February 27, 2017

President Donald J. Trump
1600 Pennsylvania Avenue NW
Washington, DC 20500

Dear President Trump,

The undersigned 200 patient organizations write today about the challenges facing rare disease patients in America and the vital role that federal agencies play in helping to accelerate the research, development, review, and approval of treatments. These agencies must have the ability to hire and retain personnel in order to accomplish their respective missions and to achieve the broader goal of treating and curing diseases.

It is estimated that 1 in 10 individuals has a rare disease, defined as a condition affecting 200,000 or fewer patients in America. When combined, these diseases are not “rare” as more than 30 million Americans have a rare disease. There may be as many as 8,000 rare diseases, but unfortunately the vast majority (95%) do not yet have a treatment approved by the Food and Drug Administration (FDA). Many rare disease patients go years without receiving an accurate diagnosis, during which time their disease may progress unchecked.

However, the Orphan Drug Act of 1983 spurred substantial progress in the development of new treatments. This legislation helped enable the creation of an orphan drug industry, which is undergirded by critical investments in basic and applied research through the National Institutes of Health (NIH).

NIH funding supports research projects that strengthen the biomedical infrastructure across America while driving forward science that can lead to new therapies and cures. The Clinical Center at NIH, the world’s premier research hospital, conducts life-saving clinical trials and works with rare disease patients who have run out of options. The FDA also plays a key role by providing a thorough review of innovative medicines and devices that may benefit rare disease patients.

In order to ensure America’s continued global leadership in health innovation and biomedical discovery, the hiring of key personnel for these agencies must keep pace with the latest science and legislation such as the 21st Century Cures Act and upcoming reauthorization of the Prescription Drug and User Fee Act (PDUFA). It is critical to empower the FDA and NIH to bring on the best and brightest staff, so that our nation can ensure that rare disease patients have an opportunity to obtain safe and effective FDA-approved treatments.

We thank you for your service to the nation and look forward to working with your administration on strengthening biomedical innovation for the millions of Americans with a rare disease.

Sincerely,

EveryLife Foundation for Rare Diseases
NGLY1.org

National Lymphedema Network
Fibrous Dysplasia Foundation
Bridge The Gap Syngap Education & Research Foundation
United Leukodystrophy Foundation
CDG CARE
Cardio-Facio-Cutaneous International
Pulmonary Fibrosis Advocates
Noonan Syndrome Foundation
GCAF GIST Cancer Awareness Foundation
Rare and undiagnosed network
Little Miss Hannah Foundation
UCI HUNTINGTON'S DISEASE - COMMUNITY ADVOCACY, RESEARCH & EDUCATION
Noonan Syndrome Foundation
WideTrial
NTM Info & Research
Vasculitis Foundation
Guardian Hands Foundation
Amyloidosis Research Consortium
Noah's Hope
Cure AHC
National MPS Society
RASopathies Network USA
Jonah's Just Begun
AIU
ADCYS.org
Rare Disease on The Mighty
The Atypical HUS Foundation
Lipodystrophy United
American Behcet's Disease Association
The Cambria Lord Foundation
International Cystinuria Foundation
Miracle for Madison and Friends
Sarcoidosis of Long Island
CARES Foundation, Inc.
PPMD
Ovm
Global genes
Hackensack University Medical Center
International Cystinuria Foundation
BORN A HERO
cureCADASIL
Seal The Deal Real Estate
Global Genes
Pmg Awareness Organization
Jill Tullman & Associates

Marilyn's Fight For Life KICK
SARCOIDOSIS Campaign
Kids Conquering Sickle Cell Disease Foundation
SCDAA
Kids Conquering Sickle Cell Disease Foundation
M-CM Network
Organization for rare diseases india
Usher Syndrome Coalition
Oklahoma RSD CRPS
International Waldenstrom
Macroglobulinemia Foundation
Histiocytosis Association
FACIAL network (for children with craniofacial microsomia)
VHL Alliance
BioPontins Alliance for Rare Diseases
SAHM
HAE
Children's PKU Network
debra of America
Bluefield Project to Cure FTD
IWMF
CJD Aware!
AGMD
ARMS. (Sarcoma group)
PCD Foundation
Organic Acidemia Association
Oxalosis & Hyperoxaluria Foundation
Tarlov cysts disease foundation
Klippel-Feil Syndrome - rare disease patient advocate
Titin Related Muscle and Heart Disorders
National Organization for Albinism and Hypopigmentation
SWLA Sarcoidosis Support Group
SOAR-NPC
Ntsad
The Mastocytosis Society, Inc.
American Partnership for Eosinophilic Disorders
Lupus and Allied Diseases Association, Inc.
Prayers for Elijah
Relapsing Polychondritis Awareness and Support Foundation, Inc
Porphyria Foundation  
Connecting Families Urea Cycle Run for ALD  
CCHS Network  
NTSAD  
American Porphyria Foundation  
Dante's Hope  
Nicholas Volker One In A Billion Foundation  
IPPA  
Sickle cell  
GCAF GIST Cancer Awareness Foundation  
International Pemphigus & Pemphigoid Foundation  
Noah's Hope  
Polycystic Kidney Disease Foundation  
NBIA Disorders Association  
Helping Hands for GAND  
DADA2 Foundation  
UCI HUNTINTON'S DISEASE- COMMUNITY ADVOCACY, RESEARCH & EDUCATION  
NATIONAL PKU ALLIANCE AND MICHIGAN PKU  
Hereditary Coproporphyria patient CureAHC  
Rare Genomics Institute  
Solve ME/CFS Initiative  
Wilson Disease Association  
International Waldenstrom's Macroglobulinemia Foundation  
RUN and undiagnosed network  
International Pemphigus and Pemphigoid Foundation  
National Urea Cycle Disorder Foundation  
Brian's Hope  
The Nicholas Conor Institute for Pediatric Cancer  
Moebius Syndrome Foundation  
Epilepsy Foundation of Greater Chicago  
Association for Creatine Deficiencies Cure CMD  
The International Cystinuria Foundation  
Jack-Whelan, Inc. Research Advocacy  
International Cystinuria Foundation  
ADNP Kids Research Foundation  
American Porphyria Foundation  
Chloe’s Fight Rare Disease Foundation  
Cure CMD  
IWMF-VA  
Batten Disease Support and Research Association  
Sacroidosis  
Team Spiderman for Micah Man  
Sacroidosis  
PWN4PWN  
ADNP Kids Research Foundation  
FMDSA.org  
Childhood Cancer Awareness Group of Coffee County  
The Desmoid Tumor Research Foundation  
Choroideremia Reseach Foundation  
K-T Support Group  
Mucolipidosis Type IV Foundation  
PBCers Organization  
Foundation for Prader-Willi Research  
VHL.ORG  
Autoinflammatory Alliance  
The Cholangiocarcinoma Foundation  
Klippel feil syndrome  
Cures Within Reach  
Amyloidosis Research Consortium  
Bohring-Opitz Syndrome Foundation  
Abigail Alliance for Better Access to Developmental Drugs  
Batten Disease Support and Research Association  
American Behcet's Disease Association  
Let's Breathe Sarcoidosis Support  
PTEN World  
National Tay-Sachs & Allied Diseases Association  
National Adrenal Diseases Foundation  
SilvermanSoldiers, Inc.  
CADASIL-Together we Can  
Chase After a Cure  
Amyloidosis Research Consortium  
Narcolepsy Awareness  
Alpha-1 Foundation  
American Institute for Medical & Bio. Engineering  
National Niemann-Pick Disease Foundation, Inc.