June 11, 2012

The Honorable Tom Harkin
Chairman
Committee on Health, Education Labor and Pensions
United States Senate
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Michael Enzi
Ranking Member
Committee on Health, Education, Labor and Pensions
United States Senate
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Fred Upton
Chairman
Committee on Energy & Commerce
United States Congress
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Henry Waxman
Ranking Member
Committee on Energy & Commerce
United States Congress
2322A Rayburn House Office Building
Washington, DC 20515

Dear Chairmen Harkin & Upton and Ranking Members Enzi & Waxman:

As you work to finalize the Food and Drug Administration (FDA) user fee legislation, we are writing to thank you for including a number of provisions to spur the delivery of lifesaving treatments for rare disease patients. We also urge that the final legislation include the strongest possible provisions as described below.

The need to accelerate development of therapies for rare diseases is clear. Currently, there are fewer than 400 approved treatments for 7,000 rare diseases affecting more than 30 million Americans. The science exists for many of these diseases to be treated; however, treatments may never be developed because of roadblocks in the development process, such as a lack of investment and a challenging regulatory environment. Your action is critical as there has been no major legislation to spur the development of treatments for rare diseases since the Orphan Drug Act was enacted more than 25 years ago. The inclusion of all the rare disease provisions in the final bill is our community’s best hope for spurring the development of treatments.

Specifically, we are grateful that both the EXPERRT Act that will allow the FDA to consult with rare disease experts and the Breakthrough Therapies Act that requires the FDA to take actions to expedite the development and review of a “breakthrough” drug are included in both H.R. 5651, the Food and Drug Administration Reform Act of 2012 and S. 3187, the Food and Drug Administration Safety & Innovation Act of 2012. These two provisions are an important first step at improving the regulatory process for rare diseases.

In addition to these sections, we urge you to include the following three provisions in the final version of the legislation, one of which is included in S. 3187 and the other two within H.R. 5651:
1) **S. 3187, Section 1132, Patient Participation in Medical Product Discussions:** This provision provides for a path forward to deepen patient engagement in reviewing medical products. Expanding and strengthening the patient voice in FDA discussions is important to help the FDA truly understand the risk/benefit for a rare disease treatment and other perspectives only a patient can provide. This section would build upon the EXPERRT Act that is included in both bills.

2) **H.R. 5651, Subtitle D-Accelerated Approval, Section 842:** Both bills include sections to enhance FDA's Fast Track and Accelerated Review tools to expedite review of therapies. This section, included in the House bill, fixes a “catch-22” for rare diseases that is not addressed elsewhere in the bill. It states the FDA, when issuing its guidance on Accelerated Approval, shall consider “how to incorporate novel approaches to the review of surrogate endpoints based on pathophysiologic and pharmacologic evidence in such guidance, especially in instances where the low prevalence of a disease renders the existence or collection of other types of data unlikely or impractical.” Without this specific language, it will remain extremely difficult for rare diseases to access the accelerated approval process, negating the intent of Congress in including this overall provision.

3) **H.R. 5651, Sec. 865:** This language creates additional incentives for industry to develop treatments for rare pediatric diseases and cancers by granting a voucher for priority review status that the developer can use to expedite review of another product.

These reforms will speed the approval of much-needed therapies and cures for rare disease patients as well as for others who are facing serious and life-threatening conditions. These provisions promise to maintain the FDA’s high standard for approval while at the same time ensuring that the agency can help facilitate the development of novel therapies to patients in a more timely manner.

We applaud your commitment to accelerating the delivery of safe and effective therapies to patients in need, and we urge that you include the most comprehensive provisions possible in the final legislation. If you have any questions, please feel free to contact Julia Jenkins of the EveryLife Foundation for Rare Diseases at jjenkins@everylifefoundation.org or Nick Manetto with Parent Project Muscular Dystrophy at nicholas.manetto@faegrebd.com.

