

November 18, 2016

The Honorable Mitch McConnell, Majority Leader
United States Senate
Washington, D.C. 20510

The Honorable Harry Reid, Minority Leader
United States Senate
Washington, D.C. 20510

The Honorable Paul Ryan, Speaker
United States House of Representatives
Washington, D.C. 20515

The Honorable Nancy Pelosi, Minority Leader
United States House of Representatives
Washington, D.C. 20515

Dear Majority Leader McConnell, Speaker Ryan, and Minority Leaders Reid and Pelosi,

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 pass the *21st Century Cures Act (H.R.6)* and include a long-term, optimally permanent, reauthorization of the *Rare Pediatric Disease Priority Review Voucher (PRV) Program*.

There are an estimated 7,000 rare diseases, which are defined as a disease affecting fewer than 200,000 people. Of the nearly one in ten Americans with a rare disease, over half are children. Despite significant unmet medical need (the approximately 550 approved orphan products treat only about 375 rare diseases), there are significant obstacles that can hinder the pursuit of rare disease therapies. Pediatric development often proves additionally difficult due to issues associated with conducting clinical trials.

The *21st Century Cures Initiative* includes various provisions that are widely supported by the rare disease patient community, including additional resources for the NIH, the expansion of the FDA Patient-Focused Drug Development Initiative (S.1597), provisions to streamline FDA review of targeted orphan therapies (S.2030), and additional incentives for adding rare disease indications onto labels (*OPEN Act*, S.1421).

The *21st Century Cures Act* and the *Senate Innovation for Healthier Americans Initiative* both included reauthorizations of the *Rare Pediatric Disease PRV Program*. This program has enjoyed bipartisan and bicameral support since its inception, and it was recently unanimously extended in September through the end of this year.

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 priority review of a new drug or biologic for another product. Unfortunately, this program is again set to expire at the end of this year, eliminating a clear pathway that encourages innovators to pursue treatments in a difficult disease space. Still in its infancy, this program has not been allowed to show its true effectiveness as short-term reauthorizations do not maximize the potential benefits of this powerful incentive.

Through *21st Century Cures*, Congress has bipartisanly recognized that more needs to be done for the rare disease patient community. The *Rare Pediatric Disease PRV Program* is just one of many important provisions, but it uniquely requires immediate Congressional attention due to its approaching expiration date. Continued inaction or short-term reauthorizations will only work to discourage industry from investing in rare pediatric diseases.

Thousands of patients and advocates have asked Congress to act. It is imperative that Congress passes the *21st Century Cures Act* and includes a long-term, optimally permanent, reauthorization of the *Rare Pediatric Disease PRV Program*.

Sincerely,

A Twist of Fate-ATS
Action CF
Adrenal Insufficiency United
Adult Polyglucosan Body Disease Research Foundation
ALD Connect
Alport Syndrome Foundation
Alternating Hemiplegia of Childhood Foundation
American Autoimmune Related Disease Association
American Behcet's Disease Association
American Childhood Cancer Organization
American Liver Foundation
American Partnership for Eosinophilic Disorders
American Thoracic Society
Amyloidosis Support Groups
Angelman Syndrome Foundation
APBD Research Foundation
Aplastic Anemia & MDS International Foundation
Arizona network for PKU and allied disorders
Association for Creatine Deficiencies
Association for Glycogen Storage Disease
Association of Gastrointestinal Motility Disorders, Inc. | AGMD |
Batten Disease Support and Research Association
Bohring-Opitz Syndrome Foundation, Inc.
BOS Foundation
Bridge The Gap Syngap Education & Research Foundation 501c3
Brittle Diabetes Foundation
CADASIL Together We Have Hope
The Calliope Joy Foundation
Canavan Foundation
CARES Foundation, Inc.
CCHS Network
Child Neurology Foundation
Children's Cardiomyopathy Foundation
Children's PKU Network
Children's Tumor Foundation
Chorouderemia Research Foundation
Chromosome Disorder Outreach Inc.
Chronic Granulomatous Disease Association
Cicatrical Alopecia Research Foundation
CJD Aware!
The Cluster Headache Support Group
Clusterbusters, Inc
CMTA (Charcot-Marie-Tooth Association)
Congenital Hyperinsulinism International
Connecting Families Urea Cycle Disorders
Consortium of Multiple Sclerosis Centers
Council for Bile Acid Deficiency Diseases
cureCADASIL
Cure JM Foundation
CurePSP
Cure AHC
Cure Sanfilippo Foundation
Curing Retinal Blindness Foundation

