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 HUNTINTON'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
ADCY5.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 Centet
International Cystineuria 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 Associaton
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
Sarcoidosis
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.

The Progeria Research Foundation
Bridge Clinical Solutions
Klippel Feil Syndrome Freedom
Dyskeratosis Congenita Outreach
PROS Foundation
National PKU News
MLD Foundation
Cure Sanfilippo Foundation
Friedreich's Ataxia Research Alliance (FARA)
National MPS Society
Li-Fraumeni Syndrome Association (LFS Association / LFSA)
National Organization for Rare Disorders (NORD)
Phoenix Fox Foundation
Sickle Cell Foundation of MN
jesscah sickle cell foundation
ADNP Kids Research Foundation
EmesARMY |fighting childhood blindness
LCA hope Alliance
Lipodystrophy United
Hannah's Hope Fund
PKD Foundation
Stiff Person Syndrome
FMDSA
IWMF Waldesnstroms
International WAGR Syndrome Association
Innovatients, LLC
Mo Songs for Kerry.org
Cystic Fibrosis Research, Inc. (CFRI)
FSH Society Inc.
United Mitochondrial Disease Foundation
Lupus Foundation of America
Roses for Sarcoidosis
KIF1A.org
Dysautonomia Advocacy Foundation
EDSers United
CVS.Speaks
GNE Myopathy International