
FOUNDATION TO FIGHT H-ABC
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IWMF
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EVERYLIFE FOUNDATION FOR RARE DISEASES
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Genetic Alliance
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nfed
NATIONAL FOUNDATION FOR ECTODERMAL DYSPLASIA
cure CMD
a cure is among us
HCU NETWORK AMERICA
cureSHANK
BOUVIER GRANT GROUP
Clinic for Special Children
Cure MUCOLIPIDOSIS
LUPUS AND ALLIED DISEASES Association, Inc.
NAF National Ataxia Foundation
Organic Acidemia Association
CUTANEOUS LYMPHOMA FOUNDATION
MUCOLIPIDOSIS TYPE IV FOUNDATION
Hydrocephalus Association
HAYSTACK PROJECT The Voices of Rare & Ultra Rare
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CURED Campaign Urging Research for Eosinophilic Disease www.CuredFoundation.org
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TEXAS NEUROFIBROMATOSIS FOUNDATION
CSNK2A1 FOUNDATION
TSC Alliance
IMMUNOCOMPROMISED ASSOCIATION
PHA Pulmonary Hypertension Association Empowered by hope
Progeria Research Foundation FOR THE CHILDREN ♥ FOR THE CURE
Barth Syndrome Foundation
pmsf Phelan-McDermid Syndrome FOUNDATION
STOMACH CANCER PREVENT. TREAT. LIVE.
Mission:Cure
ACROMEGALY COMMUNITY
HOPE CHARITIES
STRONGER THAN Sarcoidosis
cureCADASIL
GFPD GLOBAL FOUNDATION for PEROXISOMAL DISORDERS FAMILY • RESEARCH • HOPE
UNITED MITOCHONDRIAL DISEASE FOUNDATION
Hannah's HOPE FUND
TMJA The TMJ Association, Ltd.
National Tay-Sachs & Allied Diseases Association
NF MIDWEST
SCN8A
MKGENETIX
FDMAS ALLIANCE
HPS Harmonin-Protein Synthesis Network, Inc.
Turner Syndrome Society of the United States
CURE CMT4J ADVANCING TREATMENTS FOR RARE DISEASES
KCNT1 EPILEPSY HOPE IS ON THE HORIZON
MISSION MSA Leading the Charge to Cure Multiple System Atrophy
CVSA
Global Genes Allies in Rare Disease
IFCR International Foundation for CDKL5 Research
Epilepsy Support Network of Orange County
Wiskott-Aldrich foundation RESEARCH | EDUCATION | SUPPORT for Wiskott-Aldrich Syndrome
USHER 1F COLLABORATIVE
RETT SYNDROME RESEARCH TRUST
WILSON DISEASE ASSOCIATION Unmasking Strength. Unleashing Promise.
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APS Foundation of America
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NF MICHIGAN
FAMILY HEART FOUNDATION
Lipoprotein(a) & Familial Hypercholesterolemia
CALIFORNIA RARE DISEASE ACCESS COALITION
LGMD2D Foundation
team sanfilippo foundation
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THE JANSEN'S FOUNDATION
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FOUNDATION FOR ANGELMAN SYNDROME THERAPEUTICS
SCAD ALLIANCE SPONTANEOUS CORONARY ARTERY DISSECTION
DANNY'S DOSE the VOICE for Spinal Muscular Atrophy
THE ALEX MANFULL FUND
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CURE LGMD2I
The Ehlers Danlos Society
pmsf
Dravet Syndrome Foundation
sat b2 gene foundation
5P FIVE P MINUS SOCIETY
CUREGM1 FOUNDATION
COALITION TO CURE CALPAIN 3 OVERCOMING WEAKNESS WITH STRENGTH
FOXG1 RESEARCH FOUNDATION
CHI Congenital Hyperinsulinism International When too little sugar hurts, we help.
