BIO Patient Advocacy Showcase Summary

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- ZTTK SON-Shine Founation

Accelerated Cure Project for Multiple Sclerosis

Research-focused patient advocacy group providing access to biosamples, patient data, insights, and trial participants

www.acceleratedcure.org – info@acceleratedcure.org

Quick facts

- Primary therapeutic area is **Multiple Sclerosis**; secondary areas are NMO and related conditions
- Primary focus: Supporting and conducting research that leads to new breakthroughs in MS
- Headquartered in Massachusetts; we collaborate with scientists and teams globally

We are looking for research collaborations:

- Biosample and data analysis for therapy and diagnostic development
- Surveys and insights research to understand and meet patient needs and preferences
- Patient-focused drug development partnerships
- Inclusive research collaborations
- Dissemination of research results to the MS community

Key initiatives / projects/ priorities

- ACP Biorepository: Blood samples, clinical and PRO data, multi-omics data from 3,200 people with MS and controls – all open-access
- iConquerMS: Virtual network of >9,000 participants that share their insights, expertise, health data, and biosamples
- Patient-centered research expertise including decentralized trials
- Inclusive research focus; strong connections with traditionally underrepresented communities

What we can offer:

- Blood sample repository (plasma, serum, DNA, RNA, PBMCs)
- Bioanalytic, clinical, and PRO databases
- Surveys/focus groups of people with MS for insights & preferences
- Decentralized trial guidance & support
- Assistance with trial recruitment
- Patient-centric trial design to maximize enrollment and retention

AIMEDALLIANCE

Aimed Alliance is a non-profit health policy organization that seeks to protect and enhance the rights of consumers, caregivers, and providers.
AimedAlliance.org

Quick Facts

- **Therapeutic Areas:** Aimed Alliance works across therapeutic conditions and ages to ensure consumer can access the care and treatments they need.
- **Unique Perspective:** Aimed Alliance is staffed and supported by a team of attorneys that collectively have over 30 years of experience in health law and policy. With this experience, we center legal analysis throughout our policy work and conversations with regulators, legislators, and other non-profits.
- **Key Facts:** 501(c)(3) non-profit health policy organization based in Washington, D.C. and working on access and affordability across all 50 states.

What we are looking for:

- **Artificial Intelligence:** Aimed Alliance is looking to engage thought leaders on how AI can be used to advance innovation, patient experience, and care while still ensuring consumers benefit from individualized human-led care.
- **Access and Affordability:** Aimed Alliance is looking to engage stakeholders to understand how emerging trends in health insurance impact consumer access to care and affordability.
- **Oversight:** Aimed Alliance is looking to engage stakeholders to better understand how we can empower state regulatory agencies to ensure compliance with enacted consumer protection laws.

Key initiatives / projects/ priorities

- **Improving Access and Affordability:** Working with state and federal regulators and legislators to address, step therapy, copay accumulators, and alternative funding programs.
- **Judicial Advocacy:** Leading and empowering non-profit health policy and advocacy organizations to engage in judicial advocacy through education and engagement.
- **Women's Health:** Developing a collective of young women working to address research, access, affordability, and workplace accommodations related to perimenopause and menopause symptoms.
- **Artificial Intelligence:** Ensuring legislators and regulators place guardrails on the use of AI to ensure consumers can receive human-led and individual care considerations.

What we can offer:

- Aimed Alliance is a leading non-profit that provides a unique legal perspective on health care access, affordability, and reform.
- Aimed Alliance produces novel research on a variety of topics related to health care access, disability rights, and employer sponsored health insurance.
- Aimed Alliance publishes resources annually and frequently host webinars on emerging health policy issues and topics.

ALD Alliance & Newborn Screening Alliance

Our mission is to advocate for newborn screening for x-linked adrenoleukodystrophy (ALD) and other rare conditions and to provide current, functional information to families of children with ALD. Our website is www.aldalliance.org and our founder, Elisa Seeger, can be reached at elisa@aldalliance.org.

Quick Facts

- **About X-ALD: Most severely affects boys and men and is caused by mutations in the ABCD1 (women may also be affected in adulthood). The result is an inability to breakdown very long chain fatty acids (VLCFAs). The disease destroys myelin, the protective sheath that surrounds the brain's neurons. ALD involves multiple organs in the body, but most prominently affects the brain and spinal cord.**
- **Primary focus: Patient support & newborn screening advocacy.**
- **Key facts: Founded in Brooklyn, NY in 2012. *Aidan's Law* was passed in New York in 2013. There are currently 46 states screening for ALD.**

What we are looking for:

- **Funding to partner with expert centers to help connect researchers to the ALD community**
- **More research for potential medical or holistic interventions to prevent cerebral ALD from developing (most dangerous phenotype)**
- **Finding treatment options for a late diagnosis of cerebral ALD**
- **Funding for Federal and State newborn screening initiatives for all rare conditions that have valid treatments**
- **Raising awareness of the importance and need for newborn screening**

Key initiatives / projects / priorities

- **ALD incidence project**
- **Continued family support through care package program and educational events - annual meeting**
- **Advocating for newborn screening for all disease with a valid newborn screening test & treatment**

What we can offer:

- **Connection with the ALD community (affected families and presymptomatic boys identified through newborn screening)**
- **Excellent relationships with newborn screening labs**
- **Understanding of legislative process and guidelines for advocates**
- **Expertise in advocating at both the state & federal level for newborn screening, gene therapy, and other lifesaving industry initiatives**

www.amputee-coalition.org

Leading national organization supporting people who have limb loss and limb difference.
Mission is to support, educate, and advocate for our community so that everyone impacted by limb loss and limb difference has the resources, connections, and opportunities to succeed.

Shree Thaker | Director of Communications & Partnerships | sthaker@amputee-coalition.org | 301-848-0737

Quick Facts:

- Founded in 1986; 39 years serving pediatric and adult population of amputees, people who have limb difference, and their caregivers
- Headquartered in Washington, DC with a nationwide team of ~20 staff
- Provides Information & Referral Services, Peer Visit Matching, Support Groups, Community Education Days, Youth Programming, Guides & Publications, Workforce Development, Outreach, and Education
- Partners with VA and DoD for Veteran and Active-Duty Servicemember Peer Support (Certified Peer Visitor Training and Peer Matching)
- Operates the National Limb Loss Resource Center through a cooperative agreement with Administration for Community Living (ACL) through Department of Health and Human Services (HHS)

What We Are Looking For:

- Partnership and collaboration with experts of intersectional disease states, illnesses, and disabilities
- Involvement of people who have limb loss and limb difference in research, clinical trials, and marketing
- Opportunities to network, raise awareness, and highlight the importance of addressing comorbidities for vulnerable populations

Key Initiatives/Projects/Priorities:

- Antimicrobial Resistance (AMR) Advocacy
- Non-Diabetic Limb Loss Prevention (paralysis, spina bifida, bone and joint disorders)
- Obesity Care and Combatting Weight Bias
- Pain Management
- Peripheral Arterial Disease (PAD)
- Physical Activity Access (Activity-Specific Prosthetic and Orthotic Devices)
- Prevention of Diabetes-Related Limb Loss
- Telehealth
- Vaccine Education

What We Can Offer:

- Advocacy and Public Policy Activities/Initiatives
- Research Partnerships, Volunteer Research Opportunities, Letters of Support (www.amputee-coalition.org/information-for-researchers)
- Convening opportunities with our national community of experts including those who have lived experience and partner patient groups for consumer feedback on intersectional disease states, illnesses, and disabilities

autoimmune.org

Amy Phillips, Strategic Alliance Director
amy@autoimmune.org

WE'RE THE WORLD'S LEADING NONPROFIT ORGANIZATION DEDICATED TO AUTOIMMUNE DISEASE ADVOCACY, AWARENESS, EDUCATION, AND RESEARCH.

Our mission:
The Autoimmune Association leads the fight against autoimmune disease by advocating and collaborating to improve healthcare, advance research, and empower the community through every step of the journey.

