



July 25, 2013

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

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

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

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

Dear Chairmen Camp and Baucus and Ranking Members Levin and Hatch:

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

According to Marlene E. Haffner, MD, MPH, former Director of the FDA Office of Orphan Products Development: “Tax credits are an integral part of the Orphan Drug Program and are a lifeline to small and medium-sized firms as they are developing and growing their business and their products to treat rare diseases. The tax credits are a very small portion of tax revenue, but a very large incentive for the development of products for the treatment of rare diseases, many of which are life-threatening; 50% of those affected by these diseases are children.”

The ODTC allows drug manufacturers to claim a tax credit of 50% 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* 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 2,700 potential treatments have entered the research pipeline as orphan products and more than 300 have been approved by the FDA, largely as a result of the incentives provided by the ODA including the tax credit.

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 those people while also aiding the U.S. economy by enhancing the growth of firms developing products to treat rare diseases.

We appreciate your attention to this important matter.

Alpha-1 Association
Alpha-1 Foundation
Alport Syndrome Foundation
Alstrom Syndrome International
Alternating Hemiplegia of Childhood Foundation
American Partnership for Eosinophilic Disorders
Amyloidosis Support Groups
Aplastic Anemia & MDS International Foundation
ARPKD/CHF Alliance
Association for Frontotemporal Degeneration
Association for Glycogen Storage Disease
Association for Multiple Endocrine Neoplasia Disorders (AMEND)
Benign Essential Blepharospasm Research Foundation
BMT InfoNet (Blood & Marrow Transplant Information Network)
Caring for Carcinoid Foundation
CFC International
Cicatricial Alopecia Research Foundation (CARF)
CJD Aware!
Coalition for Pulmonary Fibrosis
Coalition of Heritable Disorders of Connective Tissue (CHDCT)
Congenital Hyperinsulinism International
Council for Bile Acid Deficiency Diseases
Creutzfeldt-Jakob Disease Foundation
Cushing Support and Research Foundation
Cutaneous Lymphoma Foundation
Fabry Support & Information Group
Families of Spinal Muscular Atrophy
Foundation for Prader-Willi Research
Friedreich's Ataxia Research Alliance (FARA)
GBS/CIDP Foundation International
Global Foundation for Peroxisomal Disorders
Guthy-Jackson Charitable Foundation
Hageman Foundation
Hereditary Hemorrhagic Telangiectasia Foundation International
Histiocytosis Association
HLRCC Family Alliance
Huntington's Disease Society of America
Hydrocephalous Association
International Fibrodysplasia Ossificans Progressiva Association
International Foundation for CDKL5 Research
International Myeloma Foundation
International Pemphigus and Pemphigoid Foundation
International Rett Syndrome Foundation
Kennedy's Disease Association
Les Turner ALS Foundation
Lymphangiomatosis & Gorham's Disease Alliance
Lymphedema Advocacy Group
Mastocytosis Society

M-CM Network
Moebius Syndrome Foundation
Mucopolidosis Type IV (ML4) Foundation
National Alopecia Areata Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National Marfan Foundation
National MPS Society
National Organization for Rare Disorders (NORD)
National PKU Alliance
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
Oley Foundation
Osteogenesis Imperfecta Foundation
Oxalosis & Hyperoxaluria Foundation
Pachyonychia Congenita Project
Pancreatic Cancer Action Network
Phelan-McDermid Syndrome Foundation
PSC Partners Seeking a Cure
Pulmonary Hypertension Association
Rare Disease United Foundation
Reflex Sympathetic Dystrophy Syndrome Association
Scleroderma Foundation
United Mitochondrial Disease Foundation
VHL Family Alliance

For additional information, contact Diane Edquist Dorman, Vice President, Public Policy, National Organization for Rare Disorders (NORD), ddorman@rarediseases.org, (202) 588-5700 ext. 102.

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