May 5, 2015

The Honorable Gus Bilirakis U.S. House of Representatives Washington, D.C. 20515	The Honorable G.K. Butterfield U.S. House of Representatives Washington, D.C. 20515

Dear Representatives Bilirakis & Butterfield:

We are writing to commend your introduction of the Orphan Product Extensions Now Accelerating Cures & Treatments, also known as the OPEN ACT HR 971. On behalf of the patients we represent, we wish to add our names in support of this important legislation.

We applaud the OPEN ACT because it will bring hundreds of safe, effective, and affordable medicines to rare disease patients within the next several years by incentivizing drug makers to "repurpose" major market drugs for the treatment of life-threatening rare diseases and pediatric cancers. Although biopharmaceutical companies are not currently repurposing major market therapies to treat rare diseases, the OPEN ACT solves this problem by making available an "Orphan Product Exclusivity Extension," which would provide an additional six months of market exclusivity to the patent life of the major market drug being repurposed so long as the sponsor company establishes that the therapy is designated to treat a rare disease and obtains a rare disease indication from the federal Food and Drug Administration (FDA) on the drug label.

With 95 percent of rare diseases having no FDA-approved cure, we are confident the OPEN ACT will result in a significant increase in the number of well-tested therapies approved by the FDA for use in treating rare disease patients. We also believe the OPEN ACT will lead to rare disease therapies priced at major market prices, fewer rare disease patients using untested and potentially ineffective drugs off–label, and a boost in investment in the biotech sector.

In closing, we thank you for championing this important piece of legislation which promises to improve the quality of life for the nearly 30 million Americans suffering from rare diseases. We are eager to work with you and your staff in advocating this important legislation and look forward to the day it is enacted.

Sincerely,

National MPS Society
With Purpose
National PKU Alliance
Taylor's Tale
RASopathies Network USA
Kids v Cancer
Let Them Be Little X2 Inc.
Info and Resources for Idiopathic Pulmonary Hemosiderosis (IPH-NET)
Noah's Hope

Mary Payton's Miracle Foundation
Hope4Bridget Foundation
Batten Disease Support & Research Association
Cure Sanfilippo Foundation
Beyond Batten Disease Foundation
Drew's Hope Scientific Research Foundation
International Pemphigus and Pemphigoid Foundation (IPPF)
Cure AHC
Autoinflammatory Alliance
MLD Foundation
Fabry Support & Information Group
Children's PKU Network
FMD Chat
National Tay-Sachs & Allied Diseases Association (NTSAD)
Little Miss Hannah Foundation
Rare Disease United Foundation
Global Genes Project
Fibromuscular Dysplasia Society of America (FMDSA)
Lymphatic Malformation Institute
Mastocytosis Society
EB Research Partnership
BRBN Alliance
Jonah's Just Begun
Abigail Alliance for Better Access to Developmental Drugs
Hannah's Hope Fund
GNE Myopathy International
The Ryan Foundation
Organic Acidemia Association
Cardio-Facio-Cutaneous International
NGLY1.org
Gwendolyn Strong Foundation
POMC Island One boy an Ocean of friends
Gene Giraffe Project
International FOP Association
Aware of Angels
CureCADASIL
GT23 FOUNDATION
Desmoid Tumor Research Foundation (DTRF)
The Association for Glycogen Storage Disease
Gene Spotlight Inc.
Amyloidosis Foundation
Heriditary Neuropathy Foundation
Relapsing Polychondritis

Klippel-Feil Syndrome Freedom
CureDuchenne
Prader-Willi Syndrome Association
EveryLife Foundation for Rare Diseases
Bert's Big Adventure
Parent Project Muscular Dystrophy
Sarcoma Foundation of America
The Nicholas Conor Institute
Luck2Tuck Foundation
Team Sanfilippo Foundation
The Rally Foundation for Childhood Cancer Research
CARES Foundation, Inc.
Help Extinguish Hunter Syndrome
Sephardic Health Organization for Referral & Education
Hunter Syndrome Research Coalition
The Kortney Rose Foundation
Saving Case & Friends
Phelan-McDermid Syndrome Foundation
The Children's Medical Research Foundation, Inc.
Cure SMA
Narcolepsy Network
Celiac Support Association
Caleb's Crusade Against Childhood Cancer
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
PKD Foundation
EDSers United Foundation
Choroideremia Research Foundation, Inc.
Genetic Alliance
The Life Raft Group
The Will Luthcke Foundation
Angioma Alliance
Smashing Walnuts Foundation
Castleman Disease Collaborative Network/Castleman's Awareness & Research Effort
The GIST Cancer Awareness Foundation
The Truth 365
The Arms Wide Open Childhood Cancer Foundation
Sophia's Fund
Journey4ACure
Princesses on a Mission, Inc.
Noah's Light Foundation
Pediatric Cancer Foundation
West Virginia Kids Cancer Crusaders, Inc.
Bear Necessities Cancer Foundation

A Kids' Brain Tumor Cure
RARE Science, Inc.
ISMRD (the International Advocate for Glycoprotein Storage Diseases)
Hermansky-Pudlak Syndrome Network Inc.
Run4Rare
A-T Children's Project
The Global Foundation for Peroxisomal Disorders
The Adult Polyglucosan Body Disease Research Foundation (APBDRF)
Alexa Nawrocki Pediatric Cancer Foundation
Beckwith-Wiedemann Children's Foundation International
The Brooke Healey Foundation
Talia’s Legacy Children’s Cancer Foundation
The Rare Childhood Cancer Advocacy Group
Alex's Army Childhood Cancer Foundation
The Catherine Elizabeth Blair Memorial Foundation
Stillbrave Childhood Cancer Foundation
Cures Within Reach
ALL4Trey
Team Sabrina
Sofia's Hope, Inc.
ALL4Trey
Delainee’s Battle
Joey's Wings Foundation
The Bozeman 3
Team Ashley Bragg
Cole vs Cancer
Dominick One in a Million
Samuel Szabo Foundation
Wilms Tumor Survivor Group
Aiden's Army
Sofia's Hope, Inc.
Mikey's Way Foundation
Team Serena
Supporting Our Cancer Kids
The Champ's Corner
Habitat for Hope
Ali's Angels Foundation
Gold Rush Cure Foundation
Sickle Cell Warriors, Inc.
The Rare Cancer Research Foundation
Carson Leslie Foundation
Amyloidosis Research Consortium
Pulmonary Fibrosis Advocates

The Coalition for Pulmonary Fibrosis
Mytonic Dystrophy Foundation
LMSarcoma Direct Research Foundation
BioPontis Alliance for Rare Diseases
Foundation for Ichthyosis & Related Skin Types, Inc.
5p- Society
The Santonio Holmes III & Long Foundation
National Fragile X Foundation
National Organization for Rare Disorders (NORD)
OsteoPETrosis Society
Curing Retinal Blindness Foundation
The MAGIC Foundation
Cure HHT
DEFY Foundation
Chase After a Cure
DC Outreach Inc.
Children's Cardiomyopathy Foundation