Quick Facts

- Therapeutic areas / diseases / populations: All autoimmune, immune-mediated & related diseases
- Primary focus: advocacy, awareness, patient education, research
- Key facts: For over 30 years, the Autoimmune Association has been a pioneer in serving autoimmune patients, promoting research, advocating for access to healthcare, and fostering collaboration to identify and explore the common threads that link autoimmune diseases. Today, the Autoimmune Association is an internationally recognized leader reaching over 60 countries worldwide.

What we are looking for:

- Partnership Opportunities
- Support for education, programming and events
- Support for advocacy and policy efforts
- Assistance with raising awareness about autoimmune disease and the estimated 50 million Americans living with an autoimmune condition

Key initiatives / projects / priorities

- Autoimmune Community Summit (Virtual) - September 18-19, 2025
- Hope Journey Walk – Smithsonian National Zoo, Washington, DC, October 12, 2025 (online option)
- Autoimmune Association Congressional Fly-In, March 2026
- Patient education and programming focused on emerging therapeutics and research, disease-specific education and patient empowerment

What we can offer:

- The patient perspective that represents the voices of those living with one or more of the more than 100 autoimmune diseases
- A platform for communicating with the patients, care partners, family members, providers and researchers who comprise the autoimmune community
- Clinical Trial Outreach Programs

CACNA1A Foundation

To find specific treatment options and a cure for CACNA1A patients by building a collaborative network of patients, families, clinicians and scientists that will work together to raise awareness and accelerate the understanding, diagnosis and treatment of CACNA1A-related diseases.

www.cacna1a.org info@cacna1a.org

Quick Facts

- CACNA1A-related disorders are neurodevelopmental disorders that include Episodic Ataxia Type 2 (EA2), Familial Hemiplegic Migraine Type 1 (FHM1), Developmental and Epileptic Encephalopathy (DEE) 42, with a mostly pediatric onset.
- The estimated incidence rate is 1:11,700 (8 out of every 100,000).
- The CACNA1A Foundation is built on three pillars: advancing research, supporting families and raising awareness.
- The Foundation is a 501(c)(3) parent-led nonprofit organization founded in 2020.

What we are looking for:

- **Collaborative partnerships** to accelerate drug development across diverse therapeutic modalities (e.g., ASOs, gene therapy, small molecules)
- **Genotype-inclusive strategies** that address both common and rare CACNA1A variants.
- **Shared expertise and infrastructure** to streamline preclinical testing, biomarker development, and clinical trial readiness

Key Initiatives/Projects/Priorities

- Identifying key IND-enabling studies to advance our CACNA1A gene therapy
- Developing disease-modifying therapies (ASOs) with broad impact
- Increasing our clinical trial readiness with focus on identifying biomarkers and endpoints for CACNA1A-related disorders

What we can offer:

- Access to over 50 KOLs and CACNA1A expert researchers and clinicians in our collaborative research network
- Access to a global community of CACNA1A families (400+)
- Access to our CACNA1A preclinical research toolbox, including 13 patient-derived iPSC lines, 3 natural history studies, and insight into clinical trial design for CACNA1A-related disorders.

Caregiver Action Network's Mission

to promote resourcefulness and respect for tens of millions of family caregivers across the country.

CAREGIVER HELP DESK
855.227.3640
CaregiverAction.org

CAREGIVER ACTION NETWORK

CAREGIVER **QUICK FACTS**

- **Over 100 million** Americans provide unpaid care to a loved one.
- The majority are **family caregivers**, caring for aging parents, children with special needs, or loved ones with chronic or rare conditions.
- Caregivers face **emotional, physical, and financial challenges**, often without formal training or support.
- 60% of caregivers are **employed**, juggling work and care responsibilities.
- Caregivers contribute an estimated **$600 billion** in unpaid care annually.

ABOUT CAREGIVER ACTION NETWORK

- CAN is the **leading national organization** for family caregivers of all ages, backgrounds, and care situations.
- CAN is a **nonprofit organization** dedicated to improving the lives of **family caregivers.**
- Offers **free education, peer support, and resources** to caregivers across the lifespan.
- Programs span across areas like **chronic illness, mental health, rare diseases, military caregiving, and workplace support.**

WE ARE LOOKING FOR PARTNERS!

- Are committed to **supporting caregivers** in tangible and visible ways.
- Want to engage with caregivers through **targeted outreach, educational initiatives, or health equity programs.**
- Share a mission-aligned interest in chr**onic conditions, rare disease, aging, mental health, or work-life balance.**
- Are looking to build **brand trust** and demonstrate **corporate social responsibility.**

WHAT CAN OFFERS

- **Visibility** through co-branded campaigns, webinar series, and caregiver resource hubs.
- Recognition on **landing pages, e-newsletters, and event promotions**.
- **Thought leadership opportunities** via webinars, blogs, and advisory panels.
- **Targeted outreach** to caregiver demographics that match sponsor's markets or CSR goals.
- Measurable **impact metrics** showing sponsor engagement and reach within the caregiver community.
- The opportunity to support a **high-trust, high-need audience** during critical moments.

Child Neurology Foundation

Our mission is to serve as a collaborative center of education, resources, and support for children and their families living with neurologic conditions, and facilitate connection with medical professionals who care for them.

Who We Are

- Founded in 2001, headquartered in Lexington, KY, serving nationwide
- Focused on pediatric neurologic conditions including rare, ultra-rare, and undiagnosed conditions
- Governed by a board composed primarily of neurologists
- Promotes equity and access with peer support, social work services, and community-driven programs

Our Core Focus

Education • Family Support • Advocacy • Care Coordination • Research Funding

What We Are Looking For

Mission-aligned industry partners to:

- Co-develop accessible, evidence-based educational tools
- Collaborate on advancing family-centered support systems
- Drive innovation in care models that respond to real-world challenges across the neurologic disease spectrum

Current Key Priorities

Lifespan Care /Transition of Care

- Actively advancing smoother transitions from pediatric to adult care through collaborative models that center continuity, equity, and self-advocacy for youth with neurologic conditions.

Family Support

- Providing personalized emotional and social services through a national network of 95+ advocacy organizations. We are building a dedicated Social Services Fund to reduce non-medical barriers to care and address urgent family needs.

Education

- Delivering timely, trusted, and data-informed educational content across digital platforms to meet families, clinicians, researchers, and advocates where they are—and drive shared learning and engagement.

What We Offer

Strategic Transition of Care Collaboration

- Leverage our expertise and infrastructure to co-develop scalable, data-informed models that improve continuity and outcomes as youth with neurologic conditions move into adult care.

National Family Support Platform

- Partner with us to expand an established, scalable system—built on a network of 95+ advocacy groups and a Social Services Fund—addressing social determinants of health and reducing non-clinical barriers to care.

Trusted Education & Engagement Channels

- Reach targeted audiences through our high-impact digital platforms, using real-time insights to deliver evidence-based content, strengthen community trust, and maximize educational reach.

COMMUNITY LIVER ALLIANCE ™
GLOBAL AWARENESS. LOCAL IMPACT.

📍 Based in Pittsburgh. Impact across the country.

The Community Liver Alliance (CLA) is a national nonprofit shaking up the status quo in liver health. We're the people behind the scenes — and on the front lines — educating communities, supporting patients, and bringing together the brainpower of providers, researchers, and industry leaders. We don't just raise awareness — we move the needle.

🔥 **What We're About**

- Hosting powerhouse education events, national summits, and straight-talk patient programs
- Teaming up on cutting-edge research and real-world community engagement
- Advocating like it matters — because it does. We're making sure access to treatment and screening isn't a luxury

💡 **Fun Facts**

- Working across liver cancer, ALD, MASLD/MASH, hep B & C, and rare/autoimmune liver diseases.
- Reaching rural and underserved communities with real tools and real talk.
- Proud to be scrappy, strategic, and serious about impact.