M-CM NETWORK
Huntington's Disease Society of America
MISSISSIPPI MMF METABOLICS FOUNDATION
LGDA
ALS ASSOCIATION
children's cancer cause
RASOPATHIES NETWORK connect · collaborate · accelerate
National MPS Society Support for Families. Research for a Cure.
GBS | CIDP Foundation International
cmtovm
NPKUA national PKU alliance
CURE SANFILIPPO FOUNDATION
SADS FOUNDATION SUDDEN ARRHYTHMIA DEATH SYNDROMES
Head for the Cure Foundation
UNITEDMSD FOUNDATION MULTIPLE SULFATASE DEFICIENCY
GABA ALLIANCE
FARA Friedreich's Ataxia Research Alliance
KCNQ2 Cure ORG
Alive and Kick'n LIVING WITH LYNCH SYNDROME
AFTD
HIDE & SEEK FOUNDATION
SynGAP RESEARCH FUND
FD
IDF Immune Deficiency Foundation
FOUNDATION FIGHTING BLINDNESS
The Louisa Adelynn Johnson Fund for Complex Disease
Chromosome Disorder Outreach
DART DANA'S ANGELS RESEARCH TRUST ...aiming to cure Niemann-Pick Type C
wyner nation foundation
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Juju and Friends CLN2 Warrior Foundation
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FSR FOUNDATION FOR SARCOIDOSIS RESEARCH
MYASTHENIA GRAVIS KY SUPPORT GROUP
National FABRY DISEASE Foundation
PARTNERSHIP TO FIGHT CHRONIC DISEASE
amyloidosis foundation
USHER SYNDROME COALITION
BLUEFIELD Caring FD
Aislinn's Wish
ALDConnect
SICKLE CELL ASSOCIATION OF KENTUCKIANA
THE LAM FOUNDATION
IGT Institute for Gene Therapies
LITTLE HERCULES FOUNDATION
debra because the cost of doing nothing is too great
nfxf NATIONAL FRAGILE X FOUNDATION
RYA-1 FOUNDATION STRENGTH IN NUMBERS
ALPORT SYNDROME FOUNDATION
HAEA US HEREDITARY ANGIOEDEMA ASSOCIATION
BDSRA foundation
ACD ASSOCIATION FOR CREATINE DEFICIENCIES
Team Telomere
Galactosemia Foundation
v-ATPase Alliance
LGMD AWARENESS FOUNDATION
TBRS community
MDA
Oxalosis & Hyperoxaluria Foundation
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ARPKD/CHF ALLIANCE improving the lives of those affected
RNE RARE NEW ENGLAND
GRIN2B Foundation
E.W.E Foundation
cares CONGENITAL ADRENAL HYPERPLASIA FOUNDATION
Child Neurology FOUNDATION Creating a Community of Support
ASPIRE
Accessia Health
Taylor's tale
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Nephcure
THE BONNELL FOUNDATION: LIVING WITH CYSTIC FIBROSIS LIVE • BREATHE • INSPIRE
HOPE for Dante' "Batting against Batten Disease"
CURE VCP DISEASE, INC.
ALPHA-1 FOUNDATION
Amyloidosis Research Consortium
ALSGA
good days effective compassion
ippf International Pemphigus Pemphigoid Foundation
PROJECT ALIVE
MUENZER MPS RESEARCH & TREATMENT CENTER
USTMA
HFA Hemophilia Federation of America
Pulmonary Fibrosis Foundation
CONQUER MG
MSUD FAMILY SUPPORT GROUP
ZTTK
STXBP1disorders Science + Love = Cure
Maryland RARE Advocacy in Action
GIG
GLUTEN INTOLERANCE GROUP
Alliance for Regenerative Medicine
UCLA
PETRONILLE HEALTHY SOCIETY
nnpdf NATIONAL NIEMANN-PICK DISEASE FOUNDATION, INC.
ASSOCIATION FOR CREATINE DEFICIENCIES
MGA
Treatment Foundation ZMYND11
Precision Healthcare Ecosystem
PATIENTLY STUDIOS
NEMSN
g6pd Deficiency Foundation
Parent Project Muscular Dystrophy
live fearlessly
EOSINOPHILIC & RARE DISEASE COOPERATIVE

