

December 30, 2014

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

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

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

The Honorable Gene Green
Ranking Member
Subcommittee on Health
Committee on Energy and Commerce
2470 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton, Chairman Pitts, Ranking Member Pallone and Ranking Member Green,

We would like to commend you and the Energy and Commerce Committee for the great strides you are taking to advance the 21st Century Cures initiative. Each of our organizations has been deeply engaged in the Cures process throughout the year and is hopeful about this initiative's momentum in 2015. Specifically, **we are writing to urge that a provision known as the Patient-Focused Impact Assessment Act (PFIA) be included in your draft legislation when released in early 2015.**

The PFIA aims to build on the Food and Drug Administration Safety and Innovation Act (FDASIA) and its many provisions intending to strengthen the voice of the patient throughout the drug and larger medical product development process. FDASIA has catalyzed the movement toward patient-focused drug development (PFDD) and has motivated stakeholders to move toward developing PFDD tools informed through patient engagement. While patient organizations and industry strongly supported these reforms, much remains unknown as to the impact these policies are ultimately having and how these new tools are being used by the FDA. A feedback loop is needed in order to give confidence to stakeholders that these efforts are worth the time and resources being spent to provide them.

To address these concerns and ensure that the FDA applies existing and future patient-focused drug development tools and authorities to the greatest extent possible, Congress should enact the Patient-Focused Impact Assessment through 21st Century Cures. This proposal calls for greater transparency into the FDA review process to determine if the agency is – or is not – using its new tools and authorities, and for greater clarity from FDA as to activities patients and industry can take to further develop this field. A core component of the proposal is a simple patient-impact assessment that reviewers would complete at the time a product is developed and that would be publicly available as part of a final review package. This document would ask if reviewers used various PFDD tools in making their decisions, providing much needed transparency into the review process.

We strongly believe this proposal complements and builds upon the reforms of FDASIA and will help drive implementation of the FDASIA authorities and encourage further developments to enhance the patient voice and perspective in the product development process. To ensure that the FDA uses existing

and future patient-focused drug development tools and authorities to the greatest extent possible, we urge that the Committee include the PFIA provision within your draft legislation.

If you have any questions, please feel free to contact any of our organizations or Annie Kennedy with Parent Project Muscular Dystrophy at annie@parentprojectmd.org.

Thank you for your leadership and for considering this request.

Sincerely,