April 18, 2018

The Honorable Andre Carson
2135 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Ryan Costello
326 Cannon House Office Building
Washington, D.C. 20515

Dear Congressmen Carson and Costello:

As organizations representing the millions of men, women, and children living in the United States with rare diseases, we are writing to express our strong support for the *Rare disease Advancement, Research, and Education Act of 2018* (H.R.5115) or the "*RARE Act of 2018*." This legislation, if enacted, has the potential to improve the lives and wellbeing of the 30 million Americans with a rare disease. We thank you for your leadership in introducing this bill.

The *RARE Act* would provide much-needed investment in rare disease research; direct the Centers for Disease Control and Prevention (CDC) to increase its efforts in tracking and quantifying rare diseases; require the Agency for Healthcare Research and Quality (AHRQ) to implement rare disease outreach and awareness campaigns for physicians; and commission additional analysis and recommendations from the National Academy of Sciences on how to accelerate rare disease therapeutic development.

Each of the provisions within the bill would be of substantial benefit to the rare disease patient community. First, this legislation would greatly increase the funding authorization for the Rare Disease Clinical Research Network (RDCRN) administered by the National Center for Advancing Translational Sciences (NCATS) within the National Institutes of Health (NIH). The RDCRN represents one of the most robust and collaborative research networks dedicated to rare diseases in the world. However, the program is only open to a small number of diseases due to funding limitations. This bill would help address this problem by authorizing sorely-needed additional funding.

Second, this legislation would increase the understanding of rare diseases by requiring CDC to create the "National Rare Disease or Condition Surveillance System." This system would track and quantify rare diseases in the U.S. in order to facilitate research, development, and treatment for rare disease patients.

Third, this legislation would require AHRQ to expand its efforts in educating our nation's physicians on rare diseases. The physician community is generally unaware of rare diseases, and significant additional outreach is needed to better equip our physicians to treat the rare disease patient population.

Finally, this bill would require the National Academy of Sciences to update its 2010 report on the state of rare disease therapeutic development and put forward new recommendations within the next several years. Given that 95 percent of rare disease patients are still awaiting their first treatment approved by the Food and Drug Administration (FDA), these recommendations are badly needed.

We thank you again for your proven dedication to the rare disease patient community, and we look forward to working with you to ensure the *RARE Act* is enacted.

Sincerely,

A Twist of Fate-ATS
ADNP Kids Research Foundation
Adrenal Insufficiency United
Adult Polyglucosan Body Disease Research Foundation
Alagille Syndrome Alliance
Alport Syndrome Foundation
ALS Association
American Behcet's Disease Association
American Multiple Endocrine Neoplasia Support
American Partnership for Eosinophilic Disorders (APFED)
Angelman Biomarkers and Outcome Measures Alliance
Aplastic Anemia and MDS International Foundation
APS Type 1 Foundation, Inc.
Association for Creatine Deficiencies
Association of Gastrointestinal Motility Disorders, Inc. (AGMD)
Autoimmune Hepatitis Association
Autoinflammatory Alliance
Batten Disease Support and Research Association
Bridge the Gap - SYNGAP Education and Research Foundation
Charcot-Marie-Tooth Association
Children's PKU Network
Children's Tumor Foundation
Chloe Barnes Rare Disease Foundation
Cholangiocarcinoma Foundation
CMTC-OVM
Congenital Hyperinsulinism International
Cure SMA
CureCMT4J/Talia Duff Foundation
CurePSP
Cutaneous Lymphoma Foundation
DC Outreach, Inc.
Defeat MSA: Defeat Multiple System Atrophy
The Desmoid Tumor Research Foundation
Dravet Syndrome Foundation
Family Caregiver Alliance
Fibrous Dysplasia Foundation
Foundation Fighting Blindness
Foundation for Prader-Willi Research
Friedreich's Ataxia Research Alliance (FARA)
The Global Foundation for Peroxisomal Disorders
Glut1 Deficiency Foundation
The Guthy-Jackson Charitable Foundation
HCU Network America
Hermansky-Pudlak Syndrome Network
Histiocytosis Association

HSAN1E Society
Hydrocephalus Association
The Hyper IgM Foundation
Immune Deficiency Foundation
Indian Organization for Rare Diseases
International Fibrodysplasia Ossificans Progressiva (FOP) Association
International Pemphigus and Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
Jack McGovern Coats' Disease Foundation
The Jansen's Foundation
KIF1A.ORG
LAL Solace
The LAM Foundation
Li-Fraumeni Syndrome Association (LFS Association / LFSA)
Lung Transplant Foundation
Lymphangiomatosis & Gorham's Disease Alliance
M-CM Network
MLD Foundation
Moebius Syndrome Foundation
The Morgan Leary Vaughan Fund, Inc.
The MSA Awareness Shoe
Mucolipidosis Type IV Foundation
Multiple System Atrophy Coalition
Myasthenia Gravis Foundation of America
The Myelin Project
The Myocarditis Foundation
Myotonic Dystrophy Foundation
National Adrenal Diseases Foundation
National Foundation for Ectodermal Dysplasias
National MPS Society
National Organization for Albinism and Hypopigmentation
National Organization for Rare Disorders (NORD)
National PKU News
National Spasmodic Dysphonia Association
National Urea Cycle Disorders Foundation
NBIA Disorders Association
Neurofibromatosis Network
Neurofibromatosis Northeast
NGLY1.org
Oley Foundation
Organic Acidemia Association
Osteogenesis Imperfecta Foundation
Phelan-McDermid Syndrome Foundation
PKD Foundation
Platelet Disorder Support Association

Prader-Willi Syndrome Association (USA)
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association
Rare and Undiagnosed Network (RUN)
RYR-1 Foundation
Shwachman-Diamond Syndrome Foundation
The Snyder-Robinson Foundation
Sofia Sees Hope
Soft Bones, Inc., The U.S. Hypophosphatasia Foundation
Spinal CSF Leak Foundation
SSADH Association
TargetCancer Foundation
Tarlov Cyst Disease Foundation
Team Audrey HLH
The Transverse Myelitis Association
Tuberous Sclerosis Alliance
United Leukodystrophy Foundation
Vasculitis Foundation
VHL Alliance
Wilhelm Foundation - the Undiagnosed
Williams Syndrome Association
The XLH Network, Inc.