

Support Accelerated Approval of Drugs for Ultra-Rare Diseases to Benefit Vulnerable Patients, Promote Biotechnology Innovation and Stimulate Job Growth in the United States

December 21, 2011

The Honorable Fred Upton, Chairman
The Honorable Henry Waxman, Ranking Member
Energy & Commerce Committee
United States House of Representatives
Washington, DC 20515

The Honorable Joe Pitts, Chairman
The Honorable Frank Pallone, Ranking Member
Energy & Commerce Committee
Subcommittee on Health
United States House of Representatives
Washington, DC 20515

Dear Chairmen Upton and Pitts and Ranking Members Waxman & Pallone;

We the undersigned patient advocacy groups and supporters of improving the development process for ultra rare diseases are writing you to ask for your support of H.R.3737, the Unlocking Lifesaving Treatments for Rare diseases Act of 2012 to promote the discovery and development of safe and efficacious drugs and biologics to prevent, diagnose or treat ultra-rare diseases. This bipartisan legislation, introduced by Representatives Stearns (R-FL) and Towns (D-NY), will improve access to the existing Accelerated Approval pathway for patients with life threatening ultra-rare diseases with the added attribute of promoting private investment in new biotechnology companies and job growth in the United States.

More than 25 years ago, Congress enacted the Orphan Drug Act, very successful legislation that created incentives for the development of treatments for diseases that affect no more than 200,000 patients in the United States. Additionally, Congress created the Fast Track process to speed up the development of products intended to treat serious or life-threatening diseases with unmet medical needs. The Fast Track process permits expedited review under the Accelerated Approval regulation, which allows the use of surrogate endpoints (or biomarkers) to demonstrate the efficacy of a drug instead of a clinical endpoint.

Accelerated Approval was created during the AIDS crisis to allow for the use of a biomarker to spur the development of treatments for HIV/AIDS, which turned the disease from a death sentence to a managed chronic disease. Over the years, the FDA has gained significant experience approving innovative therapies with well-established safeguards through Accelerated Approval. However, there remain very few approved treatments for ultra-rare diseases via the Accelerated Approval pathway despite the significant unmet need.

The small number of patients with a particular ultra-rare disease makes it virtually impossible under the current FDA requirements to qualify novel surrogate endpoints, since clinical data are required. The absence of prior clinical data makes it nearly impossible for low prevalence orphan treatments to access the Accelerated Approval pathway. However, ultra-rare diseases typically arise from a single gene defect or error in a biochemical pathway, allowing a surrogate endpoint to demonstrate a therapy's efficacy with great scientific confidence. This legislation will empower the FDA to consider the full scope of existing scientific data when reviewing surrogate endpoints for use under the Accelerated Approval pathway.

This legislation would only apply if the FDA designates a drug as both a Fast Track and an orphan product for use against a disease affecting a small number of patients in the United States. HR 3737 will create not just hope but a real opportunity for the development of treatments for individuals with ultra-rare diseases. By allowing better access to the Accelerated Approval process to address rare diseases, we will unlock the existing science and spur the development of lifesaving treatments for millions of patients. The potential to develop these treatments will ignite private investment in small biotech firms bringing high paying jobs to thousands of Americans.

Thank you for your leadership to help bring treatments to patients with ultra rare diseases.

Sincerely,

Abigail Alliance for Better Access to Developmental Drugs
Addi & Cassi Fund
Advocacy for Patients with Chronic Illness, Inc
Alliance Against Alveolar Soft Part Sarcoma
American Behcet's Disease Association
American Porphyria Foundation
Amschwand Sarcoma Cancer Foundation
BDSRA (Batten Disease Support and Research Association)
Beyond Batten Disease Foundation
Blake's Purpose Foundation
Breakthrough Cancer Coalition
Canadian PKU & Allied Disorders
Center for Orphan Disease Research and Therapy, University of Pennsylvania
CheckOrphan
Children's Medical Research Foundation, Inc.
Cooley's Anemia Foundation
Cryoglobulinemia Vasculitis Education & Awareness Organization
Dani's Foundation
Dravet Syndrome Foundation
Drew's Hope Research Foundation
EveryLife Foundation for Rare Diseases
Fabry Support & Information Group
Fight for Nicolas
Georgia PKU Connect
GIST Cancer Awareness Foundation
Hannah's Hope Fund
Hereditary Disease Circle
Histiocytosis Association
Hope4Bridget Foundation
Hypertrophic Cardiomyopathy Association - HCMA
I Have IHH
iBelieve Foundation
IPH-NET, Idiopathic Pulmonary Hemosiderosis
ISRMD (International Society for Mannosidosis and Related Diseases)

Jacob's Cure
Jain Foundation
Jonah's Just Begun-Foundation to Cure Sanfilippo Inc.
Kawasaki Disease Fund
Kids V Cancer
Klippel-Trenaunay Support Group
Kurt+Peter Foundation
LGBT Cancer Project
LGMD2I Research Fund
Life Raft Group
Lymphangiomas & Gorham's Disease Alliance
Lymphatic Malformation Institute
MADISONS Foundation
MAGIC Foundation
Malecare Cancer Support
Manton Center for Orphan Disease Research
MarbleRoad
Mary Payton's Miracle Foundation
Midwest Asian Health Association (MAHA)
MLD Foundation
Moebius Syndrome Foundation
MPD Support
National Adrenal Diseases Foundation (NADF)
National Ataxia Foundation
National Gaucher Foundation
National MPS Society
National Organization Against Rare Cancers
National PKU Alliance
National Tay-Sachs & Allied Diseases Association
New Hope Research Foundation
NextGEN Policy
NKH International Family Network
Noah's Hope - Batten disease research fund
NOMID Alliance
Our Promise to Nicholas Foundation
Pachyonychia Congenita Project
Paget Foundation for Paget's Disease of Bone and Related Disorders
Partnership for Cures
Patient Power
Periodic Paralysis Association
Potentials Foundation
PrimordialDwarfism.com
RARE Project
Russell-Silver Syndrome Support
Ryan Foundation for MPS Children
Sanfilippo Foundation for Children

Sarcoma Alliance
Sarcoma Foundation of America
Save Babies Through Screening Foundation
Solving Kids' Cancer
Stevens-Johnson Syndrome Foundation
Taylor's Tale: Fighting Batten Disease
Team Sanfilippo Foundation
The Many Faces of Moebius Syndrome
The XLH Network, Inc.
Transverse Myelitis Association
United Pompe Foundation