

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

January 4, 2012

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  
ALD Foundation  
Alliance Against Alveolar Soft Part Sarcoma  
American Behcet's Disease Association  
American Childhood Cancer Organization  
American College of Medical Genetics  
American Porphyria Foundation  
Amschwand Sarcoma Cancer Foundation  
Ara Parseghian Medical Research Foundation  
Autoimmunity Research Foundation  
BDSRA (Batten Disease Support and Research Association)  
Beyond Batten Disease Foundation  
Blake's Purpose Foundation  
Breakthrough Cancer Coalition  
Californians 4 Cures  
Californians4cures  
Campaign Urging Research for Eosinophilia Disease (CURED)  
Canadian PKU & Allied Disorders  
CARES Foundation  
Celiac Sprue Association  
Center for Orphan Disease Research and Therapy, University of Pennsylvania  
CheckOrphan  
Children's Medical Research Foundation, Inc.  
Children's PKU Network  
Chronic Granulomatous Disease Association  
Cooley's Anemia Foundation  
Cryoglobulinemia Vasculitis Education & Awareness Organization  
CurePSP Foundation  
Dana's Angels Research Trust  
Dani's Foundation  
Dravet Syndrome Foundation  
Dravet.org

Drew's Hope Research Foundation  
EveryLife Foundation for Rare Diseases  
Fabry Support & Information Group  
Fight for Nicolas  
FMD Chat  
Gene Spotlight Inc.  
Georgia PKU Connect  
GIST Cancer Awareness Foundation  
Hannah's Hope Fund  
Hereditary Disease Circle  
Hide & Seek Foundation  
Histiocytosis Association  
Hope4Bridget Foundation  
Hunter's Hope Foundation  
Hypertrophic Cardiomyopathy Association - HCMA  
I Have IIIH  
iBelieve Foundation  
In Need of Diagnosis, INC  
International Dravet Syndrome Epilepsy Action (IDEA) League  
IPH-NET, Idiopathic Pulmonary Hemosiderosis  
ISRMD (International Society for Mannosidosis and Related Diseases)  
Jacob's Cure  
Jain Foundation  
Janine's Sarcoidosis Outreach Foundation  
JLK Sanfilippo Research Foundation  
Jonah's Just Begun-Foundation to Cure Sanfilippo Inc.  
Kawasaki Disease Fund  
Kids V Cancer  
Klippel-Trenaunay Support Group  
Kurt+Peter Foundation  
Let Them Be Little X2  
LGBT Cancer Project  
LGMD2I Research Fund  
Life Raft Group  
Lymphangiomatosis & 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 Eczema Association  
National Gaucher Foundation  
National MPS Society  
National Niemann-Pick Disease Foundation  
National Organization Against Rare Cancers  
National PKU Alliance  
National Tay-Sachs & Allied Diseases Association  
Neuromuscular Disease Foundation  
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  
Race For Adam Foundation  
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  
Sephardic Health Organization for Referral & Education - SHORE  
Shwachman Diamond Syndrome Foundation  
Solving Kids' Cancer  
Stevens-Johnson Syndrome Foundation  
Taylor's Tale: Fighting Batten Disease  
Team Sanfilippo Foundation  
The Global Foundation for Peroxisomal Disorders  
The Many Faces of Moebius Syndrome  
The XLH Network, Inc.  
Transverse Myelitis Association  
United Mashai Jewish Community of America - UMJCA  
United Mitochondrial Disease Foundation  
United Pompe Foundation  
Wiscosin Sarcoma Group