January 24, 2025

President Donald Trump
1600 Pennsylvania Ave., NW
Washington, DC 20500

Dear President Trump,

On behalf of the EveryLife Foundation for Rare Diseases and the 217 undersigned organizations representing leading rare disease patient advocacy organizations serving the estimated 30 million Americans living with a rare disease or disorder, we are pleased to provide you with a set of priority policies to support the rare disease community as you begin your term as our nation's 47th President.

The policy priorities articulated below are informed by the needs of our community and our shared missions of ensuring all Americans affected by one or more of the estimated 10,000 rare diseases can access a timely diagnosis, expert clinical care, and optimal treatment options. We are a community who feels an intense sense of urgency on behalf of the millions of Americans living with rare diseases and their families, a disproportionate percentage of whom are children. Having worked with this administration before to help advance the use of patient experience data in research and therapy development, we know that you prioritize the health of the children of this nation, especially those living with rare diseases. As you may be aware, over two thirds of rare diseases are genetic in origin and about 70 percent of rare diseases begin in childhood, a statistic that becomes more sobering when acknowledging that fewer than 5% of the estimated 10,000 rare diseases have an FDA-approved therapy.[1]

Over 1,120 orphan designated approvals are changing lives of the patients and families thanks in large part to remarkable advances in science and technology, investments in rare disease research, significant efforts by patient communities, and the leadership of both the National Institute of Health (NIH) and the Food and Drug Administration (FDA) in embracing regulatory science innovation and the application of appropriate flexibility in rare disease product evaluation.[2]

Our optimism is balanced by the staggering extent of unmet need that remains and the recognition that in some cases, it is process and policy hurdles preventing scientific possibility from reaching patients. Rare disease patients and their families face significant challenges at every stage of diagnosis, treatment, and disease management. These barriers often lead to avoidable diagnostic and treatment delays, irreversible disease progression, and devastating social and economic consequences.

The National Economic Burden of Rare Disease Study estimated that in 2019 the overall annual economic impact of rare diseases in the United States exceeded $966 billion.[3] Of the total health economic assessment, indirect costs from productivity losses comprised the most significant economic

[1] https://ncats.nih.gov/sites/default/files/NCATS_RareDiseasesFactSheet.pdf
[2] https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm
[3] Yang, G., Cintina, I., Pariser, A. et al. The national economic burden of rare disease in the United States in 2019. Orphanet J Rare Dis 17, 163 (2022). https://doi.org/10.1186/s13023-022-02299-5

impact at $437 billion, followed by direct medical costs at $418 billion, and non-medical and uncovered healthcare costs of $111 billion absorbed directly by families living with rare diseases[4]. These findings highlight the necessity of focusing on policies that address rare disease community needs, spanning the overall healthcare system.[5]

Our nation's rare disease community deserves access to comprehensive, affordable health insurance that enables us to receive timely diagnoses, recommended clinical care, and access to prescribed therapies. While rare disease patients are included among every public and private insurer's membership, Medicaid and CHIP play a critical role in ensuring kids and adults with rare diseases and disabilities can access the care they need. Covering almost half of all children with special healthcare needs, Medicaid coverage is an essential component to diagnosis, care, and treatment for the rare disease community.[6]

Alongside patients, families, researchers, clinicians, the diagnostic and biopharmaceutical industry, and other committed rare disease community stakeholders, our organizations drive evidence-based policy solutions and partner with regulatory agency leaders to ensure our public health infrastructure is optimized to work for all Americans, regardless of the rarity of one's condition.

To further your policy goals to create efficient, affordable, and high-quality healthcare, our organizations recommend the inclusion of the following policy areas among those of your administration.

1. **Preserve and enhance therapeutic development incentives**
2. **Support predictable and consistent approaches to the evaluation of rare disease therapies**
3. **Modernize the nation's newborn screening program to ensure all babies can benefit from lifesaving health interventions at the earliest moment possible**
4. **Support policies that facilitate timely and affordable access to approved therapies**

Preserve and enhance therapeutic development incentives

Rare disease research and development is often more complex, time-intensive, and costly compared to non-orphan products. A condition qualifies as a rare disease if it affects fewer than 200,000 people in the U.S.,[7] but many innovative therapies target conditions with far smaller patient populations. Developing treatments for such small populations requires significant investment to address challenges like understanding the natural history of diseases, resolving diagnostic uncertainties, and validating

[4] Ibid
[5] Ibid
[6] Musumeci, M., Chidambaram, P., & O'Malley Watts, M. (2019, June 14). Medicaid financial eligibility for seniors and people with disabilities: Findings from a 50-state survey - issue brief - 9318. KFF. https://www.kff.org/report-section/medicaid-financial-eligibility-for-seniors-and-people-with-disabilities-findings-from-a-50-state-survey-issue-brief/
[7] 21 U.S.C. § 316 (1983)

outcome measures. Clinical trials for rare disease products take twice as long as those for non-orphan products,[8] with higher risks of failure.

