RAREVOICE Awards

THE 13TH ANNUAL

RAREVOICE AWARDS

DECEMBER 11, 2024

RONALD REAGAN BUILDING AND
INTERNATIONAL TRADE CENTER
WASHINGTON, DC

In honor of advocates who champion and amplify
the rare disease patient voice in state and federal policy

PRESENTED BY

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EVERYLIFE FOUNDATION FOR RARE DISEASES

RDLA Rare Disease Legislative Advocates

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VERTEX

RARE VOICE Awards

EVENT PROGRAM

Masters of Ceremonies

Nell Choi

Nathanael Milam II

Announcement of the RareVoice Award Recipients

Lifetime Achievement

Congressional Leadership

Federal Advocacy: Congressional Staff

Federal Advocacy: Patient Advocate or Organization

Federal Advocacy: Federal Agency, Individual, Team, or Office

State Advocacy: State Legislator

State Advocacy: Patient Advocate or Organization

Federal Advocacy: Youth and Teen

State Advocacy: Youth and Teen

Artist-To-Advocate

Diversity Empowerment: Patient Advocate or Organization

MASTERS OF CEREMONIES

NELL CHOI

Nell Choi is a 16-year-old patient advocate, speaker, and author. Diagnosed with Neuromyelitis Optica at age nine, Nell developed a passion for storytelling that led her to publish her first book at age twelve, called "My Hospital Story," to provide hope to others living with chronic illness. In 2022, Nell's elephant sculpture, titled "Elephant in the Room," became a RareArtist awardee. Since then, Nell has become involved in legislative advocacy, participating in both Rare Disease Week and Rare Across America, and she used her RareArtist piece to communicate her story to legislators. Nell is the first junior ambassador to The Sumaira Foundation for NMO. She has been a panelist at patient days and has moderated panel discussions with key opinion leaders at UCLA. Through her advocacy, Nell hopes to give voice to other young patients living with rare diseases.

NATHANAEL MILAM II

Nathanael Milam II is a relentless advocate, survivor, and voice for the rare disease community. Diagnosed with the rare and often misunderstood condition, Hemophagocytic Lymphohistiocytosis (HLH), after being misdiagnosed with Crohn's disease for over a decade, Nate's life changed profoundly when he received a life-saving bone marrow transplant in 2019. His journey of navigating a healthcare system unprepared for his rare and complex needs ignited a passion for advocacy and inspired him to ensure that no one in the rare disease community faces their battles alone.

Nate serves as an Ambassador for the Histiocytosis Association and Co-Chair of the Patient Advisory Board in partnership with the North American Consortium for Histiocytosis (NACHO), he formerly interned with RDLA and is an active member of the RDLA Advisory Committee and the EveryLife Foundation.

Nate's work extends beyond his own story, as he actively collaborates with organizations such as Global Genes, Johns Hopkins, and Children's National Hospital, where he has led a panel on the intersectionality of pride and rare, educated medical students, and initiated the production of Seacrest Studios. His leadership in advocacy has helped raise awareness, support, and funds for essential programs, including palliative care and funding for stem cell research.

LIFETIME ACHIEVEMENT AWARD

ROBERT M. CALIFF, M.D.,

Commissioner, U.S. Food and Drug Administration

Robert M. Califf, M.D., is Commissioner of Food and Drugs. President Joe Biden nominated Dr. Califf to head the U.S. Food and Drug Administration and Dr. Califf was sworn in on February 17, 2022. Previously, Dr. Califf served as Commissioner of Food and Drugs from February 2016 to January 2017. As the top official of the FDA, Dr. Califf is committed to strengthening programs and policies that enable the agency to carry out its mission to protect and promote the public health. Dr. Califf served as the FDA's Deputy Commissioner for Medical Products and Tobacco from February 2015 until his first appointment as Commissioner in February 2016.

Prior to rejoining the FDA, Dr. Califf was head of medical strategy and Senior Advisor at Alphabet Inc., contributing to strategy and policy for its health subsidiaries Verily Life Sciences and Google Health. He joined Alphabet in 2019, after serving as a professor of medicine and vice chancellor for clinical and translational research at Duke University. He also served as director of the Duke Translational Medicine Institute and founding director of the Duke Clinical Research Institute. A nationally and internationally recognized expert in cardiovascular medicine, health outcomes research, health care quality, and clinical research, Dr. Califf has led many landmark clinical trials and is one of the most frequently cited authors in biomedical science, with more than 1,300 publications in the peer-reviewed literature.

RAREVOICE Awards

Official RareVoice Correspondent

Stephanie Riordan

Follow the Rare Disease Legislative Advocates (RDLA) on Instagram @Rare_Advocates for behind the scenes content from our RareVoice correspondent.

ALEXION

COMMUNITY CONGRESS

The Community Congress is a membership-based program dedicated to bringing patient organizations, industry leaders, and other rare disease stakeholders together. The Congress acts as a coalition of collaborators with shared priorities, providing strategic guidance and insight on policy issues and initiatives. It comprises four permanent working groups that meet at least quarterly to advance federal and state legislative and regulatory policy solutions for the rare disease community.

Thank you to our 2024 Working Groups Co-Chairs and members for their collaboration and hard work on the different projects that help advance policy for the rare disease community.

For more information about Community Congress membership visit:
https://everylifefoundation.org/community-congress/

Thank you!

The EveryLife Foundation is grateful for the decades of leadership, dedication, and support from outgoing members of Congress that served on key healthcare-related committees. While in Congress, they have collaborated with patient advocates to advance policies that accelerate regulatory decision-making, drive research and drug development, and ensure the earliest and most equitable access to treatments and cures.

