Dear Chairman Alexander and Ranking Member Murray,

We write today in strong support of the Orphan Product Extensions Now, Accelerating Cures & Treatments (OPEN ACT), S. 1421. On behalf of 173 patient organizations and the individuals we represent, we wish to add our names in support of this critical legislation, which is a top priority for the rare disease community.

We applaud the OPEN ACT because it will bring hundreds of safe, effective, and affordable medicines to rare disease patients within the next several years by incentivizing drug makers to “repurpose” major market drugs for the treatment of life-threatening rare diseases including pediatric cancers. Biopharmaceutical companies rarely repurpose major market therapies to treat rare diseases. The OPEN ACT would solve this problem by making available an “Orphan Product Exclusivity Extension,” which would provide an additional six months of market exclusivity to the patent life of the major market drug being repurposed, so long as the sponsor company establishes that the therapy is designated to treat a rare disease and obtains a rare disease indication from the Food and Drug Administration (FDA).

With 95 percent of rare diseases having no FDA-approved treatment, we are confident the OPEN ACT will result in a significant increase in the number of well-tested therapies approved by the FDA for use in treating rare disease patients. We also believe the OPEN ACT will lead to rare disease therapies priced at major market prices, fewer rare disease patients using untested and potentially ineffective drugs off-label, and a boost in investment in the biotech sector.

We thank you for your consideration of this critical legislation, which promises to improve the quality of life for the nearly 30 million Americans suffering from rare diseases. We are eager to work with you and your staff in advancing this important legislation and look forward to the day it is enacted.

