May 6, 2010

Honorable David Obey, Chairman Honorable Rosa DeLauro, Chairwoman

Honorable Jerry Lewis, Ranking Member Honorable Jack Kingston, Ranking Member

Committee on Appropriations Ag-Rural Development-FDA Subcommittee

United States House of Representatives Committee on Appropriations

Washington, DC 20510 United States House of Representatives

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Dear Chairman Obey, Chairwoman DeLauro, and Ranking Members Lewis and Obey:

As leaders in the rare disease community, we are writing to you today in support of providing additional funding to the Food and Drug Administration (FDA) to build the human and scientific resources necessary to create a more specialized drug review by experts who understand rare diseases. **We specifically request $10 million in the fiscal year 2011 Ag-Rural Development-FDA Appropriations bill directing the FDA to establish a new review division for Biochemical and Genetic Diseases within the Center for Drug Evaluation Research, Office of New Drugs.**

***Rare diseases need more treatments.*** There are more than ***7,000 rare disorders*** that together affect over ***25 million Americans*** and their families. The Orphan Drug Act (enacted in 1983) encourages pharmaceutical companies to develop drugs for diseases that have relatively small patient populations; however, in the last 25 years less than 5% of these disorders have had a treatment approved by the FDA. Treatments for many of these diseases may never be developed because of a lack of investment due to a challenging regulatory environment. Improving the FDA regulatory process will help these patients get treatments.

***A dedicated FDA review division will improve the development path.*** Providing funding for a new review division for biochemical and genetic diseases will help create a more specialized drug review by experts who understand complex genetic diseases. The new division will focus on specific diseases needing increased expertise and provide assistance, as needed to other review offices with rare disease issues. The new division will facilitate collaboration with the Office of Orphan Product Development and improve the overall academic environment and links with the National Institutes of Health. In addition, this new group will create new guidance and policy for FDA that could make the Accelerated Approval pathway available for rare diseases. The improved regulatory environment will encourage more treatment development and allow more patients with rare disorders to finally have access to safe, effective treatments.

***Improved FDA regulation will drive more U.S. Biotech job creation.*** The creation of this new division will also provide a strong signal to the biotechnology industry and investors that the FDA is working to improve the regulatory path for thousands of rare disorders. This new division, combined with new policy, will drive more investment in early stage biotech companies focused on rare diseases while at the same time producing a positive impact in local communities by creating new U.S.-based biotechnology jobs.

With your support, we can expedite the approval process, expand access to new and effective treatments, and create new quality U.S. jobs.

Sincerely,

Kakkis EveryLife Foundation, CA

The Manton Center for Orphan Disease Research, Children's Hospital Boston, MA

Society for Inherited Metabolic Disorders, OR

Children's Rare Disease Network, CA

R.A.R.E. (Rare Disease Advocacy, Research & Education) Project, CA

National MPS Society, NC

John F. & Aileen A. Crowley Foundation, NJ

Noah's Hope--Batten Disease Fund, IL

National PKU Alliance, WI

Addi and Cassi Fund, NV

The Ryan Foundation for MPS Children, TX

Fibrous Dysplasia Foundation, DC

Fight for Nicolas, FL

National Foundation for Ectodermal Dysplasias, IL

Foundation for Sarcoidosis Research, MD

Kennedy's Disease Association, CA

National Eczema Association, CA

The Lymphatic Research Foundation, NY

Children’s PKU Network, CA

National Alopecia Areata Foundation, CA

Children's Tumor Foundation, NY

CureDuchenne Muscular Dystrophy, CA

Hide & Seek Foundation for Lysosomal Disease Research, CA

Institute for Families, CA

International Pemphigus and Pemphigoid Foundation, CA

ISMRD, International Society for Mucolipidosis and Related Disorders, CA

Sarcoma Alliance, CA

Advocacy for Patients with Chronic Illness, Inc., CT

Dravet Syndrome Foundation, CT

Reflex Sympathetic Dystrophy Syndrome Association, CT

Myeloproliferative Disease Support, FL

Campaign Urging Research for Eosinophilia Disease (CURED), IL

International Dravet Syndrome Epilepsy Action (IDEA) League, MD

Magic Foundation, IL

National Tay-Sachs & Allied Diseases Association, Inc., MA

VHL Family Alliance, MA

National Organization Against Rare Cancers, MD

Sarcoma Foundation of America, MD

FOD (Fatty Oxidation Disorders) Family Support Group, MI

Fabry Support & Information Group, MO

CARES Foundation, NJ

American Behcets Disease Association, NY

Alpha-1 Association, FL

Hannah's Hope Fund, NY

Liddy Shriver Sarcoma Initiative, NY

Solving Kids' Cancer, NY

Batten Disease Support and Research Association, OH

MLD Foundation, OR

Abigail Alliance for Better Access to Investigational Drugs, VA

Transverse Myelitis Association, WA

Kids with Heart, National Association for Children's Heart Disorders, WI

Tuberous Sclerosis Alliance, MD

Nathan’s Battle Foundation, IN

Hope 4 Bridget Foundation, IL

Hayden's Batten Disease Foundation Inc., WI

Beyond Batten Disease Foundation, TX

Drew’s Hope Research Foundation, PA

Lysosomal Diseases New Zealand

Foundation for Nager and Millers Syndromes, WA

Intractable Childhood Epilepsy Alliance, NC

National Niemann-Pick Disease Foundation, WI

Association of Gastrointestinal Motility Disorders, Inc., MA

NKH International Family Network, NY

Coalition for Pulmonary Fibrosis, CA

APS Foundation of America, Inc, WI

Iowa Ataxia Support Group, IA

Pediatric Hydrocephalus Foundation, Inc., NJ

Ara Parseghian Medical Research Foundation, AZ

DEBRA - Dystrophic Epidermolysis Bullosa Research Association of America, NY

SADS Sudden Arrhythmia Death Cardiac Syndromes Foundation, UT

 CFC International, NY

Parkinson's Action Network, DC

Jain Foundation, WA

National Gaucher Foundation, GA

United Pompe Foundation, CA

Kids V Cancer, NY

Children's Cardiomyopathy Foundation, Inc., NJ

Cooleys Anemia Foundation, NY

Dandy-Walker Alliance, Inc, MD

Invisible Diseases, CA

The Myositis Association, VA

Partnership for Compassionate Use Therapies, CA

Georgia PKU Connect, GA

LGS Foundation- New York, NY

Macular Degeneration Support, MO

Russell Silver Syndrome Advocacy and Support, VA

Pediatric OMS Research Fund, IL

CNY Bleeding Disorders Association, NY

Adult Congenital Heart Association, PA

Mary Payton’s Miracle Foundation, LA

Macular Degeneration Support, MO

AMDA (Acid Maltase Deficiency Association), TX