June 16, 2015

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

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

Dear Chairman Alexander and Ranking Member Murray,

We would like to commend you and the Committee on Health, Education, Labor and Pensions for the strides you are taking to advance the Senate's Innovation initiative to accelerate the development and delivery of therapies to patients in need. Each of our organizations has been deeply engaged over the past several years in advocating for laws and regulations that ensure the voice of the patient is taken into account during the medical product development and review process. As a result, tremendous strides have been made in the nascent field known Patient-Focused Drug Development (PFDD), and new PFDD tools are being developed with each passing day. Given these developments, it is critical that we understand how the Food and Drug Administration is – or is not – using such authorities and tools. To address this issue, we are **writing to urge that you include with your draft legislation an assessment tool known as the Patient-Focused Impact Assessment Act, or PFIA.**

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 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, we urge Congress to enact the Patient-Focused Impact Assessment (PFIA) through the Senate's Innovation initiative. 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,

Parent Project Muscular Dystrophy
ALS Association
Association for Frontotemporal Degeneration (AFTD))
BCC Nevus Syndrome Life Support Network
BioNJ
CARES Foundations
Charley's Fund
Coalition Duchenne
Congenital Hypernsulinism International
Cure Sanfilippo Foundation
cureCADASIL/CADASIL Association Inc.
CureCMD
CureSMA
Everylife
Fabry Support & Information Group
Foundation for a Cure
Foundation for Prader-Willi Research
Foundation to Eradicate Duchenne (FED)
Friedreich's Ataxia Research Alliance (FARA)
Genetic Alliance
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
Justin Fallon, PhD Brown University
Kids With Heart National Assn for Children's Heart Disorders, Inc
Life Raft Group
Little Hercules Foundation
MLD Foundation
Marathon Pharmaceuticals
Muscular Dystrophy Association
Myotonic Dystrophy Foundation
National Down Syndrome Society
National Fragile X Foundation
National Hydrocephalus Association
National MPS Society
National Organization for Rare Disease (NORD)
National Psoriasis Foundation
Pediatric Hydrocephalus Foundation
Phelan-McDermid Syndrome Foundation
Pietro's Fight
Powerful Patient
PXE International (Pseudoxanthoma elasticum)
RASopathies Network USA
Reveragen
Santhera Pharmaceuticals
Sarepta Therapeutics
Summit
Suneel's Light
United Leukodystrophy Foundation
Team Saij
Two Smiles One Hope Foundation