December 7, 2013

The Honorable Leonard Lance
133 Cannon House Office Building
Washington, DC 20515

The Honorable Anna G. Eshoo
241 Cannon House Office Building
Washington, DC 20515

Dear Representatives Lance and Eshoo:

As organizations committed to advancing patient access to innovative technologies and therapies that can diagnose, treat and cure rare diseases, we are writing to offer our strong support of H.R. 2725, the *Food and Drug Administration Safety Over Sequestration ("S.O.S.") Act*. This legislation will exempt the FDA user fees – paid by pharmaceutical, biologic, medical device, and other manufacturers to help support the FDA's review of new drugs, biologics, devices and diagnostics – from being sequestered in fiscal year 2014 and beyond.

Established by Congress more than 20 years ago, user fees have helped provide the agency the resources it needs to conduct thorough reviews in the most expeditious manner possible, helping speed life-saving medications to patients as a result. Unfortunately, as a result of the sequester, FDA cannot access a significant portion of the resources that it uses to review candidate therapies.

Last year, passage of the *FDA Safety and Innovation Act* (FDASIA) renewed FDA's authority to collect user fees and provided much-needed improvements to regulatory review processes. In fact, industry agreed to pay *increased* user fees in order to facilitate the needed system and process improvements under the renewed FDA user fee law. One such key improvement was an updating of FDA's authorities to help speed the development and availability of drugs that treat serious diseases, including the creation of a new "Breakthrough Therapy" designation allowing FDA to assist drug manufacturers to expedite the development and review of new drugs with preliminary clinical evidence that indicates the drug may offer a substantial improvement over available therapies for patients with serious or life-threatening diseases.

In recent months, FDA granted Fast Track, Priority Review or Breakthrough Therapy designations to potential therapies for Alzheimer's disease, metastatic lung cancer, muscular dystrophy, metastatic melanoma, hepatitis C, acute myeloid leukemia (AML), and chronic lymphocytic leukemia (CLL). In addition to these exciting advancements, a number of highly encouraging therapeutic targets remain in the pipeline, and it is critical that FDA be equipped with every available tool to expedite the review of rare disease candidate therapies once they are submitted to the agency.

However, under the terms of the sequester, industry-paid user fees – which account for about 60-65% of FDA's budget for review activities related to drugs and biologics, and 1/3 of FDA's budget for review activities related to devices and diagnostics – are subjected to the across-the-board cuts. This action is estimated to cost FDA about $85 million in valuable user fee resources in fiscal year 2013 alone and even greater amounts in the years to come if not addressed.

The bottom line: the user fees are private monies – not federal taxpayer dollars – and should not be subject to sequestration.

While lawmakers are working to identify a solution for releasing the $85 million sequestered in FY13, swift enactment of H.R. 2725 is critical for ensuring FDA has full access to these precious non-government fees paid to the agency to ensure potential therapies can be appropriately reviewed and made available to patients as quickly as possible.

Should you have any questions, please contact Julia Jenkins with EveryLife Foundation for Rare Diseases at jjenkins@everylifefoundation.org or 202-803-6047.

Thank you again for your important leadership of H.R. 2725, the *FDA S.O.S. Act*.

Sincerely,

Abigail Alliance for Better Access to Developmental Drugs
AIDS Institute
Alliance for Aging Research
Alliance for Patient Access
ALS Association
Alstrom Angels
American Association for Cancer Research (AACR)
American Association of Neurological Surgeons
American Autoimmune Related Diseases Association
American Behcet's Disease Association (ABDA)
American Brain Coalition
American Cancer Society Cancer Action Network, Inc.(ACS CAN)
American Childhood Cancer Organization
American Epilepsy Society
American Porphyria Foundation
American Society for Reproductive Medicine
Animal Health Institute
Aplastic Anemia & MDS International Foundation
ARPKD/CHF Alliance
Association for Frontotemporal Degeneration (AFTD)
Association for Glycogen Storage Disease
Association of Clinical Research Organizations
Batten Disease Support and Research Association
Ben's Friends
Beyond Batten Disease Foundation
CADASIL Association
CHARGE Syndrome Foundation
Charley’s Fund
Children’s Medical Research Foundation, Inc.
Coalition for Pulmonary Fibrosis
Colon Cancer Alliance
Colon Cancer Alliance for Research & Education for Lynch Syndrome
Community Access National Network (CANN)
Community Health Charities of America
Congress of Neurological Surgeons
Cooley’s Anemia Foundation
Cure AHC – Alternating Hemiplegia of Childhood
CureDuchenne
Cushing Support and Research Foundation
Erythromelalgia Association
Everylife Foundation for Rare Diseases
Fabry Support and Information Group
FasterCures
Fight Colorectal Cancer
FORCE: Facing Our Risk of Cancer Empowered

Foundation Fighting Blindness
Foundation for Prader-Willi Research
Gavyn's Voice for Dandy Walker
Gene Spotlight, Inc.
Genetic Alliance
Global Genes | RARE Project
Global Healthy Living Foundation
GT23 Foundation
Gwendolyn Strong Foundation
Hannah's Hope Fund
Hereditary Neuropathy Foundation
Hopes & Dreams for ALS
Hunter Syndrome Foundation
Hydrocephalus Association
International Advocate for Glycoprotein Storage Diseases
International Essential Tremor Foundation
International Myeloma Foundation
International Pemphigus and Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
IPH-NET
Jackson Gabriel Silver Foundation
Jett Foundation
Jonah's Just Begun-Foundation to Cure Sanfilippo Inc
Juvenile Diabetes Research Foundation (JDRF)
Kids With Heart National Assn for Children's Heart Disorders, Inc
Klippel Feil Syndrome Alliance (kfs alliance)
Kortney Rose Foundation
Let Them Be Little X2 Foundation
Leukemia & Lymphoma Society
Liddy Shriver Sarcoma Initiative
Little Miss Hannah Foundation
Lymphatic Education & Research Network (LE&RN)
Macular Degeneration Support Inc.
Mastocytosis Society
M-CM Network
Melanoma Research Alliance
Midwest Asian Health Association
MLD Foundation
Muscular Dystrophy Association
Myelin Project
National Alliance on Mental Illness
National Down Syndrome Society
National Family Association for Deaf-Blind
National Fragile X Foundation
National Health Council
National Hemophilia Foundation
National Hispanic Medical Association
National Kidney Foundation
National MPS Society
National Multiple Sclerosis Society
National PKU Alliance
National Tay-Sachs & Allied Diseases Association, Inc. (NTSAD)
National Venture Capital Association
New Zealand Lysosomal Storage Diseases Support Group
NKH International Family Network
Noah's Hope: Batten Disease Research Fund
NOMID Alliance
Non-ketotic Hyperglycinemia (NKH) Family Network
Ovarian Cancer National Alliance
Parent Project Muscular Dystrophy
Phelan-McDermid Syndrome Foundation
PKD Foundation
PMG Awareness Organization
Prevent Cancer
PXE International
Rare Disease United Foundation
RASopathies Network USA
Research!America
RetireSafe
Ryan Foundation, Inc.
Sanfilippo Foundation for Children
Sarcoma Alliance
Sarcoma Foundation of America
SMA Foundation
St. Baldrick's Foundation
Taylor's Tale
Team Sanfilippo Foundation
The Addi & Cassi Fund
United Leukodystrophy Foundation
Veterans Health Council