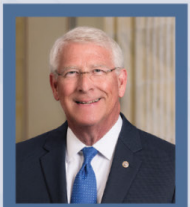
- Representative Kelly Armstrong (ND)
- Representative Earl Blumenauer (OR-3)
- Representative Michael Burgess (TX-26)
- Representative Larry Bucshon (IN-8)
- Representative Tony Cardenas (CA-29)
- Representative Jeff Duncan (SC-3)
- Representative Anna Eshoo (CA-16)
- Representative Drew Ferguson (GA-3)
- Representative Kay Granger (TX-12)
- Representative Dan Kildee (MI-8)
- Representative Ann Kuster (NH-2)
- Representative Jake LaTurner (KS-2)
- Representative Barbara Lee (CA-12)
- Representative Michelle Steel (CA-45)
- Representative Greg Pence (IN-6)
- Representative Cathy McMorris Rodgers (WA-5)
- Representative John Sarbanes (MD-3)
- Representative Brad Wenstrup (OH-2)
- Senator Mike Braun (IN)
- Senator Sherrod Brown (OH)
- Senator Ben Cardin (MD)
- Senator Joe Manchin (WV)
- Senator Mitt Romney (UT)
- Senator Debbie Stabenow (MI)
- Senator Bob Casey (PA)

RARE DISEASE CONGRESSIONAL CAUCUS

Thank you to our Rare Disease Congressional Caucus Co-Chairs and Members

Representative
Doris Matsui (CA)

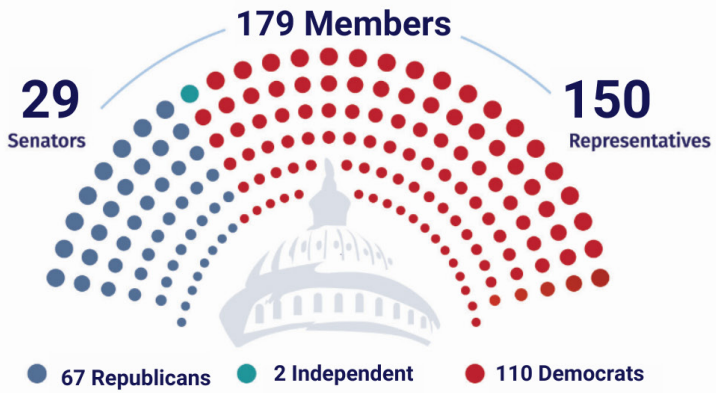
Representative
Gus Bilirakis (FL)



Senator
Roger Wicker (MS)

Senator
Amy Klobuchar (MN)

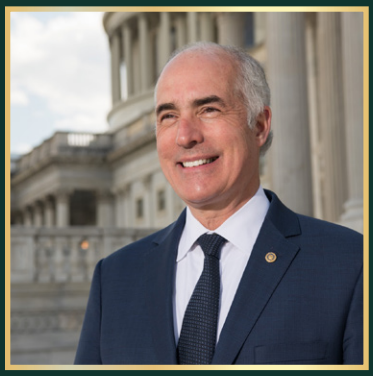
The Rare Disease Congressional Caucus is a bipartisan, bicameral caucus to voice constituent concerns, collaborate on ideas, facilitate conversations between the medical and patient community and build support for legislation that will improve the lives of people with rare diseases.



Ask your Member to join today.

RARECAUCUS.ORG

CONGRESSIONAL LEADERSHIP AWARD



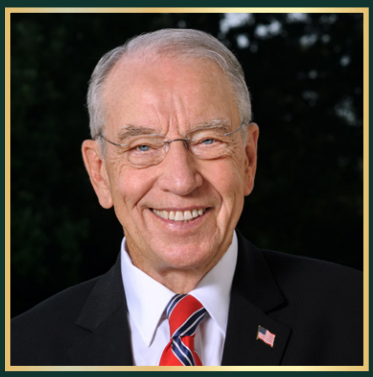
SENATOR ROBERT P. CASEY JR

Robert P. Casey Jr., Senator from Pennsylvania, has served since 2006. Born and raised in Scranton, Pennsylvania. After earning his law degree from Catholic University in 1988, he practiced law in Scranton before transitioning to public service.

As a member of the Committee on Health, Education, Labor, and Pensions and the Committee on Finance, Senator Casey plays a pivotal role in shaping policies that expand access to health care. A dedicated advocate for the rare disease community, he has led significant initiatives to advance the development of therapies for rare diseases. Notably, he spearheaded the reauthorization of the *Pediatric Rare Disease Priority Review Voucher (PRV) Program* in the Senate as the lead sponsor of the *Creating Hope Reauthorization Act*.

Senator Casey is also a champion for individuals with disabilities. He served as the prime Senate sponsor of the *Stephen Beck Jr. Achieving a Better Life Experience (ABLE) Act*, landmark legislation that enables millions of families to save for the long-term care of loved ones with disabilities through tax-advantaged savings accounts.

Through his leadership, Senator Casey continues to drive meaningful change, improving the lives of individuals with rare diseases and disabilities across the nation.



SENATOR CHUCK GRASSLEY

Senator Chuck Grassley, the longest-serving U.S. Senator in Iowa's history, is known for his diligence, reliability, and commitment to bipartisan leadership.

In 2020, Senator Grassley joined the Rare Disease Legislative Advocates (RDLA) Rare Disease Congressional Caucus, a bipartisan, bicameral group dedicated to addressing policy challenges faced by individuals with rare diseases.

A steadfast advocate for rare disease communities, Senator Grassley sponsors the *Accelerating Kids' Access to Care Act*, ensuring pediatric Medicaid patients can receive timely care across state lines—a critical measure for rare disease patients requiring specialized treatment. He has also championed other initiatives, including serving as the Republican lead on the *Medical Nutrition Equity Act* during the 115th Congress, advancing access to medically necessary nutrition for patients with rare conditions.

Through his leadership, Senator Grassley continues to amplify the voices of those affected by rare diseases, driving meaningful policy change to improve lives.