Sincerely,

Abigail Alliance for Better Access to Developmental Drugs
Acromegaly Community
Addi and Cassi Fund
Adult Congenital Heart Association
Advocacy for Patients with Chronic Illness, Inc.
Alpha-1 Foundation
Alstrom Syndrome International
American Association for Respiratory Care
American Association on Health and Disability
Amyloidosis Support Groups Inc
Angioma Alliance
APBD Research Foundation
ARPKD/CHF Alliance
Batten Disease Support and Research Association
Bereaved Parent
BioSTL
Breakthrough Cancer Coalition
Brinson Patrick Securities Corporation
CADASIL Together We Have Hope Nonprofit Organization
CFC International
Children's Sickle Cell Foundation, Inc.
Children's Tumor Foundation
Choroideremia Research Foundation
Coalition of Heritable Disorders of Connective Tissue
Coriell Institute for Medical Research
Costello Syndrome Family Network
CRB1 Fund for Curing Retinal Blindness
Cure Duchenne
Detroit Medical Research Corps
EveryLife Foundation for Rare Diseases
Expression Analysis®
Families of Spinal Muscular Atrophy
Family Voices
Foundation for Prader-Willi Research
Foundation to Eradicate Duchenne (FED)
Friedreich’s Ataxia Research Alliance
Gastroparesis Patient Association for Cures and Treatments (G-PACT)
Generabio, Inc
Gene Spotlight Inc
Genetic Alliance
Ground Zero Pharmaceuticals, Inc.
Hannah's Hope Fund
Hereditary Disease Circle
HHT Foundation International
Hope4Bridget Foundation
Incontinentia Pigmenti International Foundation
InnoThink Center for Research in Biomedical Innovation
Institut für Medizinische Genetik und Humangenetik
International Cancer Advocacy Network
Jacob's Cure
Jain Foundation
Jeffrey Modell Foundation
Jonah’s Just Begun-Foundation to Cure Sanfilippo Inc.
Joshua Frase Foundation
Katsujinken Foundation
Kennedy's Disease Association
Kidney Cancer Association
Kids V Cancer
LAM Treatment Alliance
Lanai Community Health Center
Let Them Be Little X2
Lymphangiomatosis & Gorham's Disease Alliance
MarbleRoad
M-CM Network
MLD Foundation
Moebius Syndrome Foundation
Muscular Dystrophy Association
National Adrenal Diseases Foundation
National Bone Marrow Transplant Link
National Down Syndrome Society
National Fabry Disease Foundation
National Gaucher Foundation, Inc.
National MPS Society
National Organization for Disorders of the Corpus Callosum
National Tay-Sachs & Allied Diseases Association
National Tourette Syndrome Association
NBIA Disorders Association
Neurofibromatosis Michigan
NJ Center for Tourette Syndrome & Associated Disorders
NKH International Family Network
Noah's Hope - Batten Disease Fund
Pachyonychia Congenita Project
Parent Project Muscular Dystrophy
Partnership for Cures
Pediatric Adolescent Gastroesophageal Reflux Association
PMWC Intl.
Positive Exposure
Prader-Willi Syndrome Association of Pennsylvania
Prostate Health Education Network
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association
PXE International
Rett Syndrome Research Trust
RhizoKids International
Russell-Silver Syndrome Support
Sanfilippo Foundation for Children
Sarcoma Foundation of America
Schola Ministries
Shire HGT
Sohn Health Strategies, LLC
South College School of Pharmacy at Knoxville, Tennessee
Special Olympics Inc.
Stickler Involved People
Team Sanfilippo Foundation
The Association for Frontotemporal Degeneration (AFTD)
The Myelin Project
The National Neurofibromatosis Network
The RARE Project
The Ryan Foundation for Orphan Disease Research
Thisbe and Noah Scott Foundation, Inc.
Tom Curran DDS
Trimethylaminuria Foundation
Trisomy 18 Foundation
Tuberous Sclerosis Alliance
Turner Syndrome Society
United Pompe Foundation
Veracyte
Vermont Family Network
Washington State Neurofibromatosis Families