continues to build on provisions included in the FDA Safety and Innovation Act to further strengthen the voice of the patient and caregiver in the therapy developments process. We believe that meaningful patient engagement is the blockbuster innovation of the 21st Century. We appreciate that H.R. 6 seeks to provide additional clarity and refinement to the PFDD development process, something all stakeholders, particularly patient advocacy organizations, need to move forward with confidence to further develop such tools. And we applaud the committee for looking to the future by directing the FDA to issue guidances and other regulations to support review of precision medicines, to allow for greater use of novel trial designs and for recognizing the immense value possible through patient experience data.

While we appreciate this support for PFDD, we continue to believe that the legislation would be further strengthened by including a simple and transparent assessment instrument to better understand how such tools are – or are not – being used by FDA reviewers. Such transparency would provide valuable insights that PFDD developers can use to develop products, would send a message to PFDD developers that the agency is paying attention to how such tools are or are not being used and would help drive overall innovation in therapy development. As the legislative process continues, we encourage the committee to be open to incorporating such an assessment tool.

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Thank you, again, for your tremendous leadership on 21st Century Cures and for your commitment to all Americans seeking therapies, treatments means of prevention and cures.

Sincerely,



Pat Furlong
Founding President

CC:

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

The Honorable Gene Green
Ranking Member
Subcommittee on Health
Energy and Commerce Committee
2470 Rayburn House Office Building
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The Honorable Diana DeGette
Ranking Member
Subcommittee on Oversight & Investigations
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2322A Rayburn House Office Building
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