Since the Orphan Drug Act (ODA) was enacted in 1983,[9] policymakers have acknowledged the complexities of rare disease research and the critical need for policies that affirm no disease is too rare to warrant treatment. The ODA, along with various policies introducing targeted incentive mechanisms in the years since, has successfully spurred investment in the development of therapies for rare diseases. However, recent regulatory changes, potential threats to the sustainability of these incentives, and shifts in the scientific and economic landscape have created an uncertain future—particularly for the development of treatments targeting extremely small patient populations.

Reauthorize the Rare Pediatric Disease Priority Review Voucher Program

The Rare Pediatric Disease Priority Review Voucher (PRV) Program is a market-based incentive that has led to new treatments for 40 rare diseases at no cost to taxpayers. Unfortunately, this life-saving program lapsed on December 20, 2024, despite being extended for six years in the House-passed Give Kids a Chance Act and having broad bipartisan support. Over 200 patient organizations have advocated for the PRV Program to be extended.[10] Immediate action is needed from Congress to rectify this oversight early in 2025. We urge your administration to work with Congress to prioritize an extension to the PRV Program in the early days of the 119th Congress. Children's lives are at stake.

Preserve and Restore the Orphan Drug Tax Credit

The Orphan Drug Tax Credit (ODTC), established as part of the ODA, allows companies to claim a tax credit for a portion of their qualified research expenses. Originally set at 50% of qualified expenses, the credit was reduced to 25% in 2017. Over the years, the ODTC has played a vital role in the approval of orphan drugs, reducing the financial burden of clinical trials that can be more complex, longer, and pricier than non-orphan products. Rare disease patients and families facing life-changing diagnoses need more tools in the drug development toolchest, not fewer. A robust ODTC, as intended in the bipartisan ODA, means more companies can take a chance on a novel therapy prospect. It also enables companies to conduct clinical studies to find new uses in orphan diseases after a treatment is already on the market.

Enable federal research agencies to drive innovation and fill critical gaps in resources for rare disease therapy development

With over 10,000 rare diseases, each presenting differently across patients, orphan drug investments remain high risk. Federal programs like the Orphan Products Grant Program (OPGP) at the FDA, the National Center for Advancing Translational Sciences (NCATS) at the NIH, and the Advanced Research

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[8] Jayasundara, K., Hollis, A., Krahn, M. *et al.* Estimating the clinical cost of drug development for orphan versus non-orphan drugs. *Orphanet J Rare Dis* 14, 12 (2019). https://doi.org/10.1186/s13023-018-0990-4

[9] 21 U.S.C. § 316 (1983)

[10] EveryLife Foundation for Rare Diseases. (2024, December 6). EveryLife Foundation Joins Forces with NORD and Leading Patient Organizations to Call for Congress to Pass Long-Term Extension of the Rare Pediatric Disease PRV Program. https://everylifefoundation.org/wp-content/uploads/2024/12/Rare-Pediatric-Disease-Priority-Review-Voucher-Program-Reauthorization-Community-Letter.pdf

Projects Agency for Health (ARPA-H) have spurred innovative collaborations, built infrastructure to attract private investment, and funded research targeting diseases that were once deemed too rare or complex to treat. These investments allow non-traditional drug development models, such as initiatives led out of academic centers, non-profit research institutes, and patient organizations to be viable when commercial interest will never be. The OPGP alone has funded clinical trials that led to over 80 FDA-approved therapies and in its 13-year history;[11] NCATS has contributed to 55 Investigational New Drugs and 14 approved therapies and encouraged innovation in areas such as cell and gene therapies and catalyzed industry interest in rare conditions that were previously overlooked.[12]

Promote policies that will incentivize the development and repurposing of treatments and cures for rare diseases

Despite the complex nature of rare diseases, advances in science, technology, and drug development platforms have increased the possibilities that one therapy might be effective across a variety of diseases with shared pathways. Increasingly, willingness to invest in rare disease therapy development is contingent on a target molecule's potential to treat multiple rare diseases. Decisions about investments, the order of trials and FDA submissions, and repurposing efforts often carry life-or-death consequences for patients. Policies should maximize a therapy's potential, letting science drive outcomes, and enabling the use of the established and broadly supported expedited pathways for FDA approval, that for some rare disease communities, may be the only viable option to get treatments to patients.

