November 8, 2017

The Honorable Paul Ryan, Speaker
United States House of Representatives
H-232, The Capitol
Washington, D.C. 20515

The Honorable Nancy Pelosi, Minority Leader
United States House of Representatives
H-204, The Capitol
Washington, D.C. 20515

The Honorable Kevin Brady, Chairman
U.S. House Committee on Ways & Means
1102 Longworth House Office Building
Washington, D.C. 20515

The Honorable Richard Neal, Ranking Member
U.S. House Committee on Ways & Means
1106 Longworth House Office Building
Washington, D.C. 20515

Dear Speaker Ryan, Leader Pelosi, Chairman Brady, and Ranking Member Neal:

As organizations representing millions of American men, women and children with rare diseases, we are writing to express strong concern with the proposed repeal of the Orphan Drug Tax Credit (ODTC) within the *Tax Cuts and Jobs Act*. The Orphan Drug Tax Credit is one of the only tax credits that saves lives. With 95 percent of individuals with a rare disease still waiting for a treatment, we implore you to maintain this critical incentive for orphan drug development.

The ODTC allows drug manufacturers to claim a tax credit of 50 percent of the qualified costs of clinical research and drug testing of orphan drugs (drugs for diseases affecting 200,000 Americans or fewer). The ODTC is part of a package of provisions enacted in 1983 in the *Orphan Drug Act* (ODA) that provides incentives for drug companies to develop products for rare diseases. This legislation has been extremely successful.

In the decade before the *Orphan Drug Act*, only 10 medicines were developed by industry for rare diseases. Since 1983, however, more than 3,500 potential treatments have been designated as an orphan drug, and more than 500 orphan therapies have been approved by the FDA. This is a direct result of the incentives provided by the ODA, including the tax credit.

In June 2015, the National Organization for Rare Disorders (NORD) and the Biotechnology Innovation Organization (BIO) published an economic analysis of the ODTC that quantifies the impact the ODTC has on incentivizing orphan drug development. The analysis found that without the ODTC, approximately 33 percent fewer orphan therapies would have been developed over the previous 32 years, and 33 percent fewer orphan therapies will be developed going forward if the tax credit is repealed. This would be a critical blow to individuals with rare diseases across the country.

Much remains to be done. Of the approximately 7,000 diseases considered rare in the U.S., only a few hundred have FDA-approved treatments. This leaves millions of Americans with diseases that currently have no treatment or cure.

The Orphan Drug Tax Credit gives hope to the nearly 95 percent of individuals with rare diseases without a treatment that one day they too will have a treatment, or even cure. We cannot afford to move backwards.

Sincerely,

A Cure In Sight
A Twist of Fate-ATS
Acoustic Neuroma Association
ACPMP
Acromegaly Community
Adenoid Cystic Carcinoma Research Foundation
ADNP Kids Research Foundation
Adrenal Insufficiency United
Adult Polyglucosan Body Disease Research Foundation
AKU Society of North America
All Things Kabuki Inc
Alpha-1 Foundation
Alport Syndrome Foundation
ALS Association
American Cancer Society Cancer Action Network
American Lung Association
American Lyme Disease Foundation
American Partnership for Eosinophilic Disorders (APFED)
American Porphyria Foundation
American Society of Gene & Cell Therapy (ASGCT)
American Thoracic Society
Amyloidosis Foundation
Amyloidosis Research Consortium
Amyloidosis Support Groups
Angelman Biomarkers and Outcome Measures Alliance
Angioma Alliance
Aplastic Anemia and MDS International Foundation
APS Foundation of America, Inc.
The APS Type 1 Foundation, Inc.
Association for Creatine Deficiencies
Association for Frontotemporal Degeneration (AFTD)
Autism Speaks
Axis Advocacy
Benign Essential Blepharospasm Research Foundation
BORN A HERO
Bridge the Gap - SYNGAP Education and Research Foundation
Canavan Research Illinois
The CCHS Network
CdLS Foundation
The Charlotte and Gwenyth Gray Foundation to Cure Batten Disease
Children's Cardiomyopathy Foundation

The Children’s Fund for Glycogen Storage Disease Research
Children's PKU Network
Children's Tumor Foundation
Chloe's Fight Rare Disease Foundation
Chromosome Disorder Outreach, Inc. (CDO)
CJD Aware!
Cluster Headache Support Group
Congenital Hyperinsulinism International
Consortium of Multiple Sclerosis Centers
Crohn’s & Colitis Foundation
Cure HHT Foundation
cureCADASIL
CurePSP, Inc.
CureSMA
Cushing's Support & Research Foundation (CSRF)
Cutaneous Lymphoma Foundation
Cyclic Vomiting Syndrome Association (CVSA)
Cystinosis Research Network
Daybreak Children's Rare Disease Fund
debra of America
The Desmoid Tumor Research Foundation
Digestive Disease National Coalition
Dravet Syndrome Foundation, Inc.
Dysautonomia Foundation, Inc.
Dyskeratosis Congenita Outreach, Inc.
Dystonia Advocacy Network
Epilepsy Foundation
The Erythromelalgia Association
EveryLife Foundation
Fabry Support & Information Group
Family Caregiver Alliance
Fat Disorders Research Society
Fibrolamellar Cancer Foundation
Fibromuscular Dysplasia Society of America
Fibrous Dysplasia Foundation
FOD Family Support Group
Foundation for Angelman Syndrome Therapeutics (FAST)
Foundation for Prader-Willi Research
Foundation for Sarcoidosis Research
Friedreich's Ataxia Research Alliance (FARA)
Friends of Cancer Research