🚀 **We're looking for:**

- Biotech & pharma players ready to collaborate. We're looking for partners, not just sponsors.
- Clinical trial partners who care about reaching the people who need it most.
- Innovators focused on metabolic health, obesity, oncology, and liver disease.

We bring to the table:

- Authentic relationships with patients & providers.
- Proven track record turning good ideas into action.
- Nimble, customizable programming that actually connects.

🎯 **Bottom Line: If you're ready to do work that matters — and make liver health a lot louder — let's connect in Boston.**

COALITION OF SKIN DISEASES

Advocating on behalf of the 84 million Americans living with a skin disease

www.skincoalition.org

Quick Facts

With 46 member groups, the CSD endeavors to be at the forefront of progress toward a day where all individuals affected by skin disease have access to life-preserving/changing treatments and high-quality care. We aim to accomplish this by:

- Fostering education and awareness of skin diseases and disorders;
- Advocating for access to appropriate and quality health care and treatments;
- Contributing to burden and related skin disease research; and
- Strengthening dermatologic patient advocacy organizations through the sharing of resources.

What we are looking for:

Relationship building with potential corporate partners and collaboration to improve the lives of dermatology patients.

Current corporate partners are:

Current & Ongoing Projects

- Annual development of joint advocacy priorities and legislative agenda;
- Grassroots advocacy training and year-round federal advocacy efforts through action alerts and local meetings with policy makers;
- Promotion of a Congressional Skin Caucus;
- Capitol Hill Day fly-in for members in May with congressional briefing;
- Participating in national group efforts to amplify the CSD's priorities through joint sign-on letters, campaigns, and meetings;
- Member development webinar series on relevant topics; and
- Maintain a repository of shared resources on the CSD Member Portal

What we can offer:

Corporate partners receive numerous benefits, including:

- Attendance at the CSD's Hill Day event and congressional briefing;
- Advance access to CSD policy updates;
- CSD Development Day and Annual Meeting non-voting participation;
- CSD quarterly membership meetings non-voting participation;
- Annual membership survey(s) courtesy review and contribution;
- And more!

CUREGM1 FOUNDATION

EIN #47-3154419
WWW.CUREGM1.ORG
INFO@CUREGM1.ORG

MISSION: Research, Advocacy, Drug Development Awareness for GM1 Gangliosidosis, a fatal pediatric lysosomal storage disease

ABOUT CURE GM1

- 501(c)(3) nonprofit founded in 2015 in honor of Iris and all who suffer from GM1 gangliosidosis
- Only organization wholly dedicated to GM1 gangliosidosis awareness, advocacy, drug development and research in the United States
- $5.5M+ raised to date
- Foundation's investments and direct efforts have enabled therapeutic development for GM1
  - ✓ Collaborated with the Jackson Laboratory to make mouse model publicly available
  - ✓ Funded newborn screening assay development, which has been deployed in pilot studies
  - ✓ Funded xCures EHR Natural History pilot study
  - ✓ Launched biobank with CombinedBrain
  - ✓ Provided initial funding that enabled first intravenous AAV9 gene therapy program at UMass licensed to Sio Gene Therapies, and now investigator-led at NIH
  - ✓ Conducted Externally-Led Patient-Focused Drug Development Meeting with FDA; Voice of the Patient Report published 2022
  - ✓ Published Caregiver experience and Caregiver burden studies
- In contact with more than 300+ GM1 families

WHAT WE ARE LOOKING FOR

- INVESTORS
- BIOTECH / PHARMA PARTNERS
- VENTURE PHILANTHROPY
- DISCOUNTED CRO and CMO FEES

ENZYME REPLACEMENT THERAPY FOR GM1 FOLLOWING A PROVEN PATH TO CLINICAL SUCCESS

ENZYME REPLACEMENT THERAPY IS A PROVEN STRATEGY IN LYSOSOMAL STORAGE DISEASE

17 ERTs have been approved for 12 lysosomal storage diseases with a remarkably high approval rate

COMPELLING PROOF OF CONCEPT FOR ERT IN GM1 ALREADY EXISTS

POC in two mouse models of disease
Substrate reduction to near normal levels
Behavioral analysis ongoing
No safety issues

BIOMARIN'S BRINEURA® PROVIDES BENCHMARK FOR GM1 ERT

ICV administered ERT for CLN2 Batten disease, a lysosomal storage disorder
Four years from IND to approval

FOUNDATION HAS INITIATED CMC WORK TO ENABLE IND

Have identified a high producing cell line
Currently manufacturing at small scale
Will scale up and generate material for IND-enabling tox and clinic
Pre-IND meeting planned for early 2026

WHAT WE CAN OFFER

- PROOF OF CONCEPT DATA IN TWO MOUSE MODELS
- CELL LINE FOR GMP MANUFACTURE
- EHR NATURAL HISTORY STUDY DATA
- RESEARCH-READY, MOTIVATED PATIENT COMMUNITY
- NATURAL HISTORY DATA SHARING CONSORTIUM
- NEWBORN SCREENING ASSAY
- RELATIONSHIPS WITH KOLs AND DISEASE EXPERTS

DIABETES LEADERSHIP COUNCIL

Driving Patient-Centered Diabetes Policy

DIABETES PATIENT ADVOCACY COALITION

Empowering Action on Diabetes Policy

- **What we are seeking:**
  - Mission-aligned partners
  - Legislative champions
  - Unified voices
  - Passionate advocates
- **Quick Facts:**
  - 51,000 + Registered Advocates
  - Centuries of Lived and Professional Experience
  - Representation in 50 States
  - Policy, Advocacy and Lobbying Engagement

- **Through legislative, regulatory, and administrative advocacy and partnerships, we are addressing:**
  - Pharmacy Benefit Manager (PBM) reform
  - Threats to Medicaid and Expansion
  - Cuts to Research Funding and Clinical Trials
  - Insulin and Prescription Affordability
  - Access to Obesity Treatment and Therapy
  - Prevention and Management of Complications
  - Early Diabetes Detection and Screening
  - State Legislator Education and Stewardship
  - Employer Risk Reduction and Benefits Design

(EB) RESEARCH PARTNERSHIP

EBRP has one bold audacious goal: cure the devastating and life-threatening genetic rare skin disease epidermolysis bullosa (EB) by 2030 and in the process, pioneer an innovative business model to lead the way for all rare disease

QUICK FACTS

- While EB is a rare disese, affecting ~500K worldwide, it is just one of 10K+ rare diseases impacting 400M people
- In the last decade, we've raised $75M, funded 160+ innovative research projects to heal EB ,advanced science and technology at large, and directly accelerated 3 FDA-approved treatments.

WHAT WE'RE LOOKING FOR

- Technology partners to advance our data platform
- Funders to help accomplish our mission and make rare disease history
- Researchers and biotech/pharma companies to apply for grant funding and drive research forward

KEY INITIATIVES

- Accelerate treatments and cures for EB
- Scale our Venture Philanthropy model to help additional rare diseases
- Launch a first of it's kind patient-driven data platform to curate the rare disease journey

WHAT WE CAN OFFER

- Innovative Venture Philanthropy model
- De-identified patient data to accelerate clinical trials and drug development
- Network of best-in-class medical centers specialized in rare, pediatric skin diseases

ebresearch.org | michael@ebresearch.org | @EBResearch | @EB Research Partnership
244 Madison Ave, Ste 104, New York, NY

emily whitehead foundation

Mission: To support patients and caregivers affected by childhood diseases and advocate for all patients who can be treated with advanced therapies

Inspiring Beginning

The Foundation is named for the **first child in the world** whose cancer was cured with CAR T-cell therapy. We are driven to turn Emily's survivor story into **tens of thousands more** survivor stories.

