

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over the previous 32 years, and 33 percent fewer orphan therapies will be developed going forward if the tax credit is repealed.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  
ACPMF  
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  
Mucopolidosis 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 Association  
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  
RZR-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](mailto: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