Sincerely,

5p- Society - Lakewood, California
A Kids' Brain Tumor Cure - Chappaqua, New York
Abigail Alliance for Better Access to Developmental Drugs - Lorton, Virginia
Adrenal Insufficiency United - Springfield, Oregon
Aiden's Army - Wheaton, Illinois
Alexa Nawrocki Pediatric Cancer Foundation - Marlton, New Jersey
Alex's Army Childhood Cancer Foundation - Herdon, Virginia
Ali's Angels Foundation - Pearcy, Arkansas
ALL4Trey - Charleston, West Virginia
Amyloidosis Foundation - Clarkston, Michigan
Amyloidosis Research Consortium - Lincoln, Massachusetts
Angioma Alliance - Norfolk, Virginia
A-T Children's Project - Coconut Creek, Florida
Autoinflammatory Alliance - San Francisco, California
Aware of Angels - Provo, Utah
Batten Disease Support & Research Association - Columbus, Ohio
Bear Necessities Pediatric Cancer Foundation - Chicago, Illinois
Beckwith-Wiedemann Children's Foundation International - Oregon City, Oregon
Bert's Big Adventure - Atlanta, Georgia
Beyond Batten Disease Foundation - Austin, Texas
BioPontis Alliance for Rare Diseases - Raleigh, North Carolina
Born a Hero - Bothell, Washington
BRBN Alliance - Raleigh, North Carolina
Bridge the Gap - SYNGAP Education and Research Foundation - Cypress, Texas
Caleb's Crusade Against Childhood Cancer - Fort Myers, Florida
Cardio-Facio-Cutaneous International - Vestal, New York
CARES Foundation, Inc. - Union, New Jersey
Carson Leslie Foundation - Dallas, Texas
Castleman Disease Collaborative Network/Castleman's Awareness & Research Effort - Paso Robles, California
Celiac Support Association - Omaha, Nebraska
Center for Jewish Genetics - Litwack, Illinois
Chase After a Cure - Summerville, South Carolina
Children's Cardiomyopathy Foundation - Tenafly, New Jersey
Children's PKU Network - Encinitas, California
Choroideremia Research Foundation, Inc. - Springfield, Massachusetts
Cole vs Cancer - Charlotte, North Carolina
Cooley's Anemia Foundation - New York, New York
Cure AHC - Raleigh, North Carolina
Cure HHT - Monkton, Maryland
Cure JM Foundation - Hume, California
Cure Sanfilippo Foundation - Columbia, South Carolina
Cure SMA - Elk Grove Village, Illinois
CureCADASIL - Plainsboro, New Jersey
CureDuchenne - Newport Beach, California
CurePSP - New York, New York
Cures Within Reach - Skokie, Illinois
Curing Retinal Blindness Foundation - Warner Robins, Georgia
DADA2 Foundation - Nashville, Tennessee
DC Outreach Inc. - Washington, DC
DEFY Foundation - West Chester, Pennsylvania
Delainee’s Battle - , Virginia
Desmoid Tumor Research Foundation (DTRF) - New Canaan, Connecticut
Dominick One in a Million - Pittsburg, Pennsylvania
Dravet Syndrome Foundation - West Haven, Connecticut
Drew's Hope Scientific Research Foundation - Newtown, Pennsylvania
EB Research Partnership - New York, New York
EDSers United Foundation - Plainsboro Township, New Jersey
EveryLife Foundation for Rare Diseases - Novato, California
Fabry Support & Information Group - Concordia, Missouri
Families of Spinal Muscular Atrophy - Libertyville, Illinois
Fibrodysplasia Ossifacans Progressiva (FOP) Association - Casselberry, Florida
Fibromuscular Dysplasia Society of America (FMDSA) - Rocky River, Ohio
Fibrous Dysplasia Foundation - Porter, Washington, DC
FMD Chat - Canton, North Carolina
Foundation for Ichthyosis & Related Skin Types, Inc. - Colmar, Pennsylvania
Gene Giraffe Project - Clare, Iowa
Gene Spotlight Inc. - Miami Beach, Florida
Genetic Alliance - Washington, DC
Global Genes Project - Aliso Viejo, California
Gold Rush Cure Foundation - Laguna Niguel, California
GT23 Foundation - Lakewood, Ohio
Gwendolyn Strong Foundation - Santa Barbara, California
Habitat for Hope - Memphis, Tennessee
Hannah's Hope Fund - Rexford, New York
Help Extinguish Hunter Syndrome - Huber Heights, Ohio
Heriditary Neuropathy Foundation - New York, New York
Hermansky-Pudlak Syndrome Network Inc. - Oyster Bay, New York
HHT Foundation International Hereditary Hemorrhagic Telangiectas - Monkton, Maryland
Hope4Bridget Foundation - Carpenterville, Illinois
Hunter Syndrome Research Coalition - Chicago, Illinois
Info and Resources for Idiopathic Pulmonary Hemosiderosis (IPH-NET) - Kansas City, Missouri
International FOP Association - Oviedo, Florida
International Pemphigus and Pemphigoid Foundation (IPPF) - Sacramento, California
International Waldenstrom's Macroglobulinemia Foundation (IWMF) - Sarasota, Florida
ISMRD (the International Advocate for Glycoprotein Storage Diseases) - Saratoga, California