What We Do

- **Amplify patient stories** to connect patients with each other and with support
- **Quantify the patient experience** to inform and guide healthcare policymaking
- **Connect families with resources** through our Believe Bundle care packages
- **Empower via education** to help patients and families make informed decisions

Paved the way for **40,000+ patients** to receive CAR-T

The leading **voice of the patient** for advanced therapies

Advocating for the **80% of patients** who lack access

Join Us to Activate the Cure®

There are many ways to partner with the Foundation that can benefit both the cause and your organization.
Contact Kristine Barras, Director of Development, at kristine@emilywhiteheadfoundation.org

emilywhiteheadfoundation.org

EveryLife Foundation for Rare Diseases

EveryLife Foundation for Rare Diseases is powered by the rare disease community to improve health outcomes by driving change through evidence-based policy, leading science driven policy and regulatory research, activating the community to advocate for their rights and needs, and strengthening the rare disease community.

Quick Facts

1. **A nonprofit advocacy leader** activating rare disease patients and identifying policy solutions to addresses challenges threatening the rare disease ecosystem.
2. **A leader of driving change**, we continue to make significant strides advancing the equitable development of and access to lifesaving diagnoses, treatments, and cures, through policy, advocacy, and patient engagement in partnership with the rare disease community.
3. **Rare Diseases Week on Capitol Hill,** our signature initiative, brings together hundreds of advocates from across the country to learn about key legislative issues, connect with others in the community, and share their powerful stories directly with policymakers.

What we are looking for:

1. **New strategic partners in biotech/pharma**, especially those developing novel rare disease therapies or platforms with potential rare applications.
2. **Engagement with early-stage companies and emerging innovators** who are interested in patient-centered therapy development.
3. **Volunteer experts and advisory board members** interested in contributing to equitable access, early diagnosis, and policy impact.

Key Initiatives / Projects / Priorities:

1. Rare Access Initiative & Policy Research Fund
Generating and translating policy-relevant research to address systemic barriers, economic burden, and the cost of delayed diagnosis in rare disease.
2. Patient Engagement Programs
Includes Patient Navigator Tool and Bootcamp Series—empowering patients and caregivers with actionable knowledge to accelerate therapy development.
3. Ecosystem Gap Identification and Strategic Engagement
Leveraging our unique role as a trusted convener and leader in policy and patient engagement to uncover unmet needs in the rare disease ecosystem—building cross-sector solutions that advance equitable access, accelerate diagnosis, and ensure patient-centered therapy development.

What we can offer:

1. **Policy insight and thought leadership** concerning all federal healthcare agencies relevant to rare disease therapy development and access.
2. **Robust patient engagement infrastructure** through Patient Bootcamp, Rare Across America, Rare Disease Week, Navigator Tools, and a number of other patient engagement programs.
3. **National reach and convening power** through trusted relationships with hundreds of biotech and pharmaceutical partners, more than 200 disease specific patient advocacy groups, thousands of patients, caregivers, and advocates across all 50 states—enabling partner organizations to amplify their impact, validate tools or approaches, and shape programs with real-world insights from the rare disease community.

To learn more, reach out to Ted Brasfield, Vice President of Alliance Development, at tbrasfield@everylifefoundation.org

Friedreich's Ataxia Research Alliance

FARA is a non-profit, organization supporting scientific research leading to treatments and a cure for Friedreich's ataxia https://www.curefa.org/

Quick Facts

- Friedreich's ataxia (FA) is a genetic, progressive neuromuscular disease that affects balance and coordination and leads to life-altering loss of mobility. Other common symptoms can include fatigue, serious heart conditions, scoliosis, and diabetes. FA affects an estimated 5,000 individuals in the United States and 15,000 worldwide
- FARA funds discovery, translational and clinical scientists and facilitating their access to resources. Through funded grants, global initiatives, and partnerships with pharmaceutical companies, FARA can help propel research forward

What we are looking for

Award for Innovative Mindset (AIM)) supports innovative, high-risk, high-gain FA research. We are looking for new directions, new perspectives and projects that address neglected issues in FA research. The presentation of preliminary and/or published data is encouraged, but not required

The Kyle Bryant Translational Research Award funds projects that accelerating completion of translational stage activities necessary for advancement to clinical study of a therapeutic candidate or development of novel tools that address a critical bottleneck to the discovery or development of therapies.

General grants support basic, translational and clinical research with the goal of advancing therapeutic development in FA. All proposed research must fall within at least one of FARA's Grant Program Priorities.

Key initiatives / projects/ priorities

- Advancing understanding of neurological and cardiac disease in FA
- Advancing understanding of the molecular basis of FA
- Facilitating the drug development process and translational research
- Advancing clinical research

What we can offer

- In addition to research funding, FARA has supported the development of animal models, cell models, antibodies, biorepositories, and several large datasets https://www.curefa.org/research/research-resources/research-and-drug-development-tools/
- A global network of trial ready sites and longitudinal natural history data
- Biomarker data including TRACK-FA, a longitudinal neuroimaging study and data on digital biomarkers

FNIH
Foundation for the National Institutes of Health

The FNIH leads public-private partnerships that advance biomedical breakthroughs and improve lives

We accelerate new therapies, diagnostics, and potential cures

We advance global health and seek equity

We celebrate and train the next generation of scientists

What we are looking for:

- Partnerships
- Data and knowledge-sharing
- Team science approach

Learn more at FNIH.org

What we offer:

- Governance of public-private partnership model
- Program and budget management
- Ability to convene multiple stakeholders
- Proven track record

Our focus areas:

- Cancer
- Cardiovascular
- Infectious disease
- Inflammation & immunity
- Maternal & child health
- Metabolic disorders
- Neuroscience
- Rare disease

Foundation for Sarcoidosis Research

Founded in 2000, FSR is the leading international nonprofit dedicated to sarcoidosis patients and research on to find a cure for sarcoidosis and to improve care for patients with sarcoidosis. Sarcoidosis is a rare inflammatory disease that can impact any organ of the body. There are ~175,000 impacted in the US

Website: www.stopsarcoidosis.org **Contact us:** info@stopsarcoidosis.org

Accelerate Research
Through fellowships, pilot grant, large grant programs, clinical trial support, and FSR-led research efforts

Clinician Engagement
Through delivering educational, networking, and resources to help improve quality of care for sarcoidosis patients

Support Patients
Through patient advocacy program, patient navigators and support groups

Educate
Through webinars, town halls, podcast, and infographics

Advocate
For increased grant opportunities through NIH and pharmaceutical partners, access to care, and reduction of barriers

Key Programs

- **FSR Global Sarcoidosis Clinic Alliance:** The Foundation for Sarcoidosis Research Global Sarcoidosis Clinic Alliance (FSR-GSCA) is a member program consisting of clinics, hospitals, individual providers committed to finding a cure and offering evidence-based, patient-centric care for those living with sarcoidosis.
- **FSR Funded Research:** FSR has supported $8m in sarcoidosis research
- **FSR Data Ecosystem**: FSR has a patient-reported outcomes patient registry with over 7000 participants from 80 countries and is building a clinician data registry
- **Coalition to Transform Clinical Trial Engagement:** To collaborate on developing innovative strategies that transform the landscape of clinical trial participation for Black patients

What we can offer:

- **Patient Support**: Support Groups, 1:1 navigators, educational programming like webinars, seminars, townhalls
- **Clinical Trial Protocol Design and Recruitment Support:** FSR has successful supported the design and recruitment helping to enroll 5 clinical trials between 12-24 months faster than the average in the rare space.
- **Patient Data:** FSR has robust patient data and can support in patient journey mapping.
- **Policy and Leadership:** Opportunity to join an effective coalition that is changing the face of clinical trials, through policy, a corporate champion program

FSHD Global Research Foundation
GIVING LIFE TO MUSCLES

FSHD Global Research Foundation is Australia's peak patient advocacy body for Facioscapulohumeral Muscular Dystrophy.
Our mission is to find a treatment and ultimate cure for FSHD

Quick Facts

- FSHD is an autosomal dominant genetic condition that affects approximately 1 in 8000 children and adults globally.
- FSHD is a progressive, debilitating, degenerative, muscle wasting disease caused by a toxic protein and transcription factor, *DUX4*.
- There is not pharmaceutical therapy for FSHD.
- Since 2007 FSHD Global Research Foundation has invested over $25M in research across 11 countries.
- We support patient advocacy, fund medical research grants, invest in biotechnology companies, and build infrastructure for clinical trials.

