

# LEADING THE FIGHT TO END DUCHENNE

December 7, 2016

The Honorable Roger Wicker  
555 Dirksen Senate Office Building  
Washington, DC 20510

Dear Senator Wicker,

On behalf of Parent Project Muscular Dystrophy (PPMD), the leading organization fighting to end Duchenne muscular dystrophy, I am writing to offer our heartfelt thanks for your support and leadership on ensuring that the Patient-Focused Impact Assessment (PFIA) and additional Patient Focused Drug Development (PFDD) provisions, so critically important to the Duchenne community, were included in The 21st Century Cures Act. You and your staff have worked tirelessly for the Duchenne and so many other patient communities on these important issues. With the passage of the 21<sup>st</sup> Century Cures legislation, you have made a permanent impact on how the FDA uses and reports on patient experience data.

PPMD is incredibly grateful to have had the opportunity to work with you and your office on this important piece of legislation, which builds on provisions included in the FDA Safety and Innovation Act (FDASIA) to further strengthen the voice of the patient and caregiver in the therapy developments process. We appreciate that the 21<sup>st</sup> Century Cures Act seeks to provide additional clarity and refinement to the PFDD development process and the provides a simple and transparent assessment instrument to better understand how such tools are – or are not – being used by FDA reviewers. These provisions offer our community and the patient advocacy community more broadly, with the assurance to further develop such tools.

We would also like to offer a special thank you to Sarah Lloyd Stevenson for her hard work and commitment to these issues which are so important to PPMD and the Duchenne community. We know we would not be where we are without her.

PPMD cannot convey the depth of our appreciation for your assistance and dedication in developing and advancing such important policy provisions. Because of you, we are one step closer to ending Duchenne. We truly couldn't have done it without you.

Sincerely,



Pat Furlong  
Founding President & CEO