

September 14, 2017

The Honorable Orrin Hatch, Chairman  
U.S. Senate Committee on Finance  
219 Dirksen Senate Office Building  
Washington, D.C. 20510

The Honorable Ron Wyden, Ranking Member  
U.S. Senate Committee on Finance  
219 Dirksen Senate Office Building  
Washington, D.C. 20510

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 Chairmen Hatch and Brady and Ranking Members Wyden and Neal:

As organizations representing millions of American men, women and children with rare diseases, we are writing to express our strong support for the Orphan Drug Tax Credit (ODTC). We understand that Congress is developing tax reform proposals and we urge you to keep this critical tax credit in place.

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 provide 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over the previous 32 years, and 33 percent fewer orphan therapies will be developed going forward if the tax credit is repealed.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 is one of the only tax credits that actually saves lives. It also 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.

Sincerely,

A Cure In Sight  
A Twist of Fate-ATS  
Acoustic Neuroma Association  
Acromegaly Community  
ADNP Kids Research Foundation  
Adrenal Insufficiency United  
Adult Polyglucosan Body Disease Research Foundation  
AKU Society of North America  
All Things Kabuki Inc  
Alport Syndrome Foundation  
American Lung Association  
American Partnership for Eosinophilic Disorders (APFED)  
American Porphyria Foundation  
American Society of Gene & Cell Therapy (ASGCT)  
American Thoracic Society  
Amyloidosis Research Consortium  
Amyloidosis Support Groups  
Angelman Biomarkers and Outcome Measures Alliance  
The APS Type 1 Foundation, Inc.  
Association for Creatine Deficiencies  
Benign Essential Blepharospasm Research Foundation  
BORN A HERO  
Bridge the Gap - SYNGAP Education and Research Foundation  
CdLS Foundation  
The Charlotte and Gwenyth Gray Foundation to Cure Batten Disease  
Children's Cardiomyopathy Foundation  
Children's PKU Network  
Chloe's Fight Rare Disease Foundation  
Cluster Headache Support Group  
Congenital Hyperinsulinism International  
Consortium of Multiple Sclerosis Centers  
CureSMA  
The Desmoid Tumor Research Foundation  
Digestive Disease National Coalition  
Dysautonomia Foundation, Inc.  
Dyskeratosis Congenita Outreach, Inc.  
Dystonia Advocacy Network  
The Erythromelalgia Association  
EveryLife Foundation  
Family Caregiver Alliance  
Fat Disorders Research Society  
Fibrolamellar Cancer Foundation  
Fibromuscular Dysplasia Society of America  
Fibrous Dysplasia Foundation  
Foundation for Prader-Willi Research  
Foundation for Sarcoidosis Research

Friedreich's Ataxia Research Alliance (FARA)  
Galactosemia Foundation  
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  
Healing Hugs Haven LLC  
Hereditary Neuropathy Foundation  
Hermansky-Pudlak Syndrome Network  
Histiocytosis Association  
Hope for Hypothalamic Hamartomas  
Immune Deficiency Foundation  
Indian Organization for Rare Diseases  
International Fibrodysplasia Ossificans Progressiva Association  
International Foundation for Functional Gastrointestinal Disorders  
International FOXP1 Foundation  
International Myeloma Foundation  
International Pemphigus & Pemphigoid Foundation  
International Waldenstrom's Macroglobulinemia Foundation  
Interstitial Cystitis Association  
Jack McGovern Coats' Disease Foundation  
The Jansen's Foundation  
LAL D Aware  
Li-Fraumeni Syndrome Association (LFS Association / LFSA)  
Little Miss Hannah Foundation  
Lymphangiomatosis & Gorham's Disease Alliance  
Lymphedema Advocacy Group  
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  
Mucopolysaccharidosis Type IV Foundation  
Myasthenia Gravis Foundation of America  
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 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  
Parent Project Muscular Dystrophy (PPMD)  
Parents and Researchers Interested in Smith-Magenis Syndrome (PRISMS, Inc)  
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  
Rare and Undiagnosed Network (RUN)  
RASopathies Network USA  
Reflex Sympathetic Dystrophy Syndrome Association (RSDSA)  
RYR-1 Foundation  
Sarcoidosis of Long Island  
Sarcoma Foundation of America  
SBS Cure Project  
Scleroderma Foundation  
Sitosterolemia Foundation  
Sofia Sees Hope  
Soft Bones, Inc.: The US Hypophosphatasia Foundation  
Spastic Paraplegia Foundation  
Spinal CSF Leak Foundation  
SSADH Association  
SUDC Foundation  
TargetCancer Foundation  
Tarlov Cyst Disease Foundation  
The Transverse Myelitis Association  
Tuberous Sclerosis Alliance  
United Leukodystrophy Foundation  
US Hereditary Angioedema Association  
Vasculitis Foundation  
VHL Alliance

Wilhelm Foundation  
Worldwide Syringomyelia & Chiari Task Force

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: Members of the U.S. Senate Committee on Finance  
Members of the U.S. House of Representatives Committee on Ways & Means