What We Are Looking For

- To partner with academics, biopharmaceutical companies and foundations who are investing in FSHD research to advance key technology areas that enable and accelerate clinical development:
  (1) biomarker research to replace muscle biopsy
  (2) paediatric research
  (3) disease modifying therapies
  (4) whole-body MRI analytics for paediatrics and adults
  (5) muscle regeneration technology
  (6) digital health tech to generate real world evidence

Key Initiatives and Projects

- CureFSHD App - patient led registry and clinical trial passport
- Funding saliva DNA methylation analysis and whole-body MRI analytics for ANZ patients (50 complete, with >150 in progress).
- Investing in childhood early-onset FSHD studies.
- Investing in technology enablers: cell bank, gene therapy, 3D muscle in a dish; mouse and mini-pig models of FSHD.
- Partnership with Myologica to provide access to drug candidate screening in mouse models.
- Established a biotech spinout to commercialise therapeutics.

What We Offer: Partners

- Experienced, patient-led foundation, patient support, political advocacy, strong patient voice, co-design research
- Access to patient real word data
- Medical research grants
- Co-invest in pre-clinical and early clinical candidates
- Co-invest in enabling diagnostic, imaging, regenerative technologies
- Clinical trial feasibility and site identification
- Medical affairs and KOL mapping

A: PO Box A296, SYDNEY, NSW, 1235 Australia **P:** +612 8007 7037; **E:** admin@fshdglobal.org ; **CFN:** 20628 **ABN:** 79 128 037 614

Mission: To improve the lives of individuals and families impacted by liver disease through promoting innovation, encouraging collaboration, and scaling optimal approaches to help eradicate liver diseases.

What We Offer:

- Advocacy training
- Partnership and support
- Global network
- Education and resources

Who We Work With:

- Patients, caregivers, advocacy groups
- Clinicians and researchers
- Biotech and biopharma innovators
- Policy makers

PROGRAMS

Advanced Advocacy Academy (A3)

Activation Councils

Liver Action Network

Global Liver Health is Public Health Initiative

Policy Engagement

Connect with Us at BIO 2025:
Alyssa Davenport, adavenport@globalliver.org
GlobalLiver.Org

Mission: To educate policymakers and stakeholders about the barriers FDA processes and insurance frameworks create and perpetuate in rare and ultra-rare diseases.

Website: www.haystackproject.org **Contact:** kara.berasi@haystackproject.org

Quick Facts

Haystack Project is a 501(c)(3) nonprofit organization with a focus on patients with rare and ultra-rare diseases, as well as rare cancers.

Primary Focus: For almost a decade, Haystack has been the rare/ultra-rare umbrella organization focused solely on access, value and reimbursement issues so critical to our community. More recently, we were asked to bring some new perspectives to FDA issues, and after some initial success, **Dr. Janet Woodcock** joined our Board, and we now focus on two issues – FDA processes and insurance access related to rare.

Scope/Network: National umbrella organization with **140+ patient organizations** that represent various rare diseases.

Activities: stakeholder engagement, educational programs, policy development, and advocacy

What we are looking for:

Industry Sponsors to support our initiatives and work collaboratively toward common goals.

Patient Advocacy Organizations who want to join us in our efforts to improve the landscape for rare diseases and learn about or be involved in the work we do.

Collaborative Partnerships to support specific areas of interest such as patient education through our Speaker Series, patient-oriented value reports, and more.

Key Initiatives/Projects/Priorities

Innovation at FDA- *Collaborative work with Dr. Janet Woodcock* to modernize rare disease reviews – from endpoints and protocols to trial design and evaluation frameworks, modernizing FDA's approach to rare and ultra-rare populations.

Access Barriers in Rare Diseases- Reimbursement paradigms like DRGs or Shared Savings, 340B and IRA's QSSD all represent an understanding of common conditions, without regard to implications for coverage and payment for rare disease treatments finally available to our community.

PROTECT Rare Act- A bipartisan bill aimed at leveling the playing field for rare disease patients by recognizing off-label treatments as medically necessary care.

Patient Oriented Value Reports – Tailored, expert-led, in partnership with one of our members/rare diseases initiatives that enables patient data and input to drive substantive engagement with ICER, IRA negotiations, and payer UM, and more.

What we can offer:

Reimbursement and Regulatory Expertise: Only when you deeply understand a subject, can you explain it simply to those it affects. At Haystack, we invest in the experts that can talk to CMS and FDA, but also to our patients and families in a way that empowers them to both understand and take action.

Advocacy Expertise: Extensive experience in listening and creating policy solutions that reflect our community's experiences. Educating policymakers and informing legislation that that impact rare and ultra-rare disease communities.

Educational Programs: Our speaker series and educational resources inform stakeholders about the challenges and solutions in rare diseases, while also keeping our network of **140+ patient organizations** updated with timely insights.

Collaborative Networks: Access to a broad network of patient advocacy groups, policymakers, and healthcare professionals dedicated to rare and ultra-rare diseases.

healthywomen

HealthyWomen is dedicated to educating midlife women so they can make informed health decisions, advocate for themselves, and prioritize their health and wellness.

About Our Audience

We connect with women throughout their health span, reaching 12+ million women in the United States each year. We propel our audience to take action: 86% feel more informed and in charge of their health after reading our content.

Areas of Focus

Education & Awareness

We provide scientifically reviewed, evidence-based information that educates women on health topics.

Science & Innovation

We convene experts and encourage information sharing that aims to advance and elevate conversations.

Policy & Advocacy

We support and promote policy issues that are of importance to women's health.

What Sets Us Apart

Trusted & Timely

Our content is written by professional journalists and health educators and medically reviewed for accuracy.

Credible & Inclusive

We represent diverse communities in partnership with renowned experts and amplify all women's voices.

Legacy & Leadership

For 35+ years, we've been the nation's leading women's health nonprofit. We're run *by* women *for* women.

Hypertrophic Cardiomyopathy Association

Hypertrophic Cardiomyopathy Association®
Home for the Bighearted Since 1996

Mission

Providing support, advocacy, and education to patients, families, the medical community, and the public about hypertrophic cardiomyopathy and all thick heart muscle disorders, while supporting research and development of treatments and diagnostics.

Services of the HCMA

Bighearted Warrior Tour Meetings- +84,000 Interactions

Center of Excellence (CoE)- 56 COEs, 30 programs are in pipeline

Tales from the Heart- +150 Episodes

HCM Academy (medical professionals)- +73 Free CME Course, 20 Live Webinars, and over 187,000 engagements

Online Discussion Groups- 12 per month

Institute for Follicular Lymphoma Innovation (IFLI)

Promoting Innovation for Patients with Follicular Lymphoma
Website: IFLI - The Institute for Follicular Lymphoma Innovation

Quick Facts

- **Therapeutic areas: Follicular Lymphoma (FL)**
- **Mission**: Accelerate progress of innovative treatment options to extend the life expectancy of people with follicular lymphoma and ultimately develop a cure.
- **Vision:** Build leading FL network and strong partnerships to maximize available resources to fund the best initiatives in the world that can transform Follicular Lymphoma research and ultimately deliver a cure.
- **Principal:** Catalytic and collaborative investment to magnify the total dollars funded for FL more than IFLI's budget alone. Actively promote data harmonization, sharing of scientific results, publication of key insights, and maximization of access to results that arise from IFLI funded research.