EVERYLIFE FOUNDATION FOR RARE DISEASES

"If renewing [the PRV] program means better distribution and continued development of these life-saving drugs and treatments for kids with rare diseases, then it's vital that we continue it."

– **Vanessa Werner**

Pennsylvania Mother and
Pediatric Rare Disease Advocate

If the PRV program expires on December 20th, hopes for promising treatments for kids will be dashed.

#RenewPRV #Cures4RareKids

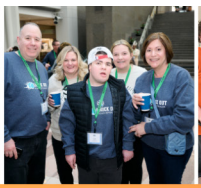
Congress must act now! Rare disease families are counting on you. **everylifefoundation.org/prv**

Advocates — make sure Congress hears your voices. **Take action: bit.ly/RareKids**

EVERYLIFE FOUNDATION FOR RARE DISEASES

15TH ANNIVERSARY

#EveryLife15for15



For 15 years, the EveryLife Foundation for Rare Diseases has been a leader of driving change by activating the patient advocate, changing public policy, and saving lives. We've made significant strides in advancing the equitable development of and access to lifesaving diagnoses, treatments, and cures, through policy, advocacy, and patient engagement—work that would not have been possible without the support of our community.

As we celebrate this milestone, we invite you to join us in our "EveryLife 15 for 15" campaign
Below are three ways you can make a difference.

SHARE YOUR STORY

MAKE A GIFT

BECOME AN AMBASSADOR

SCAN TO LEARN MORE

FEDERAL ADVOCACY CONGRESSIONAL STAFF

JACQUELINE BAGGETT

(Office of Representative Brian Fitzpatrick)
Joe Fiandra Access to Home Infusion Act

Jacqueline Baggett currently serves as Legislative Director for Congressman Brian Fitzpatrick. She has been working on health policy for the Congressman for over three and a half years. Additionally, Jacqueline staffs the Congressman on all Ways and Means Committee policy priorities, including tax, trade, Social Security, and oversight.

Prior to working with Congressman Fitzpatrick, Jacqueline worked at the White House in the Office of Science and Technology Policy and for Congressman George Holding of North Carolina. She is in her last year of law school at American University Washington College of Law where she attends class in the evening.

SARAH GILBERT

(Office of Representative Neal Dunn)
Joe Fiandra Access to Home Infusion Act

Sarah Gilbert grew up in Wilmington, North Carolina and attended the University of North Carolina at Chapel Hill where she double majored in Economics and Global Studies and minored in Spanish. She has worked in health policy roles for Members of Congress who served on the House Ways and Means Committee and the House Energy and Commerce Committee. She currently serves as Chief of Staff to Congressman Neal Dunn (FL-02).

SARA MASKORNICK

(Senate Committee on Health, Education, Labor, and Pensions)
Creating Hope Reauthorization Act

Sara Maskornick serves as the Democratic Staff Director of the Subcommittee on Children and Families of the Senate Committee on Health, Education, Labor and Pensions under Pennsylvania Senator Robert P. Casey, Jr., for whom she has been working since 2006. As Staff Director, Sara oversees Senator Casey's work on public health policy, children's issues, and family workplace issues. She has worked on legislation on public health preparedness, campus sexual assault, reasonable accommodations for pregnant workers, incentivizing new drug development for rare pediatric diseases, reforming the Food and Drug Administration's processes for regulating over-the-counter drugs and reviewing combination products, improving protections for infants born affected by substance use disorder and improving emergency medical services for children. Sara is a graduate of Wellesley College, and received her Master's in Public Policy from The George Washington University. She lives in Alexandria, VA with her husband and daughter.

ALEXANDRA KARABATSOS

(Office of Representative Lori Trahan)
Accelerating Kids' Access to Care Act

Alexandra (Alex) Karabatsos serves as the Legislative Director for Congresswoman Lori Trahan, where she has played a key role in shaping and advancing a range of critical healthcare policies. Her leadership has been pivotal in advancing the Accelerating Kids' Access to Care Act, a transformative bill designed to streamline cross-state access to life-saving healthcare for children enrolled in Medicaid, ensuring they receive timely and specialized treatments.

In addition to her work on pediatric healthcare, Alexandra is deeply involved in legislative efforts surrounding the Creating Hope Act, which provides crucial incentives for the development of treatments targeting rare pediatric diseases. This initiative aims to stimulate pharmaceutical innovation and bring life-saving therapies to children suffering from conditions that currently lack effective treatments.

Through her work, Alex continues to drive meaningful changes in healthcare policy, particularly in areas that address the needs of underserved communities and children in critical medical need.

FEDERAL ADVOCACY: PATIENT ADVOCATE OR ORGANIZATION

KELLY BERGER

Accessible Air Travel Proposal Rule & FAA Reauthorization; HELP Copays Act; BENEFIT Act; Safe Step Act; and RARE Act

Kelly Berger is a graduate of the University of Kentucky with a B.A. in Journalism and a concentration in Online Media. Kelly is the Outreach and Engagement Coordinator for the rare disease nonprofit, Cure CMD. She lives with Congenital Muscular Dystrophy (CMD) and is honored to work for an organization she's so closely affiliated with.

Kelly is very passionate about being a positive role model in the disability community and helping others. She recently began training in an Enabling Access Program: Ohio Mentorship Project, becoming an official peer mentor for others with disabilities in Ohio.

She was awarded 2023 Ms. Wheelchair Ohio Ambassador for her rare disease inclusion advocacy work. Her platform was to continue garnering support for under-represented groups. Kelly recently received a nomination for a Global Genes 2024 RARE Champion of Hope Award in Individual Advocacy for her strides this past year in advocacy.