Cystic Fibrosis Research, Inc. / CFRI
Cystinosis Research Network
debra of America
The Desmoid Tumor Research Foundation
Dravet Syndrome Foundation
Dupuytren Foundation
Dysautonomia International
Dyskeratosis Congenita Outreach, Inc.
Encephalitis Global
Epilepsy Foundation of Greater Chicago
Evans Syndrome Foundation
EveryLife Foundation for Rare Diseases
Fat Disorders Research Society
Fibromuscular Dysplasia Society of America
Fibrous Dysplasia Foundation
Focus On Rhabdo (Rhabdomyosarcoma)
Foundation Fighting Blindness
Foundation for Prader-Willi Research
FOD (Fatty Oxidation Disorders) Family Support Group
FPIES Foundation
Friedreich's Ataxia Research Alliance (FARA)
Galactosemia Foundation
The Global Foundation for Peroxisomal Disorders
Global Genes
Guardian Hands Foundation
Gut Check Foundation
The Guthy-Jackson Charitable Foundation
Hannah's Hope Fund
Hereditary Neuropathy Foundation
Histiocytosis Association
Hope for Hypothalamic Hamartomas
Huntington's Disease Society of America
Hydrocephalus Association
Immune Deficiency Foundation
Indian Organization for Rare Diseases
International Fibrodysplasia Ossificans Progressiva (FOP) Association
International Foundation for Autoimmune Arthritis
International Organization of MS Nurses
International Pemphigus & Pemphigoid Foundation
International WAGR Syndrome Association
Jack McGovern Coats' Disease Foundation
Jeffrey Modell Foundation
Jett Foundation
Joshua Frase Foundation
Kennedy's Disease Association
Kure for Kulas
LAL D Aware
Light of Life Foundation, Inc.
Lipodystrophy United
Lupus and Allied Diseases Association
Lymphedema Advocacy Group
Massachusetts Chapter of the Marfan Foundation
MDS Foundation, Inc.

MEBO RESEARCH, INC.
Medical Alley Association
Mesothelioma Applied Research Foundation
MitoAction
MLD Foundation
Moebius Syndrome Foundation
Mowat Wilson Syndrome Foundation
Mucopolidosis Type IV (ML4) Foundation
Myasthenia Gravis Foundation of America
The Myelin Project
Myocarditis Foundation
The National Adrenal Diseases Foundation
The National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National Fabry Disease Foundation
National Foundation for Ectodermal Dysplasias
National Fragile X Foundation
National MPS Society
National Organization for Albinism and Hypopigmentation
National Organization for Rare Disorders (NORD)
National PKU Alliance
National PKU News
National Tay-Sachs & Allied Diseases Association, Inc.
NBIA Disorders Association
Neurofibromatosis Northeast
Neuromuscular Disease Foundation
NGLY1.org
Noah's Hope - Hope4Bridget Foundation
North American Malignant Hyperthermia Registry of MHAUS
NOTA (The Network of Tyrosinemia Advocates)
The Oley Foundation
Organic Acidemia Association
Osteogenesis Imperfecta Foundation
Parent Project Muscular Dystrophy
Pathways.org
PCD Foundation
PKD Foundation
Platelet Disorder Support Association
Prader-Willi Syndrome Association (USA)
Prion Alliance
PSC Partners Seeking a Cure
Rare Disease United Foundation
Rare and Undiagnosed Network (RUN)
RASopathies Network
Reflex Sympathetic Dystrophy Syndrome Association
Relapsing Polychondritis Awareness and Support Foundation, Inc.
Research!America
Rett Syndrome Research Trust
Rettsyndrome.org
RYR-1 Foundation
Sanfilippo Children's Foundation
Sarcoma Foundation of America

SBS Cure Project
The Shaken Baby Alliance
Short Bowel Syndrome Foundation, Inc.
The Simon Foundation for Continence
The Sitosterolemia Foundation
The Snyder-Robinson Foundation
Sofia Sees Hope
Spastic Paraplegia Foundation
SSADH Association
Stevens - Johnson Syndrome Foundation
The Sturge-Weber Foundation
Taylor's Tale
Tourette Association of America
The Transverse Myelitis Association
Tuberous Sclerosis Alliance
Turner Syndrome Society of the U.S.
United Leukodystrophy Foundation
United Ostomy Associations of America, Inc.
Usher Syndrome Coalition
Vasculitis Foundation
Vestibular Disorders Association
VHL Alliance
We Are R.A.R.E., Inc.
Wilhelm Foundation - the Undiagnosed
Williams Syndrome Association, Inc.
Worldwide Syringomyelia & Chiari Task Force
Xeroderma Pigmentosum Society, Inc.

CC: The Honorable Lamar Alexander, Chairman, Senate Committee on Health, Education, Labor, and Pensions
The Honorable Patty Murray, Ranking Member, Senate Committee on Health, Education, Labor, and Pensions
The Honorable Fred Upton, Chairman, House Committee on Energy and Commerce
The Honorable Frank Pallone, Ranking Member, House Committee on Energy and Commerce