What we can offer:

- Priority 1 : Catalytic **Equity Investment to Biotech Ventures**
- Priority 2 : **Grant to Academic Research** in collaboration with other foundations
- Priority 3 : **Key stakeholder networks** and **expertise** – scientific, clinical, academic, commercial investment partners

Key initiatives / projects/ priorities (What we are looking for)

- **Investment to Biotech Ventures ($1M to $15M)**
  - Partner with start-up/biotech to accelerate the development of novel therapies and platforms specific to FLIdentify the most promising novel approaches/technology platforms specific to FL
  - Encourage Biotech & Pharma to include FL in their Clinical Trials
  - Identify the best AI-powered drug repurposing companies
- **Data Repository**
  - Develop and/or secure a platform for centralize & integrate FL results while preserving data privacy to provides access to expertise, and increase the potential for impactful research findings (in progress)
- **Clinical Research**
  - Biomarker Discovery : Identify prognostic/predictive biomarkers of disease progression & transformation to inform treatment selection
  - Disease Monitoring
    - Evaluate performance of existing FL disease monitoring tests, such as MRD
    - Develop novel approaches to evaluate treatment response, improve near term patient outcomes and enable faster clinical trials
- **Basic Research**
  - Unravel mechanistic understanding of disease heterogeneity to better define patient's prognosis
  - Identify the stem cell responsible for relapse, improve the understanding of factors driving treatment resistance/relapse

LGS FOUNDATION®
LENNOX-GASTAUT SYNDROME

Vision
End the devastation and suffering caused by Lennox-Gastaut Syndrome (LGS).

Mission
Improve lives of those impacted by LGS through research, education, awareness & family support.

Impact at a Glance
- 🌎 12,000+ families • 23 countries
- 💰 Over $2 M research funded
- 📊 97 % of revenue to mission programs
- 🧠 300+ clinician & researcher advisors
- 📈 Learn from Every Patient (LEP) natural-history study: caregiver-reported outcomes being collected on 1,000 LGS patients via NORD IAMRARE®

How we Support Drug Development
- ✓ Patient journey and LGS/DEE Landscape knowledge
- ✓ Natural history study data
- ✓ Investor and analyst diligence
- ✓ Co-author studies on unmet need
- ✓ Clinical trial awareness & media outreach
- ✓ Study protocol and consent design
- ✓ Site identification
- ✓Attend PI meetings, regulatory meetings, etc.
- ✓ Payer engagement
- ✓ Patient facing materials . . . and more . . .

What We're Looking For
- Biopharma / biotech partners for disease-modifying therapies
- Academics and research institutions
- Advocacy allies to amplify patient voice
- Patient-centric trial design & advisory roles
- Funding or in-kind support for programs & research

Connect: Tracy Dixon-Salazar, PhD | Executive Director | tracy@lgsfoundation.org

Massé World

Massé World is a global nonprofit empowering people with albinism and vitiligo through education, health access, advocacy, and awareness. We fight stigma, promote inclusion, and ensure equitable care and opportunity for all.

www.masseworld.org / info@masseworld.org

Quick Facts

- **Founded:** 2022
- **Headquarters:** Tampa, FL, USA
- **Regions Served:** Africa, Southeast Asia, and the United States
- **Population Focus:** Individuals with **albinism and vitiligo**, especially women, children, and marginalized populations
- **Therapeutic Areas:** Dermatology, Mental Health, Reproductive Health, Rare Genetic Conditions
- **Primary Focus:** Education, Advocacy, Access to Care, Policy Influence, and Equity-Driven Research

What we are looking for

- **Priority 1: Strategic Partnerships in Therapeutic Research**
- **Priority 2: Funding & Philanthropic Investment**
- **Priority 3: Policy & Advocacy Allies**

Key initiatives/ projects/ priorities

- **Priority 1: Health Equity for Skin Conditions**
- **Priority 2: Mental Health & Social Support**
- **Priority 3: Women & Girls Empowerment**

What we can offer

- **Priority 1: Access to Global Patient Communities**
- **Priority 2: Public Education & Awareness Campaigns**
- **Priority 3: Training & Field Programs**

National Alliance for Caregiving (NAC)

WHERE LEADERS IN CAREGIVING COLLABORATE

NAC harnesses the power of **stories and data** to champion the policy, system, and culture change needed to make **family caregivers a national priority.**

Learn more: www.caregiving.org
Connect with us: Adali Hernandez – *Adali@caregiving.org*

Quick Facts

- **Founded in 1996** | Based in Washington, D.C.
- **Known for Caregiving in the U.S.** a leading data project tracking family caregiver experiences nationwide, in partnership with AARP
- **We use research, data, and advocacy** to drive policy and systems change that supports caregivers
- **We do not provide direct services.** Our focus is national-level impact

Key Programs & Priorities

- **Caregiving in the U.S.**-Flagship ongoing data project capturing the evolving realities of family caregivers
- **Policy & Advocacy**-Shaping federal and state policy to improve caregiver health and access to services
- **Health Systems Transformation**- Embedding caregivers into healthcare systems enabling culture change through the:
  - ✓ Cancer Caregiving Collaborative
  - ✓ Transplant Caregiving Collaborative

What we are looking for:

- **Leadership & Expertise** – Partners who can bring a corporate lens to caregiving — offering insights and innovation to shape solutions.
- **Strategic Amplification** – Organizations willing to elevate NAC's mission through brand alignment and thought leadership.

What we offer:

- **NAC Membership-**Corporate engagement with thought leadership and visibility
- **Caregiver Nation Summit** -Annual policy and advocacy convening
- **Program Sponsors**-Funders advancing key NAC initiatives
- **Caregiver Nation Network-**Grassroots and state-based mobilization
- **Caregiving in the U.S**.-Flagship data project powering insights, infographics, and storytelling
- **Caregiver Voices-**Storytelling aligned with key data themes

Contact Information

Del Baker-Robertson,
Director Strategic Relationships,
dbakerrobertson@nhcouncil.org
202-903-9225

Partner with the National Health Council Advancing Patient-Centered Innovation

Who We Are

The National Health Council (NHC) unites 180+ patient advocacy groups, nonprofits, and cross-sector partners – including biotech, pharma, and MedTech - to advocate for and represent the 160+ million people with chronic diseases and disabilities.

What We Do

The National Health Council advances federal policy to reflect patient needs, supports innovation and access, leverages patient-centered evidence and value frameworks, and advocates for affordable, equitable care.

What We Offer

We offer deep insight into patient priorities across conditions, are a trusted platform for multi-stakeholder collaboration, and influence federal regulatory and legislative policy .

Let's Collaborate

We seek partners committed to improving access for patients, advancing RWE, and embedding the patient voice in innovation.

PROPIONIC ACIDEMIA FOUNDATION

Website: pafoundation.com
Email: paf@pafoundation.com
Join us in accelerating a cure for PA!

Quick Facts

- **Mission:** Dedicated to finding improved treatments and a cure for Propionic Acidemia (PA), a rare metabolic disorder caused by propionyl-CoA carboxylase deficiency.
- **Impact:** Funds cutting-edge research, supports families, and provides resources to medical professionals.
- **Achievements:** Awarded over $1.5M in 30+ research grants. Hosted multiple conferences (e.g., fall 2023 conference attended by 300 from 12 states).
- **Vision:** A future where PA can be prevented or cured, enabling affected individuals to live productive lives.

Seeking Collaborators at BIO 2025

- **Biotech/Pharma Companies**: Partners for novel therapeutics (e.g., gene therapy, enzyme replacement, small molecules).
- **Academic Researchers**: Experts in metabolic disorders, gene editing, or mitochondrial function for innovative seed projects.
- **Medical Food Providers**: Collaborators to develop specialized nutrition solutions for PA patients.
- **Patient Advocacy Groups**: Allies to amplify rare disease advocacy and share resources.
- **Technology Innovators**: AI/ML to enhance treatment monitoring or novel treatments.

We accelerate access to healthcare through financial assistance, advocacy, and education.

panfoundation.org

Quick facts:

- Since 2004, we've provided **over $4.5 billion** in financial assistance to **more than 1.3 million** people across more than 80 disease areas.
- We've **mobilized thousands** of patient advocates and achieved major policy victories, including helping to move the Part D reforms from the Inflation Reduction Act across the finish line, which limits out of pocket obligations to $2,000.
- We've **educated millions** of people on critical healthcare-related topics.