KELLY CONSIDINE

Proposed Amendment to SUPPORT Act

Kelly Considine developed Complex Regional Pain Syndrome in her right leg in 2005 after an ankle sprain playing volleyball and it has since spread to both arms. She has become a fierce advocate for CRPS Warriors across the globe. Kelly has taken on many roles within RSDSA over the years, including chairing the 2nd-5th Annual Virtual CRPS Awareness Walks, her membership on RSDSA's Development Committee, Advocacy Committee, and Patient Protective Task Force, and she's the Patient Representative on the Practical Pain Management Advisory Board. Kelly is an Adjunct Chemistry Professor at her alma mater, Sacred Heart University. She has served on the EveryLife Foundation for Rare Diseases' RDLA Advisory Committee and has participated in Rare Across America and Rare Disease Week for 3 years. Kelly has participated in a press conference with legislators regarding ADA accessibility and has received proclamations for the past 4 years from the Mayor, Connecticut General Assembly, and Governor for CRPS Awareness Month.

ALYSSA CHACON

Safe Step Act

Ally Chacon is a caregiver, advocate for the Hereditary Angioedema (HAE) community, and a devoted big sister. Her journey began in 2009 when her brother was diagnosed with HAE, inspiring her to strive to be the best caregiver and advocate possible. As the oldest daughter, Ally learned to care for her siblings before she could even read.

Beyond her HAE advocacy, Ally has gained experience in various fields, including a recent role in the professional culinary world following a year of teaching special education. She enjoys traveling, particularly for advocacy opportunities and events, but also cherishes time spent at home with her dog and partner. Ally values her friends and family deeply and treasures every moment she can spend with them.

HEREDITARY ANGIOEDEMA ASSOCIATION

Accelerating Kids' Access to Care Act

The US Hereditary Angioedema Association (HAEA) is a 501(c)(3) nonprofit organization dedicated to supporting individuals with Hereditary Angioedema (HAE) and their families. Founded and staffed by people with HAE and caregivers, the HAEA promotes education, awareness, and access to modern treatments.

They offer personalized services, assist with securing access to HAE medications, and advocate for tailored, patient-focused care. Their support for clinical research has led to multiple FDA-approved therapies, and they work with expert physicians to improve diagnosis, treatment, and quality of life. As a neutral organization, the HAEA continues to champion research for next-generation HAE therapies.

SOLER FAMILY

New Era of Preventing End Stage Kidney Disease Act

Angelica, Ariana, and Marcus Soler, have dedicated themselves to advocating for a cure for nephrotic syndrome, a rare genetic kidney disease that Marcus was diagnosed with at the age of two. Their unwavering commitment has driven them to support NephCure Kidney International by attending events and hosting impactful fundraisers, such as the "Pig Jet," inspiring their community to join their cause.

Through charity galas, local initiatives, and passionate advocacy, the Solers have raised critical awareness and funds for rare kidney diseases. They are also vocal champions of the "New Era Act," a bipartisan federal bill designed to improve outcomes for individuals living with rare kidney conditions. The family remains hopeful that this legislation will move forward in Congress and become law, ultimately alleviating the need for future advocacy and creating a better future for others impacted by rare kidney diseases.

FEDERAL ADVOCACY: FEDERAL AGENCY, INDIVIDUAL, TEAM, OR OFFICE

ENHANCING DATA-DRIVEN DISEASE DETECTION IN NEWBORNS (ED3N) TEAM

Centers for Disease Control and Prevention

The Enhancing Data-driven Disease Detection in Newborns (ED3N) program aims to provide a national data platform that will improve the quality and interpretation of newborn screening results. They will assess the functionality of a CDC data platform in the Division of Laboratory Sciences Newborn Screening and Molecular Biology Brance (NSMBB). The project aims to help improve disease detection in newborns through dried blood spot screening.

Through collaboration between U.S. newborn screening programs and CDC's Newborn Screening and Molecular Biology Branch, the ED3N pilot project will help programs evaluate disease risks. The NSMBB project will pilot two modules within ED3N.

OFFICE OF ORPHAN PRODUCTS DEVELOPMENT (OOPD)

Food and Drug Administration

The FDA Office of Orphan Products Development (OOPD) supports and advances the development and evaluation of new treatments for rare diseases. OOPD evaluates information from product sponsors to determine if drugs, biologics, or medical devices meet the criteria for certain incentives and administers grants to provide funding for research on rare diseases. The office also works on rare disease issues with medical and research communities, professional organizations, academia, government agencies, industry, and rare disease patient groups.

DR. ALIZA THOMPSON

Food and Drug Administration

Aliza Thompson is Acting Director of the Division of Cardiology and Nephrology, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration (FDA). The Division of Cardiology and Nephrology regulates and reviews Investigational New Drug and marketing applications for drug and biologic products for the treatment of cardiovascular and kidney diseases. Dr. Thompson joined the FDA as a medical officer in 2007. Prior to her current position, Dr. Thompson served as the Deputy Director of the Division. Dr. Thompson received her medical degree from Johns Hopkins Medical School and completed her Internal Medicine and Nephrology training at Columbia University/New York-Presbyterian Hospital. She holds a Master of Science in Biostatistics/Patient Oriented Research Track from Columbia University Mailman School of Public Health.

