February 27, 2017

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

Dear President Trump,

The undersigned 147 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,

Abby Grace Foundation (Dallas, NC)  ADCY5.org (La Jolla, CA)  
Abigail Alliance for Better Access to  ADNP Kids Research Foundation (Portland,  
Developmental Drugs (Lorton, VA)    OR)  
Achalasia Awareness Organization    Adrenal Insufficiency United (Astoria, OR)  
(Brownsville, TX)  Alpha-1 Foundation (Coral Gables, FL)
American Behcet's Disease Association
(Rochester, MI)
American Institute for Medical and
Biological Engineering (Washington, DC)
American Partnership for Eosinophilic Disorders (GA)
American Porphyria Foundation (Orlando, FL)
Amyloidosis Research Consortium (Newton, MA)
Association for Creatine Deficiencies
(Carlsbad, CA)
Association of Gastrointestinal Motility Disorders (Bedford, NY)
Atypical HUS Foundation (Grand Prairie, TX)
Autoinflammatory Alliance (San Diego, CA)
Batten Disease Support and Research Association (Columbus, OH)
BioPonitns Alliance for Rare Diseases (Raleigh, NC)
Bluefield Project to Cure FTD (San Francisco, CA)
Bohring-Opitz Syndrome Foundation (Orlando, FL)
BORN A HERO (Bothell, WA)
Brian's Hope (Branford, CT)
Bridge the Gap Syngap Education and Research Foundation (Cypress, TX)
CADASIL-Together We Can (Canton, GA)
Cambria Lord Foundation (Overland Park, KS)
Cardio-Facio-Cutaneous International (Delmar, NY)
CARES Foundation, Inc. (Union, NJ)
CCHS Network (Oneonta, NY)
CDG CARE (Colorado Springs, CO)
Chase After a Cure (Charleston, SC)
Childhood Cancer Awareness Group of Coffee County (Douglas, GA)
Children's PKU Network (Encinitas, CA)
Chloe's Fight Rare Disease Foundation (Hopkins, MN)
Cholangiocarcinoma Foundation (Billings, MT)
Choroideremia Research Foundation (New Orleans, LA)
CJD Aware! (New Orleans, LA)
Connecting Families Urea Cycle (Murrieta, CA)
Cure CMD (Torrance, CA)
Cure Sanfilippo Foundation (Sedro Woolley, WA)
CureAHC (Raleigh, NC)
cureCADASIL (Plainsboro, NJ)
Cures Within Reach (Skokie, IL)
CVS.Speaks (Deerfield Beach, FL)
Cystic Fibrosis Research, Inc. (Palo Alto, CA)
DADA2 Foundation (TN)
Dante's Hope (VA)
debra of America (New York, NY)
Desmoid Tumor Research Foundation (Suffern, NY)
Dysautonomia Advocacy Foundation (Escondido, CA)
Dysautonomia International (Aurora, CO)
Dyskeratosis Congenita Outreach (New York, NY)
EDSers United (Plainsboro, NJ)
EmesARMY |fighting childhood blindness (Manassas, VA)
Epilepsy Foundation of Greater Chicago (Chicago, IL)
EveryLife Foundation for Rare Diseases (Novato, CA)
FACIAL Network (Seattle, WA)
Fibromuscular Dysplasia Society of America (Cleveland, OH)
Fibrous Dysplasia Foundation (Silver Spring, MD)
Foundation for Prader-Willi Research (Walnut, CA)
Friedreich's Ataxia Research Alliance (Downingtown, PA)
FSH Society Inc. (Olney, MD)
GIST Cancer Awareness Foundation (Staten Island, NY)
Global Genes (Newport Beach, CA)
Guardian Hands Foundation (Hialeah, FL)
Hannah's Hope Fund (Rexford, NY)
Helping Hands for GAND (Niceville, FL)
Histiocytosis Association (Pitman, NJ)
International Cystinuria Foundation (Panama City, FL)
International Pemphigus & Pemphigoid Foundation (Sacramento, CA)
International WAGR Syndrome Association (San Antonio, TX)