Support predictable and consistent approaches to the evaluation of rare disease therapies

Support Successful Implementation of the Rare Disease Innovation Hub at the FDA

Following years of advocacy by the rare disease community, in 2024 the FDA announced the establishment of the Rare Disease Innovation Hub to improve consistency and predictability of therapy approaches across and within the Centers and Divisions for rare disease patients and therapy manufacturers. We urge your Administration to commit to a clear set of priorities that align with those of the rare disease community, and that focus on addressing the most significant challenges, particularly building FDA regulatory science expertise in rare disease.

Developing Treatments for Ultra-Rare Diseases

While all rare diseases pose challenges and additional costs to developing therapies and treatments, the needs are even more pronounced when it comes to ultra-rare or very small population diseases and conditions. Of the approximately 10,000 known rare diseases, more than 80 percent have a prevalence

[11] U.S. Food and Drug Administration. (2024, October 1). *Clinical Trials Grants Program*. Orphan Products Grant Program. https://www.fda.gov/industry/orphan-products-grants-program/clinical-trials-grants-program
[12] U.S. Department of Health and Human Services. (2024, August 28). *Our Impact*. National Center for Advancing Translational Sciences. https://ncats.nih.gov/research/our-impact

of less than 1 in 1 million,[13] and advances in science are leading to identification of hundreds of newly identified rare genetic diseases every year. A particular challenge for ultra-rare diseases is the limited natural history data associated with any condition as well as very small patient populations that make clinical evaluation exceedingly difficult and more costly. To help address these concerns, we urge you to support policies and development incentives that enable pipelines for small populations to thrive.

Modernize the nation's newborn screening program to ensure all babies can benefit from a timely and lifesaving diagnosis after birth for generations to come

Obtaining a confirmed rare disease diagnosis takes an average of 6.3 years, and includes visits to 16.9 medical providers, and an average of 2.4 out-of-state trips.[14] This delay not only exacerbates physical and emotional suffering, but also leads to substantial financial burdens for patients, families, employers, and the healthcare system. Moreover, delayed diagnoses can result in irreversible disease progression and missed opportunities for timely interventions within optimal therapeutic windows. Newborn screening plays a critical role in identifying pediatric-onset conditions immediately after birth.

Each year, approximately four million newborns are screened in the U.S., and an estimated 12,000 infants are identified to have a treatable condition, benefiting from intervention.[15] In addition to the life-altering benefit of newborn screening, a timely diagnosis also provides an economic benefit for patients and the health care system more broadly. The Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study calculated the avoidable costs associated with providing a timely diagnosis at birth as opposed to a delayed diagnosis for three newborn screening conditions.[16] The conditions studied – X-ALD, Pompe Disease, and SCID – showed savings of $301,647, $168,718, and $517,638 in avoidable costs respectively when a timely diagnosis at birth was provided.[17] Newborn screening leads to better outcomes for families and the healthcare system, which is why it is often referred to as one of the most successful public health programs in the country.

The newborn screening system needs modernization to keep pace with advancements in medical science and emerging treatment options for rare diseases. In the last 15 years, only nine conditions have been added to the federal recommended panel. For the conditions that have been added, states often lag behind the recommendations, taking more than five years to add new conditions. Advances in genomic sequencing and the potential to intervene in more rare diseases as science advances present

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[13] Schaefer, J., Lehne, M., Schepers, J. et al. The use of machine learning in rare diseases: a scoping review. Orphanet J Rare Dis 15, 145 (2020). https://doi.org/10.1186/s13023-020-01424-6

[14] Yang, G., Cintina, I., Pariser, A. et al. The national economic burden of rare disease in the United States in 2019. Orphanet J Rare Dis 17, 163 (2022). https://doi.org/10.1186/s13023-022-02299-5

[15] Centers for Disease Control and Prevention. (2024, May 13). Emergency preparedness for newborn screening programs. Centers for Disease Control and Prevention. https://www.cdc.gov/newborn-screening/php/about/emergency-response.html

[16] The Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study, EveryLife Foundation for Rare Diseases In Partnership With: The Lewin Group, part of Optum Serve Expert Stakeholders The Rare Disease Community, 2023. https://everylifefoundation.org/wp-content/uploads/2023/09/EveryLife-Cost-of-Delayed-Diagnosis-in-Rare-Disease_Final-Full-Study-Report_0914223.pdf

[17] Ibid

great potential for improving children's health, but policy gaps and resource constraints stand to limit the possibilities.

