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over the previous 32 years, and 33 percent fewer orphan therapies will be developed going forward if the tax credit is repealed.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 FOXG1 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 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
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 Assocation
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, (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