International Waldenstrom Macroglobulinemia Foundation
(Fairfax, VA)
Jonah's Just Begun (Levittown, NY)
Kids Conquering Sickle Cell Disease Foundation (Orlando, FL)
KIF1A.org (New York, NY)
Klippel Feil Syndrome Freedom (Belvedere, IL)
K-T Support Group (Milford, OH)
LCA Hope Alliance (Manassas, VA)
Let's Breathe Sarcoidosis Support (Zion, IL)
Lipodystrophy United (Los Lunas, NM)
Little Miss Hannah Foundation (Henderson, NV)
Lupus and Allied Diseases Association, Inc. (Verona, NY)
Lupus Foundation of America (Washington, DC)
Marilyn's Fight for Life KICK SARCOIDOSIS Campaign (New Castle, DE)
Mastocytosis Society, Inc. (Sterling, MA)
M-CM Network (Chatham, NY)
Miracle for Madison and Friends (Dublin, OH)
MLD Foundation (West Linn, OR)
Mo Songs for Kerry.org (Athens, PA)
Moebius Syndrome Foundation
Mucolipidosis Type IV Foundation (Atlanta, GA)
National Adrenal Diseases Foundation (Boston, MA)
National Lymphedema Network (Berkeley, CA)
National MPS Society (Durham, NC)
National Niemann-Pick Disease Foundation, Inc. (Fort Atkinson, WI)
National Organization for Albinism and Hypopigmentation (East Hampstead, NH)
National Organization for Rare Disorders (Washington, DC)
National PKU Alliance (Tomahawk, WI)
National Tay-Sachs & Allied Diseases Association (Boston, MA)
National Urea Cycle Disorder Foundation (Pasadena, CA)
NBIA Disorders Association (El Cajon, CA)
NGLY1.org (Salt Lake City, UT)
Nicholas Volker One In A Billion Foundation (Madison, WI)
Noah's Hope (Downers Grove, IL)
Noonan Syndrome Foundation (Aiken, SC)
Oklahoma RSD CRPS (Mustang, OK)
Organic Acidemia Association (Golden Valley, MN)
Oxalosis & Hyperoxaluria Foundation (New York, NY)
Parent Project Muscular Dystrophy (Hackensack, NJ)
PBCers Organization (Pearland, TX)
PCD Foundation (Hilton Head, SC)
Phoenix Fox Foundation (Gainesville, FL)
PKD Foundation (Kansas City, MO)
PMG Awareness Organization (Denver, CO)
Polycystic Kidney Disease Foundation (Kansas City, MO)
Porphyria Foundation (Houston, TX)
Prayers for Elijah (Concord, NC)
PROS Foundation (San Diego, CA)
PTEN World (West Roxbury, MA)
Pulmonary Fibrosis Advocates (Wayzata, MN)
PWN4PWN 9 (Tampa, FL)
Rare and Undiagnosed Network (Los Angeles, CA)
RASopathies Network USA (Williamstown, NJ)
Relapsing Polychondritis Awareness and Support Foundation, Inc. (Annandale, MN)
Roses for Sarcoidosis (Indianapolis, IN)
Run for ALD (Moorstown, NJ)
Sarcoidosis of Long Island (Coram, NY)
SCDAA (Jamaica, NY)
Sickle Cell Foundation of MN (Robbinsdale, MN)
SilvermanSoldiers, Inc. (Marietta, GA)
Society for Adolescent Health and Medicine (Oakbrook Terrace, IL)
Solve ME/CFS Initiative (Los Angeles, CA)
SWLA Sarcoidosis Support Group (Lake Charles, LA)
Tarlov Cysts Disease Foundation (St. George, UT)
Team Spiderman for Micah Man (Raleigh, NC)
The Coalition Against Pediatric Pain (Medfield, MA)
The Progeria Research Foundation (Peabody, MA)
Titin Related Muscle and Heart Disorders (Pine Brook, NJ)
United Leukodystrophy Foundation (DeKalb, IL)
United Mitochondrial Disease Foundation (Pittsburgh, PA)
Usher Syndrome Coalition (Maynard, MA)
Vasculitis Foundation (Fort Worth, TX)
VHL Alliance (Boston, MA)
VHL.ORG (Utica, MI)
VWM Families Foundation (Greenwich, CT)
Wilson Disease Association (New York, NY)
Wishes for Elliot: Advancing SCN8A Research (Washington, DC)