Jett Foundation - Kingston, Massachusetts
Joey's Wings Foundation - Gainsville, Florida
Jonah's Just Begun - Brooklyn, New York
Journey4ACure - Broadlands, Virginia
Kids v Cancer - Washington D.C., D.C.
Klippel-Feil Syndrome Freedom - Chicago, Illinois
Let Them Be Little X2 Inc. - Elmwood Park, New Jersey
Little Miss Hannah Foundation - Henderson, Nevada
LMSarcoma Direct Research Foundation - Tulsa, Oklahoma
Luck2Tuck Foundation - Southbury, Connecticut
Lymphatic Malformation Institute - Bethesda, Maryland
Mary Payton's Miracle Foundation - Mandeville, Louisiana
Mastocytosis Society - Hastings, Nebraska
Mikey's Way Foundation - Fairfield, Connecticut
MitoAction - Boston, Massachusetts
MLD Foundation - West Linn, Oregon
Mytonic Dystrophy Foundation - San Francisco, California
Narcolepsy Network - North Kingstown, Rhode Island
National Fragile X Foundation - Walnut Creek, California
National Leiomyosarcoma Foundation - Englewood, Colorado
National MPS Society - Durham, North Carolina
National Organization for Rare Disorders (NORD) - Danbury, Connecticut
National PKU Alliance - Tomahawk, Wisconsin
National Tay-Sachs & Allied Diseases Association (NTSAD) - Brighton, Massachusetts
NGLY1.org - Salt Lake City, Utah
Noah's Hope - Downers Grove, Illinois
Noah's Light Foundation - Winter Garden, Florida
Organic Acidemia Association - Minneapolis, Minnesota
OsteoPETrosis Society - Ashbury, New Jersey
Parent Project Muscular Dystrophy - Middletown, Ohio
Pediatric Cancer Foundation - Mamaroneck, New York
Pediatric Hydrocephalus Foundation - Woodbridge, New Jersey
Phelan-McDermid Syndrome Foundation - Venice, Florida
PKD Foundation - Kansas City, Missouri
POMC Island One boy an Ocean of friends - Daytona Beach, Florida
Power Over Pain CRPS Foundation - New Castle, Delaware
Prader-Willi Syndrome Association - Sarasota, Florida
Princesses on a Mission, Inc. - Windsor, North Carolina
Pulmonary Fibrosis Advocates - Wayzata, Minnesota
Rare & Undiagnosed Network - Park City, Utah
Rare Disease United Foundation - Rumford, Rhode Island
RARE Science, Inc. - Encinitas, California
RASopathies Network USA - Altadena, California
Relapsing Polychondritis - Coon Rapids, Minnesota
Run4Rare - Vacaville, California
Samuel Szabo Foundation - Chardon, Ohio
Sarcoma Foundation of America - Damascus, Maryland
Saving Case & Friends - Thompson's Station, Tennessee
Sephardic Health Organization for Referral & Education - Great Neck, New York
Sickle Cell Community Consortium - Alpharetta, Georgia
Sickle Cell Warriors, Inc. - Alpharetta, Georgia
Smashing Walnuts Foundation - Leesburg, Virginia
Sofia's Hope, Inc. - Miami, Florida
Sophia's Fund - Marlborough, Massachusetts
SSADH Association - Delafield, Wisconsin
Stillbrave Childhood Cancer Foundation - Burke, Virginia
Supporting Our Cancer Kids - Vacaville, California
Talia’s Legacy Children’s Cancer Foundation - Oviedo, Florida
Taylor's Tale - Charlotte, North Carolina
Team Ashley Bragg - Mt. Carbon, West Virginia
Team Sabrina - Falls Church, Virginia
Team Sanfilippo Foundation - Ronkonkoma, New York
Team Serena - Maryland
The Adult Polyglucosan Body Disease Research Foundation (APBDRF) - Brooklyn, New York
The Arms Wide Open Childhood Cancer Foundation - Marlboro, New York
The Association for Glycogen Storage Disease - Durant, Iowa
The Bozeman 3 - Bozeman, Montana
The Brooke Healey Foundation - New Providence, New Jersey
The Catherine Elizabeth Blair Memorial Foundation - Washington, DC
The Champ's Corner - Osprey Lane, New Jersey
The Children's Medical Research Foundation, Inc. - Western Springs, Illinois
The Coalition for Pulmonary Fibrosis - Culver City, California
The GIST Cancer Awareness Foundation - Staten Island, New York
The Global Foundation for Peroxisomal Disorders - Tulsa, Oklahoma
The Kortney Rose Foundation - Oceanport, New Jersey
The Life Raft Group - Wayne, New Jersey
The MAGIC Foundation - Oak Park, Illinois
The Nicholas Conor Institute - San Diego, California
The Rally Foundation for Childhood Cancer Research - Atlanta, Georgia
The Rare Cancer Research Foundation - Durham, North Carolina
The Rare Childhood Cancer Advocacy Group - Casanova, Virginia
The Ryan Foundation - Carrollton, Texas
The Santonio Holmes III & Long Foundation - Burleson, Texas
The Truth 365 - Marlboro, New York
The Will Luthcke Foundation - Birmingham, Alabama
West Virginia Kids Cancer Crusaders, Inc. - Charleston, West Virginia
Wilms Tumor Survivor Group - Seattle, Washington
With Purpose - Plymouth, Minnesota