Key initiatives/projects/priorities:

- Offer medication copay and premium assistance to eligible patients across more than 80 disease areas.
- Advocate for improved healthcare access, affordability, and equity for all through online campaigns, partner engagement, and our in-person Advocacy Action Summit.
- Provide education and patient financial solutions on complex topics such as clinical trials, Medicare reforms, navigating financial assistance, and more.

What we're looking for:

- **Pharmaceutical/biotech companies** interested in learning more about ways to support PAN
- **Providers/pharmacies** interested in learning more about our financial assistance programs.
- **Patient advocacy organizations** interested in partnership around our advocacy and education initiatives.

What we can offer:

- Financial assistance to help cover copay, premium, and transportation costs.
- Opportunities to effectively advocate for key policy changes that improve healthcare access and affordability.
- Training and educational resources for patients, caregivers, and healthcare professionals.

The National Bleeding Disorders Foundation Venture Fund, Pathway to Cures, invests in emerging biotech companies developing cures, therapies, or enabling technologies in support of the inheritable blood and bleeding disorders community.

PATHWAY TO CURES
NATIONAL BLEEDING DISORDERS FOUNDATION

PATHWAYTOCURES.ORG

Inheritable blood and bleeding disorders include rare and ultra-rare blood disorders, hemophilia A and B, von Willebrand disease, Sickle Cell disease, anemia, clotting disorders, and other hematological disorders affecting more than 20 million people worldwide.

First-in-class therapeutics
or novel therapeutic mechanisms with potential to cure inheritable blood and bleeding disorders.

Novel, precision diagnostics, including biomarkers,
with potential to enable earlier or more accurate diagnoses to close health equity gaps.

Novel technologies
with potential to revolutionize treatments or disrupt the conventional care model in ways that significantly close equity gaps.

Teri Willey
Managing Director
twilley@pathwaytocures.org

Tim Brent
Venture Principal
tbrent@pathwaytocures.org

Progeria Research Foundation

FOR THE CHILDREN ♥ FOR THE CURE

The Mission of The Progeria Research Foundation is to find treatments and The Cure for patients living with Progeria

Hutchinson-Gilford Progeria Syndrome (HGPS)

- Ultra-Rare (1 in 20 million, prevalence) pediatric, rapid aging disease
- Caused by a sporadic (not inherited), autosomal dominant, single base mutation in the gene for Lamin A, a structural protein of the nuclear membrane
- 90% of progeria patients have the **exact same mutation** (1824 C > T) making the disease a perfect candidate for genetic-based therapies
- Patients are born appearing normal and are usually diagnosed by age 4 due to growth failure, skin abnormalities and characteristic facial features
- Without treatment, patients live 14.6 years (on average)
- Treatment with lonafarnib increases life expectancy by approximately 5 years

The Progeria Research Foundation (PRF)

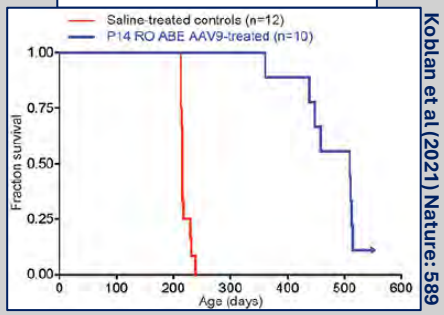
- A scientific research-focused patient advocacy organization founded in 1999
- Works to identify and support HGPS and Progeroid Laminopathy patients worldwide
  - Currently in contact with patients in over 50 countries speaking 36 languages
- Maintains a patient registry (> 390 patients) , Medical and Research Database and is conducting Natural History Studies (Boston Children's Hospital) to support clinical trials
- Has funded basic research at > 50 institutions in 14 countries
- Executes our own clinical trials and provided all clinical data submitted to FDA/EMA/PMDA for the approval of lonafarnib, the only approved therapy for Progeria

Why is PRF Attending BIO?

1. Raise awareness within the Pharma/Biotech community of the advanced pipeline of therapies in development at PRF and the exceptional in-house patient access and clinical development capabilities
2. Identify partners who are interested in **Base-Editing Gene Therapy (BEGT),** are committed to the Rare Disease patient population and want to join us in our mission to finding The Cure

   PRF hopes to enter the clinic with our Base-Editing Gene Therapy in < 18 months and we are looking for partners with complementary capabilities such as manufacturing, regulatory, clinical supplies and Quality Assurance
3. Meet with companies with leading **RNA technology platforms** that could be applied into developing novel, safe and effective therapies for HGPS

Efficacy of PRF's BEGT in the Mouse Model of Progeria



Koblan et al (2021) Nature: 589

Why Partner with PRF?

Well-funded patient advocacy organization that operates like a small biotech

- Extensive drug development experience in the ultra-rare disease space
- Significant internal capabilities in pre-clinical and non-clinical research that can be brought to the partnership
- World experts in Progeria and Progeroid Laminopathies

Our Vision is a world in which every child with Progeria is Cured

www.progeriaresearch.org

Contact:
Barbara Natke, CBO
bnatke@progeriaresearch.org

Rescue 7 Firefighters for Patients

We're here when you need us.

Rescue 7 Firefighters for Patients impacts society through integrity, compassion, and public service rooted in our decades of experience in the Fire Department, Police Departments, and Military Branches across the country. We provide **free** lodging, food, and transportation for families during clinical studies and long-term care. ***Rescue 7*** also supports advocacy groups through First Responder charity events. To learn more visit: Rescue7.org and Click here to see a video example of our work.

Quick Facts

- We are disease agnostic and completely remove the burden of transportation for families and patients traveling to participate in clinical studies or long-term treatment.
- R7 picks up families at airports in big red trucks. We bring patients and families to every study visit and treatment.
- Currently serving hospitals and patients in New York and Boston.
- We are completely staffed by first responders.

What We're Looking For

- Funds to increase staff and capacity
- Funds for three new vehicles to expand services to Chicago, Texas, and Los Angeles.
- Advocacy groups that can use our help in raising awareness and resources.
- Resources to secure free housing for patients traveling for study participation or treatment.

Key Initiatives / Programs

- Sibling programs at firehouses.
- Respite opportunities for family members/ care partners.
- Create a positive experience for patients and families.
- Sports events / training competitions with other fire /police depts. with 100% of ticket sales benefitting advocacy groups who call for a "rescue."
- Bring trucks, staff, activities, and enjoyable experiences to advocacy group conferences.
- Work closely with study teams to create precision patient programs.

What We Offer

- Every event and service for patients is 100% free of cost.
- 100% of ticket sales from any event goes directly to patient advocacy groups.
- We work closely with clinicians to synch schedules, and we make sure every patient arrives on time in the comfort of our specified fire vehicles which fits 3 caregivers, is wheelchair accessible and has plenty of room for luggage.
- Sibling programs at firehouses.
- Cost-free, healthy food during long days
- The experience of participating in treatment is less stressful, easier, and even fun.

There is a mental health emergency in rural America.

Quick Facts:

- Higher depression and suicide rates 64 to 68 percent higher than those in large urban areas.
- Severe shortage of healthcare providers that are further away.
- Broadband internet that can be unreliable or nonexistent– telemedicine is often not an option.
- Greater stigma around mental illness and a common mindset of self-reliance and independent approach to solving problems can create an insurmountable barrier to getting help.
- Rural youth are at greater risk of suicide, which is growing at a faster pace than in urban areas.

Key Initiatives / Projects / Priorities:

- Rural Mental Health Resilience Program toolkit; rural youth program is in development.
- Rural Mental Health Connections webinar series.
- "Mental Health and Suicide Prevention in Rural America" free online course.
- "Closing the Mental Health Gap Among Rural Youth in the US" award-winning video.
- Rural loneliness and social isolation campaign.
- Rural Policy & Legislation that impact wellness and warrant recognition.

What are we looking for:

Partnerships and funding to develop culturally-relevant information and resources to confront the mental health challenges in rural America and the greater stigma that surrounds mental illness.