To address these growing challenges, the federal newborn screening system must be modernized through a reauthorization of federal programs that will make needed improvements to ensure that conditions are added in a timely manner and that meets the needs of 21st century diagnostics and therapies. New policies are needed that address how conditions are added, the role of genetic testing, increased investment in public health infrastructure, education, and data systems to support a more effective and equitable newborn screening program. These issues can create a forward-looking system that keeps pace with medical advancements and delivers optimal outcomes for all children and their families.

Support policies that facilitate timely and affordable access to approved therapies

Many of the transformational therapies that are approved face access barriers that prohibit or delay these life-changing treatments from reaching patients. This counteracts the benefits of expedited pathways that speed the availability of drugs to treat serious diseases that are intended to ameliorate unmet patient needs and reduce long term costs by allowing for early access to innovative treatments. Further, utilization management techniques and programs to divert charitable assistance programs are employed by public and private payers to limit their expenses, but these policies have the effect of increasing costs for patients and creating delays that can result in irreversible disease progression and loss of life.

Policy solutions should aim to eliminate the patient access hurdles of time and cost, ensuring access to appropriate FDA-approved therapies without burdensome and medically inappropriate utilization management requirements. To achieve these goals, policies should also enable innovative payment models that recognize the full value of a rare disease treatment and incorporate meaningful input from patient and scientific perspectives.

Please know our organizations will be a resource to you in the coming weeks, months, and years. We welcome the opportunity to meet with you about the priorities of the rare disease community. To help facilitate the engagement of our community organization partners in administration policy priorities, please reach out to Annie Kennedy, Chief of Policy, Advocacy and Patient Engagement, with the EveryLife Foundation for Rare Diseases at akennedy@everylifefoundation.org.

Thank you for your consideration of the estimated 30 million Americans living with one or more rare diseases, and our collective policy priorities as you and your team begin to assess opportunities to advance the nation's health and well-being in the early days of your administration.

Sincerely,

EveryLife Foundation for Rare Diseases
Alliance to Solve PANS & Immune-Related Encephalopathies (ASPIRE)
5P- Society
Accessia Health
Acromegaly Community Inc.
Aislinn's Wish Foundation
Alagille Syndrome Alliance
ALD Connect
AliveAndKickn
Alliance for Regenerative Medicine
Alpha-1 Foundation
Alport Syndrome Foundation
ALS Association
Amyloidosis Foundation
Amyloidosis Research Consortium
Angelman Syndrome Foundation
APS Foundation of America, Inc
ARPKD/CHF Alliance
Association for Creatine Deficiencies
Avery's Hope
Barth Syndrome Foundation
BDSRA Foundation
Bouvier Grant Group LLC
CACNA1A Foundation
California Rare Disease Access Coalition
Child Neurology Foundation
Children's Cancer Cause
Children's Sickle Cell Foundation Inc.
Chondrosarcoma CS Foundation, Inc.
Chromosome Disorder Outreach Inc.
Clinic for Special Children
CLOVES Syndrome Community
CMTC-OVM
Coalition to Cure Calpain 3
Congenital Adrenal hyperplasia Research, Education & Support Foundation
DBA: CARES Foundation Inc.
Congenital Hyperinsulinism International
Conquer MG
CSNK2A1 FOUNDATION
Cure CMD
Cure GM1 Foundation
Cure HHT
Cure Mucolipidosis
Cure Sanfilippo Foundation
Cure VCP Disease
cureCADASIL
CureCMT4J/Talia Duff Foundation
CURED Nfp
CureLGMD2i Foundation
CureSHANK
Cutaneous Lymphoma Foundation
Cyclic Vomiting Syndrome Association
Dana's Angels Research Trust
Danny's Dose Alliance
debra of America
Dion Foundation for Children with Rare Diseases
Dravet Syndrome Foundation
Eosinophilic & Rare Disease Cooperative
Epilepsy Support Network of Orange County
EspeRare foundation
E.WE Foundation
Familial Dysautonomia Foundation
Family Heart Foundation
FD/MAS Alliance
FND Hope
Foundation Fighting Blindness
Foundation for Angelman Syndrome Therapeutics
Foundation for Prader-Willi Research
Foundation for Sarcoidosis Research
Foundation to Fight H-abc
FOXG1 Research Foundation
Friedreich’s Ataxia Research Alliance (FARA)
Friends of FSH Research
g6pd Deficiency Foundation
GABA-A Alliance
Galactosemia Foundation
GBS | CIDP Foundation International
Gene Giraffe Project
Genetic Alliance
Global Genes
Gluten Intolerance Group of North America
Good Days
GRIN2B Foundation
Hannah's Hope Fund for GAN
Haystack Project
HCU Network America
Head for the Cure Foundation
Hereditary Angioedema Association

