LEADING THE
FIGHT TO
END DUCHENNE

November 28, 2016

The Honorable Lamar Alexander
Chairman
Committee on Health, Education, Labor & Pensions
828 Senate Hart Office Building
Washington, DC 20510

The Honorable Patty Murray
Ranking Member
Committee on Health, Education, Labor & Pensions
428 Senate Dirksen Office Building
Washington, DC 20510

Dear Chairman Alexander and Ranking Member Murray:

On behalf of Parent Project Muscular Dystrophy (PPMD), the leading organization fighting to end Duchenne muscular dystrophy, I am writing to enthusiastically endorse **The 21st Century Cures Act.** For nearly two years, 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. 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 the 21st Century Cures Act 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 including a simple and transparent assessment instrument to better understand how such tools are – or are not – being used by FDA reviewers. Such transparency will provide valuable insights that PFDD developers can use to develop products, will send a message to PFDD developers that the agency is paying attention to how such tools are or are not being used and will help drive overall innovation in therapy development.

As our Duchenne landscape has shifted quite considerably over these past two years, many of the provisions contained within this landmark bill have taken on even greater relevance and urgency to our community. We have seen first-hand how critical the PRV extension, data-sharing incentives, industry expanded access disclosure policies, incentives for pediatric rare disease research, and explicit regulatory authority for advancement of targeted therapeutic innovations are. Each of these provisions represents the removal of a barrier to life-saving research for children in the U.S. living with Duchenne.

Parent Project
Muscular Dystrophy

401 Hackensack Avenue, 9th Floor, Hackensack, New Jersey 07601
t 800.714.5437 • 201.250-8440 **e** info@ParentProjectMD.org • ParentProjectMD.org

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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 Roger Wicker
555 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Amy Klobuchar
302 Hart Senate Office Building
Washington, DC 20510

The Honorable Johnny Isakson
131 Russell Senate Office Building
Washington, DC 20510

The Honorable Al Franken
309 Hart Senate Office Building
Washington, DC 20510

The Honorable Michael Bennet
261 Russell Senate Office Building
Washington, DC 20510

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