2023 RAREVOICE AWARDEES

Philip "PJ" Brooks

National Center for Advancing Translational Sciences

Kelly Buckland

U.S. Department of Transportation, Federal Advocacy – Federal Agency Staff

Nell Choi

Artist-to-Advocate

Carter Hemion

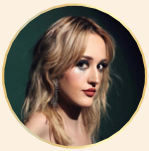
Diversity Empowerment

Mindy Henderson

Muscular Dystrophy Association, Federal Advocacy by a Patient Advocate or Organization

Lucas Lam

Office of Representative Eric Swalwell (CA-14), Federal Advocacy – Congressional Staff



Madison Lawson

Muscular Dystrophy Association, Federal Advocacy by a Patient Advocate or Organization

Amanda Lincoln

Senate Committee on Health, Education, Labor and Pensions

Annette Logan Parker

Cures 4 The Kids Foundation, State Advocacy by a Patient Advocate or Organization

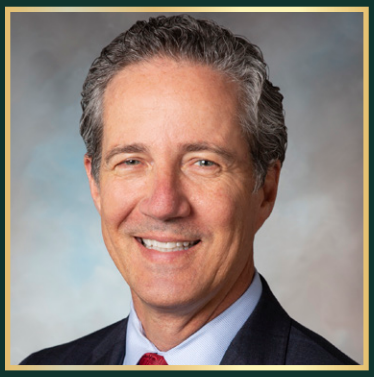
Representative Liz Reyer

Minnesota, State Advocacy by a State Legislator

Samantha Rose

Advocacy by a Youth or Teenager

STATE ADVOCACY: STATE LEGISLATOR



SENATOR KELLY HANCOCK (TEXAS)

Texas Senate bill 1249, establish a living organ donor education program

Kelly Hancock represents Senate District 9, which covers much of Tarrant County. He previously served three terms in the Texas House of Representatives and 13 years on his local school board, and remains an advocate for core conservative values of limited government and lower taxes.

Senator Hancock currently serves as Chairman of the Senate Veteran Affairs Committee, which has oversight on vital policy that honors and serves our nation's veterans. Throughout his legislative career, Kelly has worked to keep government out of the way of Texas' job creators and pass pro-border security, pro-life, pro-fiscal responsibility, and state sovereignty legislation.

A graduate of Baylor University, Kelly is an entrepreneur with an extensive business background and runs his family-founded company in North Texas. He and his wife, Robin, have been married for 37 years and have three adult children who are all married to wonderful spouses. The Hancocks are now proud grandparents to six tiny Texans.

REPRESENTATIVE EMERSON LEVY (OREGON)

Oregon HB 4113, ban co-pay accumulators in health insurance plans

Representative Emerson Levy is a mom to an elementary schooler, an attorney, an educator, and a proud Oregonian. She is passionate about the health and safety of Oregon's children, families, and seniors, and believes that Oregonians should thrive, not just get by.

In her first term, Representative Levy shepherded the legislature to fund a new grant program to improve emergency response times in schools. She also helped pass a law to fix the way that deductibles are calculated, lowering the out-of-pocket cost of life-saving prescription medications and helping some of Oregon's most vulnerable people. Representative Levy is passionate about increasing economic opportunities for Oregonians, including through registered apprenticeship programs, early childhood education, innovative manufacturing, and clean energy jobs.

She serves on the Housing and Homeless Committee and Joint Ways and Means Subcommittee for Natural Resources, and is the Vice Chair of the Climate, Energy, and Environment Committee.

DELEGATE CANDI MUNDON KING (VIRGINIA)

Virginia HB 255 (Adult Wellness Screening; Sickle Cell Disease or Sickle Cell Trait); HB 257 (Sickle Cell Anemia; Prescription of Opioids for Pain Management); HB 820 (Sickle Cell Disease; Annual Review of Medication and Treatment, Report)

Candi Mundon King has served as a member of the Virginia House of Delegates since 2021, representing the 2nd District. A passionate advocate for healthcare equity, Delegate King has championed numerous legislative efforts to improve the lives of those affected by rare diseases, specifically sickle cell disease, which her daughter battles every day. She sponsored critical legislation, including HB 255, HB 257, and HB 820, to expand access to screenings, enhance pain management, and ensure up-to-date treatment reviews for sickle cell patients. A graduate of Norfolk State University and a former nonprofit leader, Delegate King's work is deeply informed by her personal connection to the rare disease community. She continues to advocate for policies that provide comprehensive healthcare solutions for underserved populations.

REPRESENTATIVE MY-LINH THAI (WASHINGTON)

Washington HB 1079 (Concerning rapid whole genome sequencing); HB 1745 (Improving diversity in clinical trials); HB 1287 (Concerning dental hygienists)

At the age of 15, My-Linh Thai immigrated to Washington state as a Vietnamese refugee with her family. She graduated with honors from Federal Way High School and from the University of Washington School of Pharmacy. She is proud to be the first refugee elected to serve in the Washington State House of Representatives.

Rep. Thai is a passionate education advocate who is committed to ensuring equity and access for all. This commitment springs from the early support she herself received as a student. Prior to serving in her current role, she has served as a PTSA parent volunteer and received the Washington State PTA Outstanding Advocate Award in 2013. She was elected as the School Board Director for the Bellevue School District, and later elected by her fellow Board Directors to serve as Vice President of the Washington State School Board Directors Association (WSSDA) in 2017.

Rep. Thai is the Deputy Majority Leader of the House Democratic Caucus. She also serves on the Civil Rights & Judiciary Committee, Finance Committee, and Healthcare & Wellness Committee.

STATE ADVOCACY: PATIENT ADVOCATE OR ORGANIZATION

CHRISTINA CAMPOS

New Mexico General Appropriations Bill, HB2, state funding for CCM, specifically to the "UNM Health Sciences Center Cerebral Cavernous Angioma Initiative."

Christina Campos is the former CEO of Guadalupe County Hospital; a position she held from July 2004 until her retirement in June 2024. Under her leadership, Guadalupe County Hospital, was recognized as a Top 20 Community Hospital by the National Rural Health Association in 2019, 2020, and 2021, and a top 100 in 2023, and she was recognized numerous times as a Top Rural Hospital CEO by Beckers. In September 2023 Guadalupe County Hospital became the first and only Rural Emergency Hospital in New Mexico.