What we can offer:

- Collaborative development of programs and resources that support patient advocacy objectives and advance mental health equity in rural America.
- Distribution of engaging communication for people/patients living in rural America.
- Diverse grassroots network of rural mental health stakeholders.

RURAL MINDS™

Rural Minds | www.ruralminds.org

Rural Minds (www.RuralMinds.org) is the only national 501c3 nonprofit focused on advocating for rural mental health equity, promoting mental wellness, and providing educational content to confront rural mental health challenges and the stigma that surrounds mental illness. We serve the 46 million people living in farming, ranching, and agriculture communities, small towns, and isolated areas across rural America.

Contact:

Jeff Winton
Founder and Chairman
Jeff@RuralMinds.org
908-872-2682

Chuck Strand
Executive Director
Chuck@RuralMinds.org
708-990-2774

S&PAA: The only organization focused solely on the community facing schizophrenia and psychosis

Schizophrenia & Psychosis Action Alliance

Founded in 2008, S&PAA advances systemic change and promotes recovery through research, education & care and advocacy & public policy to improve the lives of those with schizophrenia and psychosis spectrum disorders and their loved ones. Currently serving over 15,000 individuals across the world.

Our cornerstone programs:

- Bring best-practice treatment to the forefront
- Advocate for equal access to care
- Empower the community with knowledge, skills and support
- Fight discrimination and stigma

- Schizophrenia affects *at least 1 in 100 people in the United States* – about 2 million adults; there is a need for innovative treatments
- S&PAA partners with pharmaceutical and biotechnology companies to bring the patient voice to medical product development
- Past projects include
  - Focus groups to understand negative symptoms and use of phone apps
  - Interviews to inform the design of adolescent schizophrenia trials
  - Questionnaires to better understand different aspects of the lived experience

We believe a cure for schizophrenia and psychosis disorders is possible

Society for Women's Health Research

Society for Women's Health Research

A national thought leader dedicated to advancing women's health and promoting research on sex differences to optimize women's health

www.swhr.org

@SWHR

Quick Facts

- Women's health research is the study of sex & gender differences and how these affect disease risk, symptoms, diagnosis, treatment.
- Until the 1990s, most health research was conducted on men. This longtime bias created a huge gap in knowledge about women's health.
- SWHR is:
  - A non-profit with strategic pillars in science, policy, education
  - Located in Washington, DC – with a national reach
  - Celebrating its 35th anniversary in 2025; was founded in 1990

What we are looking for

- General & Program funders for our science and policy work
- Collaborative partners in diverse areas of women's health
- Support & Sponsorships for our Annual Awards Gala

Learn more at https://swhr.org/get-involved/.

Key Programs

- Programming in Alzheimer's disease, menopause, bone health, maternal health, uterine health, and more!
- Women's Health Dashboard
- Women's Heath Equity Initiative

Latest Resources

- Women's Health Policy Agenda
- Women's Health Research Agenda
- Menopause Workplace Guides For Women & Managers

What we can offer

- Partnership on science and policy programming, including roundtables, webinars, and educational resources
- Free resources on 28+ health conditions for women, including 70+ hours of webinars
- Access to the SWHR Working Group network, of 375+ women's health experts across the globe
- Collaboration with the team of women's health champions on SWHR's staff

TargetCancer Foundation (https://targetcancer.org/)

TargetCancer Foundation (TCF) works to advance the development of more effective and personalized treatments for people with rare cancers.

Quick Facts

- **Population served: Primarily rare cancer patients, caregivers, and researchers, but TCF works across all rare cancer stakeholders, also including clinicians, industry, and regulators.**
- **Primary focus: Supporting the development of effective and personalized treatments for patients with rare cancers by driving innovative research, connecting the rare cancer community, and empowering patients and caregivers.**
- **Key facts: Founded in 2009 by a patient after his diagnosis with cholangiocarcinoma, which had no available treatments.**

What we are looking for:

- **Opportunities for partnerships and collaboration with other rare cancer stakeholders.**
- **Opportunities to raise awareness of rare cancers and the challenges consistent among them.**
- **Support for our work through financial contributions or other means.**
- **Interested collaborators to attend our annual Think Tank on Advancing Precision Medicine in Rare Cancers and Industry Roundtable**
- **Outreach to the rare cancer patient community to drive enrollment in our TCF-001 TRACK clinical trial.**

Key initiatives / projects/ priorities

- **TCF-001 TRACK, a fully decentralized, patient driven, national precision genomics clinical trial for rare cancers. TRACK is remotely enrolling 400 patients with rare cancers and providing them with tissue and blood biomarker testing at no cost, as well as expert treatment recommendations from a Virtual Molecuar Tumor Board.**
- **Patient educational resources focused on the unique elements of navigating a rare cancer diagnosis.**
- **Annual Think Tank on Advancing Precision Medicine in Rare Cancers and Industry Roundtable, which convene key rare cancer stakeholders in Boston each fall.**
- **Specific efforts to advocate for the development of tumor agnostic treatment guidelines at NCCN and ASCO.**

What we can offer:

- **Priority 1: Enrollment in our TRACK clinical trial for patients with any rare cancer, offering them no-cost biomarker testing and expert treatment recommendations.**
- **Priority 2: Patient education and empowerment resources to ensure that patients with rare cancers can most effectively navigate and advocate for themselves.**
- **Priority 3: Over 16 years of experience, knowledge and networks in rare cancers, as well as connectivity and collaboration to advance precision oncology for rare cancers.**

Contact:
- Ada Lio, Executive Director, alio@zttk.org
- Nathan Guo, Scientific Director, nguo@zttk.org

ZTTK SON-Shine Foundation

Patient advocacy group developing the first treatments for ZTTK Syndrome, an ultrarare neurodevelopmental disorder

ZTTK.org
1040 6th Ave, 3rd Floor
New York NY 10018
A 501(c)(3) nonprofit corporation
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ZTTK Syndrome overview

- ZTTK syndrome is a monogenic disorder caused by heterozygous *SON* gene mutations, resulting in haploinsufficiency and loss-of-function
- 300+ patients identified since ZTTK was discovered in 2016
- Infant-onset disease characterized by:
  - Global delay or inability to walk, talk, or eat independently; mild to severe intellectual disability
  - Infant-onset seizures in 50% of patients
  - Multisystem impact, including congenital malformations
- *SON* performs many critical functions, including regulating pre-mRNA splicing, nuclear organization, controlling cell cycle progression, modulating transcription, and supporting ciliogenesis

Our patient advocacy group

- **Patient-driven mission:** Founded and led by ZTTK families seeking to empower individuals with ZTTK to thrive and live their fullest lives
- **Translationally focused:** Developed world's first patient-derived ZTTK iPSC model and mouse models to accelerate therapeutic discovery and validation
- **Recognized for patient advocacy leadership:** Invited to present innovative approach at the 2025 Broad Institute/Termeer Foundation and MassBio Rare Disease Day events

Current projects

Project	Partner
Patient registry data collection	citizen health, RARE-X
Parent/caregiver focus groups	ICON
Human metabolomics project	COMBINED
SON ELISA development	Ahn Lab, Hefrsink School of Medicine
Computational RNAseq drug repurposing	UNRAVEL BIOSCIENCES
Short isoform-blocking ASO screen	THE YU LAB, ThermoFisher SCIENTIFIC

How you can help

1. **Join our team:** Seeking paid full-time or fractional support in:
   - Basic and translational research strategy and execution
   - Fundraising, partnerships, and community engagement

   Please reach out if you are interested or forward to someone you know!
2. **2025 ZTTK Conference:** *Seeking volunteers and sponsors* for our first ZTTK Family and Science Conference August 9-10 in Cambridge
3. **iPSC Model Advancement:** Lead neuronal differentiation and characterization studies using our patient-derived ZTTK iPSCs
4. **Transcriptomic biomarker:** Develop SON-regulated RNA splicing assay