Hermansky-Pudlak Syndrome Network
HFA
Hide & Seek Foundation
Hope Charities
Hope For Danté
Hope for Stomach Cancer
Huntington's Disease Society of America
Hydrocephalus Association
HypoPARAthyroidism Association
Immune Deficiency Foundation
Immunocompromised Association
Institute for Gene Therapies
International Autoimmune Encephalitis Society
International Foundation for CDKL5 Research
International Pemphigus & Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
The Jansen's Foundation
Juju and Friends CLN2 Warrior Foundation
KCNQ2 Cure Alliance
KCNT1 Epilepsy Foundation
Krishnan Family Foundation
LGMD Awareness Foundation, Inc
LGMD2D Foundation
Li-Fraumeni Syndrome Association
Lipodystrophy United
Little Hercules Foundation
Live Fearlessly Foundation
Look. Foundation
Lowe Syndrome Association
Lupus and Allied Diseases Association, Inc.
lymphangiomatosis & gorham's disease alliance (LGDA)
Maple Syrup Urine Disease Family Support Group
MarylandRARE.org
M-CM Network
Mission MSA
Mission: Cure
Mississippi Metabolics Foundation
MitoAction
MLD Foundation
Mucolipidosis Type IV Foundation
Muenzer MPS Research & Treatment Center
Muscular Dystrophy Association
Myasthenia Gravis Association
Myasthenia Gravis KY
Myositis Support and Understanding
Myrovlytis Trust
National Ataxia Foundation
National Eosinophilia Myalgia Syndrome Network
National Fabry Disease Foundation
National Foundation for Ectodermal Dysplasias
National Fragile X Foundation
National MPS Society
National Niemann-Pick Disease Foundation
National PKU Alliance
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
NephCure
Neurofibromatosis Michigan
Neurofibromatosis Midwest
Neuroimmune Foundation
No Stomach For Cancer
Noah's Hope - Hope4Bridget
Organic Acidemia Association
Parent Project Muscular Dystrophy
Partnership to Fight Chronic Disease
Patiently Studio
Petronille Healthy Society
Phelan-McDermid Syndrome Foundation
PMD Foundation
Pompe Alliance
Precision Healthcare Ecosystem
Project Alive
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association
PWSA | USA - Prader-Willi Syndrome Association
PXE International
Rare New England
Rare Trait Hope Fund
RASopathies Network
Rett Syndrome Research Trust
SADS Foundation
SATB2 Gene Foundation
SCAD Alliance
Sickle cell association of Kentuckiana
Sickle Cell Disease Association of Illinois
Stronger Than Sarcoidosis
STXBP1 Foundation
Sudden Arrhythmia Death Syndromes (SADS)

Foundation
SynGAP Research Fund dba Cure SYNGAP1
Tatton Brown Rahman Syndrome Community
T.E.A.M. 4 Travis (Together Ending Asplenia Mortality)
Taylor's Tale
Team Sanfilippo Foundation, TSF Inc
Team Telomere
Texas Neurofibromatosis Foundation
The Akari Foundation
The Alex Manfull Fund
The Association for Frontotemporal Degeneration
The Bluefield Project to Cure FTD
The Bonnell Foundation: living with cystic fibrosis
The Ehlers-Danlos Society
The Global Foundation for Peroxisomal Disorders
The LAM Foundation
The Lambert-Eaton LEMS Family Association
The Louisa Adelynn Johnson Fund for Complex Disease
The Oxalosis and Hyperoxaluria Foundation
The Progeria Research Foundation
The RYR-1 Foundation
The Sturge-Weber Foundation
The Sudden Arrhythmia Death Syndromes (SADS) Foundation
The TMJ Association
Tribal Ground
TSC Alliance
Turner Syndrome Society of the United States
UCLA
United Mitochondrial Disease Foundation
United MSD Foundation
Uriel E. Owens Sickle Cell Disease Association of the Midwest
Usher 1F Collaborative
Usher Syndrome Coalition
USTMA Alliance and Consortium
v-ATPase Alliance
Wilson Disease Association
Wiskott-Aldrich Foundation
Wylder Nation Foundation
ZMYND11 Treatment Foundation
ZTTK SON-SHINE Foundation