Christina is a former American Hospital Association Board Trustee and a former member of the HHS National Advisory Committee for Rural Health and Human Services. She has also served on numerous national rural health care committees and on the boards of the New Mexico Hospital Association, the NM Hospital Services Corporation, the NM Hospital Equipment Loan Council, and the NM Rural Hospital Network. She currently serves on the AHA Nominations Committee, and on the boards of the NM Mutual Workers Compensation Casualty Company, the Anchorum Health Foundation, ImagineNM, the NM Osteoporosis Foundation, and the Alliance to Cure Cerebral Cavernous Malformation. She is the President of her community's local development corporation/Main Street organization, and she serves on the Santa Rosa Route 66 Centennial Celebration committee.

Christina earned her BA in Economics and Latin American Studies at the University of New Mexico, and received her MBA, with an emphasis on Healthcare Management, at Regis University in Denver. She is married to Jose Campos, and together they have three adult children and two grandchildren. She and her husband own and operate a family restaurant, Joseph's Bar & Grill, in Santa Rosa.

MELANIE FLOOD

California AB2613, create a Rare Disease Advisory Council

Melanie Flood FRSA is the Founder & CEO of the Mellie J Foundation. In 2020, an attack of optic neuritis caused her to lose her vision in her left eye, and a subsequent relapse in 2021 caused permanent damage in her right eye. She endured a 22-year diagnostic journey from her first hospitalization to reach a diagnosis of seronegative NMOSD with Sjögren's syndrome. Her diagnostic journey and the adversity she has faced since her rare autoimmune diagnosis ignited a commitment in her to utilize her professional experience to empower other patients with rare diseases to advocate for themselves.

She was Director of Communications for Social Enterprise UK (a strategic partner of the Cabinet Office) and the UK Fashion & Textile Association (a trade challenge partner of the Department for International Trade). Melanie is currently Director of Development Major Gifts for Make-A-Wish, raising money to grant wishes for children with critical illnesses. She is a Fellow of the Royal Society of Arts in London and Co-Chair for Region A of the National Organization for Rare Disorders Policy and Advocacy Taskforce. She is a graduate of the American Academy of Dramatic Arts in Los Angeles and the American Conservatory Theater's Master of Fine Arts Program in San Francisco.

HALIE MAY

New York A.49/S.3552, provide for the licensing of genetic counselors; creates the state board for genetic counseling

Halie May is a certified genetic counselor and a graduate of the Joan H. Marks Graduate Program in Human Genetics at Sarah Lawrence College. She began her career working as a genetic counselor in precision medicine research at Columbia University Irving Medical Center and then worked in a start-up environment before her current role as a clinical genomic specialist at Natera. She is passionate about increasing access to genetic testing and education and is leading the New York state initiatives to advocate for genetic counseling licensure, as well as federal Medicare recognition for profession. She has served as chair of the New York State Genetics Task Force Public Policy Committee since January 2021.

AMGEN

Fighting the World's Toughest Diseases

PROUD TO SUPPORT THE 2024 RAREVOICE AWARDS

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FEDERAL ADVOCACY: YOUTH AND TEEN

SATI COOPER-MCCANN

Accelerating Kids' Access to Care Act

Raw gem faceting involves shaping and polishing to refract the light inside to maximize a stone's brilliance and fire. Sati's dispersion of light and luster began at 7 in self-awareness, self-efficacy, and community building. This rare gem is a force for equitable and legislative good in rare healthcare 'on the Hill' and beyond. Her pillars of support include her mother, key family and friends, health specialists, medical mentors, virtual public-school teachers, pediatric ballet instructors, and community organizations. Advocacy has no age barrier as the youngest of many equitable actions: a Marfan Foundation Kids' Club peer, a public #LightUpForRare Event co-host, a Global Nation Rare Gem Campaign Addressee, an RDLA's 1st Ever Youth and Teen Hill Day and Rare Across America participant, and a Global Genes Rare Advocacy Summit panelist. She's sharing her excavation from local to global civil rights for rare disease patients as part of our rare collection, so we may radiate together.

CARL JOHNSON

Safe Step Act, HELP Copays Act, and the Accelerating Kids' Access to Care Act

Carl Johnson is a 12-year-old from Connecticut who has Hereditary Angioedema (HAE), which causes swelling all over the body, including the throat. Without access to treatment, 1 in 3 people can die from an HAE attack. While medications are available to treat HAE, Carl has struggled not being able to access the medication he needs. Carl has also seen the financial burden these medications place on his family, particularly his mother who also has HAE.

Carl's rare disease advocacy has inspired him to write a short story to raise funds for NORD, serve as a FDA rare disease spokesperson, share his story on television, speak at the Capitol in Hartford for Rare Disease Day, and join the HAEA Youth Advocates in DC to lobby for legislation ensuring kids with a rare disease can access the treatment they need. He believes that no one should suffer when suffering is preventable.

TAYLOR SPENCE

VHL inclusion in Defense Appropriations Act, 2024

Taylor Spence, originally from Bergen County, NJ, is currently a student at Texas A&M University pursuing a career in pediatric nursing. Inspired by witnessing her father's battle with VHL, Taylor is dedicated to giving back and making a difference in the lives of others. Her goal is to work with children, a passion shaped by her own experiences as a child undergoing scans and seeing specialists for VHL.

Taylor vividly remembers the care and compassion shown by nurses during her medical journey and is committed to providing that same level of support to children facing tough medical situations. Her volunteer work with VHL has deepened her connection to others affected by the disease and reinforced the importance of finding a cure

STATE ADVOCACY: YOUTH AND TEEN

EMMALYN HUDSON

Mississippi Rare Disease Advisory Council

Emmalyn Hudson, 14, was diagnosed through newborn screening with a rare genetic metabolic disease called Glutaric Aciduria/Acidemia Type 1. She enjoys participating in theater, archery, 4-H, American Heritage Girls, and her Church Bible study and youth group. A creative individual, Emmalyn also loves art and was honored as a Rare Artist Awardee in 2021 by the EveryLife Foundation.

