

January 22, 2015

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 21<sup>st</sup> 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 21<sup>st</sup> 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](mailto:annie@parentprojectmd.org).

Thank you for your leadership and for considering this request.

Sincerely,

ALS Association (ALSA)  
Association for Frontotemporal Degeneration (AFTD)  
BCC Nevus Syndrome Life Support Network  
BioNJ  
Charley's Fund  
Coalition Duchenne  
Congenital Adrenal hyperplasia Research, Education & Support Foundation (CARES Foundation, Inc.)  
Congenital Hypersulinism International  
cureCADASIL/CADASIL Association Inc  
Cure CMD  
Cure Sanfilippo Foundation  
Cure SMA  
Everylife Foundation for Rare Diseases  
Fabry Support & Information Group  
Fight SMA  
Foundation to Eradicate Duchenne (FED)  
Foundation for a Future  
Foundation for Prader-Willi Research  
Friedreich's Ataxia Research Alliance (FARA)  
Genetic Alliance (GA)  
Global Genes  
Hannah's Hope Fund  
Hope for Javier  
Hydrocephalus Association  
ICAN, International Cancer Advocacy Network  
JB's Keys to DMD  
John Owen's Adventure  
Kids With Heart National Assn for Children's Heart Disorders, Inc  
Life Raft Group  
Little Hercules Foundation  
MLD Foundation  
Myotonic Dystrophy Foundation  
National Down Syndrome Society (NDSS)

National Fragile X Foundation  
National MPS Society  
National Organization for Rare Disorders (NORD)  
Parent Project Muscular Dystrophy (PPMD)  
Pediatric Hydrocephalus Foundation  
Phelan-McDermid Syndrome Foundation  
Pietro's Fight  
Powerful Patient  
PXE International  
RASopathies Network USA  
Suneel's Light  
Two Smiles One Hope Foundation  
United Leukodystrophy Foundation

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