April 13, 2015

The Honorable Lamar Alexander, Chairman
Senate Committee on Health, Education,
Labor, and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

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

Dear Chairman Alexander and Ranking Member Murray:

On behalf of the 30 million men, women and children in the U.S. living with a rare disease, the undersigned organizations urge you to permanently authorize the *Rare Pediatric Disease Priority Review Voucher (PRV) Program* to drive greater development of novel treatments for children with a rare pediatric disease.

There are an estimated 7,000 rare diseases, which are defined as a disease affecting 200,000 or fewer people. Of the nearly one in ten Americans with a rare disease, approximately two-thirds are children. Of the 350 most "common" rare diseases, 27 percent result in death before the child's first birthday.

Despite significant unmet medical need (the approximately 450 approved orphan products treat only about 350 rare diseases), manufacturers face significant obstacles that can hinder the pursuit of rare disease therapies for children, including difficulties associated with conducting clinical trials. To tackle these hurdles, Congress established the *Rare Pediatric Disease PRV Program*.

Currently, upon FDA approval of a novel rare pediatric disease treatment, the *Rare Pediatric Disease PRV Program* provides a biopharmaceutical manufacturer the opportunity to receive a voucher guaranteeing a six month priority review of a New Drug Application (NDA) or Biologic License Application (BLA) for another product, rare disease or not. The voucher can be sold to another company, and there is no limit on how often it may be transferred.

Unfortunately, this program expires March 2016, ending a clear pathway that encourages innovators to pursue treatments in a difficult disease space. To date, three vouchers have been awarded and the program has shown clear evidence that it is a valuable incentive to develop drugs and biologics in this underserved area.

Congress established the *Rare Pediatric Disease PRV Program* because it recognized the necessity of an incentive to enhance innovation in this key area of unmet patient need, a market segment previously overlooked.

We urge Congress to permanently authorize the *Rare Pediatric Disease PRV Program,* which has proven its initial effectiveness in providing hope to children who are suffering from these rare conditions, and drawing manufacturers to invest in the development of novel treatments for rare pediatric diseases.

Sincerely,

Adult Polyglucosan Body Disease Research Foundation
ALD Connect
Alstrom Angels
Alstrom Syndrome International
American Association of the Deaf-Blind
American Autoimmune Related Diseases Association
American Partnership for Eosinophilic Disorders
American Thoracic Society
Amyloidosis Support Groups
Association for the Bladder Exstrophy Community
Association for Creatine Deficiencies
Association for Glycogen Storage Disease
Autoinflammatory Alliance
Avery's Angels Gastroschisis Foundation
Batten Disease Support and Research Association
Barth Syndrome Foundation
Bridge the Gap
CADASIL Together We Have Hope Non-Profit Organization
Canavan Foundation
CARES Foundation
CCHS Family Network
CFC International
Charcot-Marie-Tooth Association
Children's Brittle Bone Foundation
Children's Cardiomyopathy Foundation
Children's PKU Network
Chronic Granulomatous Disease Association
Circadian Sleep Disorders Network
Coalition for Pulmonary Fibrosis
Congenital Hyperinsulinism International
Cooley’s Anemia Foundation
Council for Bile Acid Deficiency Diseases
CureCADASIL Association
Cure AHC
Cure HHT
Cure JM Foundation
Cure SMA
debra of America
The Dent Diseases Foundation
Dravet Syndrome Foundation
Dupuytren Foundation
EB Research Partnership
Encephalitis Global
Everylife Foundation for Rare Diseases
Fabry Support & Information Group
Fight ALD-Fighting Illness Through Education

FOD (Fatty Oxidation Disorders) Family Support Group
Foundation Fighting Blindness
Foundation for Angelman Syndrome Therapeutics
Foundation for Ichthyosis & Related Skin Types
FPIES Foundation
Friedreich's Ataxia Research Alliance
Galactosemia Foundation
GBS/CIDP Foundation International
Global Genes
Gwendolyn Strong Foundation
Hereditary Neuropathy Foundation
Histiocytosis Association
Hope for Hypothalamic Hamartomas
International FOP Association
International Pemphigus and Pemphigoid Foundation (IPPF)
Jeffrey Modell Foundation
LGS Foundation
Lipodystrophy United
Little Miss Hannah Foundation
Lymphangiomatosis & Gorham's Disease Alliance
Lymphedema Advocacy Group
The Marfan Foundation
Moebius Syndrome Foundation
Myotonic Dystrophy Foundation
National Adrenal Diseases Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National MPS Society
National Organization for Rare Disorders
National Stem Cell Foundation
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
NGLY1.org
Noah's Hope Fund
NTM Info & Research
Organic Acidemia Association
Oxalosis and Hyperoxaluria Foundation
Parents and Researchers Interested in Smith-Magenis Syndrome
PCDH19 Alliances
PF Strategies
Phelan-McDermid Syndrome Foundation
Pituitary Network Association
Potocki-Lupski Syndrome Outreach Foundation
Project DOCC - Delivery of Chronic Care

Pulmonary Hypertension Association
Rare and Undiagnosed Network
Rare Disease United Foundation
Rare Genomics Institute
The Reflex Sympathetic Dystrophy Syndrome Association
Rettsyndrome.org
Sanfilippo Foundation for Children
Scleroderma Foundation
Simons VIP Connect
Stickler Involved People
Sturge-Weber Foundation
Tarlov Cyst Disease Foundation
The Transverse Myelitis Association
The United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
U.S. Hereditary Angioedema Association
Usher Syndrome Coalition
Vascular Birthmarks Foundation
VHL Alliance
We are RARE Inc.
XLH Network
The XLP Research Trust
5p-Society

For additional information, contact Paul Melmeyer, Assistant Director of Public Policy, National Organization for Rare Disorders (NORD), pmelmeyer@rarediseases.org, (202) 588-5700 ext. 104.