Four years ago, her mother founded the Mississippi Metabolics Foundation, where Emmalyn plays an active role in helping run the organization and learning to advocate for rare diseases and newborn screening. Being homeschooled has given her the flexibility to engage in numerous advocacy opportunities, including Rare Disease Week on Capitol Hill.

In recent years, Emmalyn has advocated for both federal and state legislation, including efforts to establish a Rare Disease Advisory Council in Mississippi, align newborn screening with the Recommended Uniform Screening Panel (RUSP), and support bills such as the Medical Nutrition Equity Act and the Accelerating Kids' Access to Care Act.

AYANA LEE JOHNSON

Virginia HB 255 (Adult Wellness Screening; Sickle Cell Disease or Sickle Cell Trait); HB 257 (Sickle Cell Anemia; Prescription of Opioids for Pain Management); HB 820 (Sickle Cell Disease; Annual Review of Medication and Treatment, Report)

Ayana Lee Johnson, named Miss Virginia Teen 2022, SCDAA National Teen Ambassador 2021-2023, and the American Red Cross Sickle Cell Youth Ambassador of Virginia, created Ayana's R.E.A.D Initiative - Readiness to Empower, Advocate, and Diminish Hopelessness in Chronic Illness. The R.E.A.D Initiative was designed to encourage community members to support families living with chronic illnesses. It allows Ayana to raise awareness while identifying social determinants of health that impact access, treatment, and outcomes of those living with Sickle Cell Disease (SCD).

Ayana fosters education campaigns and secures support for SCDAA. She is an honors student, accomplished dancer, and classically trained violinist who defies SCD limitations. Ayana is a freshman at the University of Alabama where she is majoring in Microbiology with aspirations to obtain a Doctor of Medicine (MD) and become a Pediatric Hematologist.

Despite Ayana's busy lifestyle, she has many interests, including reading, listening to music, traveling, spending time with family, film and photography, and journaling.

KAYDEN MYERS

Illinois SB 67, amends Newborn Metabolic Screening Act

Kayden Myers, a 16-year-old from Rockford, Illinois, is passionate about aviation and has been working toward his pilot license since the age of 13. Inspired by his first flight, Kayden is on track to obtain his private pilot license on his 17th birthday. In addition to aviation, he enjoys snowboarding, mountain biking, football, ATV riding, and going on adventures with family and friends.

Kayden's advocacy journey began when his sister became seriously ill, exposing him to the challenges faced by medically fragile and rare disease patients. This experience sparked his commitment to advocating for his sister and others who lack a voice. Earlier this year, due to difficulties securing nursing care for his sister in Illinois, Kayden's family temporarily relocated to Guatemala, where she received exceptional care. After more than a year of effort, the family successfully secured in-home nursing care in Illinois. Kayden remains dedicated to advocating for improvements in the process to help other families navigate similar challenges.

SOPHIE THRESHER

Massachusetts Bill H. 2236, an Act Related to Lysosomal Storage Disorders in Infants

Sophie Thresher, a Junior from Massachusetts, was diagnosed with Gaucher Type 1 in 2021. Her journey was a painful process that heavily impacted her and her family. Sophie has since advocated for awareness of her disease, lobbying for a routine test on all newborns to avoid the painful, heart wrenching process she went through. Sophie has lobbied at the Massachusetts State House to pass Bill H2236, spoken at legislative briefings, and educated people through sharing her diagnosis journey. She recently attended the Gaucher Community Alliance meeting to continue bringing awareness to the need for change in how to diagnose Gaucher. When Sophie is not advocating, she can be found on the soccer field, sharing her love for the game and coaching, and spending time with close family and friends. She is also an avid artist and hopes to become a Pixar Animator someday.

ARTIST-TO-ADVOCATE

SPONSORED BY AMGEN

These finalists have utilized their artwork to advocate for federal or state legislation. Award exclusively sponsored by Amgen.

Window Into the Soul

SARAH BAILEY

Sarah Bailey was born in October 1985 in Festus, Missouri. Her profound hearing loss at birth went undetected until she was three years old, and vision issues were identified when she was seven. Shortly afterward, she was officially diagnosed with Usher syndrome Type 2a.

Sarah graduated in 2008 with a teaching degree from Missouri State University and has spent 15 years teaching elementary-aged children. Aware of her prognosis of becoming Deaf/Blind, she and her husband made it a priority to travel and create lasting memories. Her teaching career has taken her to unique locations, including a one-room schoolhouse surrounded by dinosaur fossils in Montana. Today, Sarah's journey has brought her to the remote villages of Alaska, where she now resides with her husband and three sons.

Amazing Creation

BRENNA BRUCK

Brenna Bruck is a vibrant 13-year-old from Boone, Iowa, who is deeply involved in her middle school and community. She participates in sports, theatre, and youth group clubs, and enjoys spending time with her two sisters, friends, and family. Brenna is known for her warm, compassionate nature, which allows her to connect easily with those around her. At the age of 8, Brenna was diagnosed with Fragile X Syndrome, which sparked her interest in learning more about the condition. At 9, she joined the Girl Band study at Stanford University, contributing to brain and behavioral development research in girls with Fragile X. Her involvement in this study reflects her curiosity and desire to contribute to scientific understanding. Beyond her academic and extracurricular activities, Brenna's love for creativity shines through her artwork, which has been featured in various settings, helping her express herself in unique and meaningful ways.

KATRINA BYRD

Katrina Byrd, writer and playwright living in Jackson, MS, received her MFA in Creative Writing from Mississippi University for Women. An emerging writer published in several magazines, Byrd is a six time Arts Commission grant recipient. Diagnosed with amyotrophic lateral sclerosis (ALS) November 18, 2019, Dora - Katrina Byrd's partner of 23 years - died on February 2, 2020, 76 days after diagnosis.