Galactosemia Foundation
Gastroparesis Patient Association for Cures and Treatments, Inc. (G-PACT)
GBS|CIDP Foundation International
GI Cancers Alliance
The Global Foundation for Peroxisomal Disorders
Glut1 Deficiency Foundation
Gut Check Clostridium Septicum Foundation
The Guthy-Jackson Charitable Foundation
Hannah's Hope Fund
Healing Hugs Haven LLC
Hemophilia Federation of America
Hereditary Neuropathy Foundation
Hermansky-Pudlak Syndrome Network
Histiocytosis Association
Hope for Hypothalamic Hamartomas
Huntington’s Disease Society of America (HDSA)
Hydrocephalus Association
Immune Deficiency Foundation
Indian Organization for Rare Diseases
International Fibrodysplasia Ossificans Progressiva Association
International Foundation for CDKL5 Research
International Foundation for Functional Gastrointestinal Disorders
International FOXG1 Foundation
International Myeloma Foundation
International Pemphigus & Pemphigoid Foundation
International Rett Syndrome Foundation
International Waldenstrom's Macroglobulinemia Foundation
Interstitial Cystitis Association
Jack McGovern Coats' Disease Foundation
The Jansen's Foundation
Kennedy’s Disease Association, Inc.
Kids With Heart National Assn for Children’s Heart Disorders, Inc.
KIF1A.ORG
LAL D Aware
The LAM Foundation
LGS Foundation
Li-Fraumeni Syndrome Association (LFS Association / LFSA)
Little Miss Hannah Foundation
Lymphangiomatosis & Gorham's Disease Alliance
Lymphedema Advocacy Group
The MAGIC Foundation

The Mastocytosis Society, Inc.
The Marfan Foundation
MEBO Research, Inc.
Mesothelioma Applied Research Foundation
Mila's Miracle Foundation
MitoAction
MLD Foundation
Moebius Syndrome Foundation
The Morgan Leary Vaughan Fund
MPN (Myeloproliferative Neoplasms) Research Foundation
Mucolipidosis Type IV Foundation
Myasthenia Gravis Foundation of America
The Myelin Project
Myocarditis Foundation
The Myositis Association
Myotonic Dystrophy Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National LeioMyoSarcoma Foundation
National MPS Society
National Niemann-Pick Disease Foundation
National Organization for Albinism and Hypopigmentation (NOAH)
National Organization for Rare Disorders (NORD)
National PKU Alliance
National PKU News
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
NephCure Kidney International
NGLY1.org
The NICER Foundation, Inc.
NTM Info & Research
Oley Foundation
Organic Acidemia Association
Parent Project Muscular Dystrophy (PPMD)
Parents and Researchers Interested in Smith-Magenis Syndrome (PRISMS, Inc)
Phelan-McDermid Syndrome Foundation
PKD Foundation
Platelet Disorder Support Assocation
Prader-Willi Syndrome Association (USA)

The Progeria Research Foundation
PSC Partners Seeking a Cure
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association
Quincy's Quest Foundation
Rare and Undiagnosed Network (RUN)
RASopathies Network USA
Reflex Sympathetic Dystrophy Syndrome Association (RSDSA)
Research!America
RYR-1 Foundation
Sanfilippo Children's Foundation
Sarcoidosis of Long Island
Sarcoma Foundation of America
SBS Cure Project
Scleroderma Foundation
Sitosterolemia Foundation
Snyder-Robinson Foundation
Sofia Sees Hope
Soft Bones, Inc.: The US Hypophosphatasia Foundation
Spastic Paraplegia Foundation
Spinal CSF Leak Foundation
SSADH Association
Stevens Johnson Syndrome Foundation
SUDC Foundation
TargetCancer Foundation
Tarlov Cyst Disease Foundation
The Transverse Myelitis Association
Tuberous Sclerosis Alliance
Turner Syndrome Society of the United States
United Leukodystrophy Foundation
US Hereditary Angioedema Association
Vasculitis Foundation
VHL Alliance
Wilhelm Foundation - the Undiagnosed
Wishes for Elliott: Advancing SCN8A Research
Worldwide Syringomyelia & Chiari Task Force
The XLH Network, Inc.

For additional information, contact Paul Melmeyer, Director of Federal Policy, National Organization for Rare Disorders (NORD), pmelmeyer@rarediseases.org, (202) 545-3828.

CC: The Honorable Orrin Hatch, Chairman, Senate Committee on Finance

The Honorable Ron Wyden, Ranking Member, Senate Committee on Finance
The Honorable Kevin McCarthy, House Majority Leader
The Honorable Steny Hoyer, House Minority Whip
Members of the U.S. House of Representatives Committee on Ways & Means