For the past four years, Byrd has become fluent in telling uncomfortable stories. For the past four years, Byrd served with several ALS organizations, testified before the FDA, became a NEALS Ambassador and Byrd represented a poster titled Kickin' ALS at the 2024 ALS/MND International Symposium in Montreal. Katrina serves on the Healey Recruitment and Retention team, on the LEITH Lab Community Advisory Board at Yale University and she served with the Department of Defense as a part of the 2024 CDMRP-ALSRP process.

DIVERSITY EMPOWERMENT: PATIENT ADVOCATE OR ORGANIZATION

MARIO ESTEVEZ

Mario Estevez, a dedicated father of three, is deeply connected to the rare disease community, particularly because one of his children has Hunter syndrome. His advocacy work includes serving on the board of Project Alive from 2016 to 2023, where he helped secure funding and IND approval for a gene therapy trial for the condition.

Professionally, Mario is an organizer with the Racial Equity Institute (REI), facilitating workshops on racial equity. Currently, he collaborates with the Chan Zuckerberg Initiative to promote equitable systems within the disease community. He has also worked with researchers and nonprofits to enhance support for rare diseases, successfully advocating for their inclusion in Florida's newborn screening panel and negotiating medicine donations for children in developing countries. Through these efforts, Mario aims to improve the lives of families affected by rare diseases.

THE E.WE FOUNDATION

The E.WE Foundation is a global healthcare advocacy organization dedicated to improving the lives of individuals and families impacted by rare diseases, with a hyperfocus on Trisomy 18 (Edwards Syndrome). The E.WE Foundation was founded by Kareem & Sarita Edwards as a result of their personal journey as parents of a child with Full Trisomy 18. This inspired Sarita to create a platform that supports, educates, and empowers others facing similar challenges. The foundation's mission is to provide resources and support to families impacted by rare diseases like Trisomy 18, while changing the medical perspective through advocacy, education, and public policy. Through its core programs, LEAP, ZEBRA, and STRIPE, the E.WE Foundation is committed to health literacy and community education, comfort care and mental health, and to providing economic assistance to Trisomy 18 families experiencing financial hardship.

The E.WE Foundation works collaboratively with nonprofit organizations, school systems, and state agencies across the country to advance rare disease awareness, education, and legislation. The organization is also committed to empowering diverse voices within the rare disease community to advance health system advocacy, research, and public policy initiatives. Through its work, the E.WE Foundation continues to make a profound impact, ensuring that every individual affected by a rare disease receives the care, support, and recognition they deserve.

DR. HARSHA RAJASIMHA

Harsha K. Rajasimha, Ph.D., is a serial entrepreneur, scientist, patient advocate, and philanthropist. He is the Founder and CEO of Jeeva Clinical Trials Inc., an AI-driven platform that streamlines clinical trials for universal accessibility. Harsha also serves as the founder chairperson of the Indo US Organization for Rare Diseases, fostering cross-border collaboration through initiatives like the annual Bridging RARE Summit and the Abbey Meyers Khushi Bridging RARE Awards Gala.

With 20 years of experience spanning academia, federally funded research, healthcare consulting, and startups, Harsha shifted his career focus after losing a newborn child to a rare disease in 2012 and his brother to juvenile diabetes in 2017. At Jeeva, he leads the development of a patient-centered platform that reduces burdens on patients and study teams, accelerating clinical trials by over 3x.

His work has earned recognition from organizations like the Lead India Foundation, Sanofi Genzyme, and the United Nations. He holds an M.S. in Computer Science and a Ph.D. in Genetics, Bioinformatics, and Computational Biology from Virginia Tech.

WILLIAM ROMERO

William Romero (he/him) is a queer rare disease advocate from Lafayette, Louisiana. Diagnosed with fibrous dysplasia at the age of four, William never let his rare disease define him, and in high school, he started his advocacy journey by calling for federal funding for fibrous dysplasia research. Since then, his advocacy work has been ongoing, including interning at the Louisiana Governor's Office of Disability Affairs and being a founding member of the FD/MAS Patient Advisory Council. More recently, William transformed his advocacy by creating his drag persona, Daisy Bility. With Daisy, William has been able to bridge his queer identity with his rare disease advocacy, pointing out this overlooked intersection of identities. William has used Daisy as a vehicle to present the wide range of experiences of queer rare disease patients, and this spring, William shared this experience during Rare Disease Week on Capitol Hill.

CONGRATULATIONS

TO ALL RARE DISEASE PATIENT ADVOCATES AND ORGANIZATIONS NOMINATED THIS YEAR

Avery Allmond
Tricha Shivas
Lesa Brackbill
Ann Brazeau
Leah Campbell
Paul Seifert
Felicia Morton
Erica Barnes
Kathleen Arntsen
Jessica Miles
Samantha Stallings
The HIV+ Hepatitis Policy Institute
Diabetes Leadership Council
Diabetes Patient Advocacy Coalition
Mary McGowan
Pamela Price
Carl Barnes
Heidi Edwards
Mackenzie Abramson
Jessica Graham
Brigid Brennan
Dorothea Lantz

THANK YOU

TO THE RAREVOICE NOMINATIONS COMMITTEE

Wendy Erler, Alexion Pharmaceuticals

Stephen Groft, Pharm.D., EveryLife Foundation for Rare Diseases Board of Directors

Neena Nizar, Ph.D., The Jansen's Foundation

Caitlin Van Sant, Mehlman Consulting

Shayne Woods, Alpine Group

RARE DISEASE WEEK ON CAPITOL HILL

REGISTRATION OPENS **JANUARY 6, 2025**

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FEBRUARY 24 – 26, 2025

#RareDC2025

rareadvocates.org/rdw

Yathish & Pawan, friends living with *Gaucher disease*, India

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