



THE 11th ANNUAL

RARE VOICE

Awards

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

December 14, 2022

Arena Stage, Washington, D.C.

PRESENTED BY

sanofi



Thank you to our 2022 RareVoice Award Sponsors

PRESENTED BY

The Sanofi logo, featuring the word "sanofi" in a bold, black, sans-serif font. A small purple dot is positioned above the 'i'.

LEADER



NOMINEE

The Biomarin logo, featuring the word "Biomarin" in a blue, sans-serif font. A small red and blue icon is positioned to the left of the word.

The Horizon logo, featuring a stylized, multi-colored icon (yellow, orange, red, purple) to the left of the word "HORIZON" in a bold, black, sans-serif font.

The Jazz Pharmaceuticals logo, featuring a stylized, multi-colored icon (yellow, orange, red, purple) to the left of the words "Jazz Pharmaceuticals" in a black, sans-serif font.

The Janssen logo, featuring the word "janssen" in a blue, sans-serif font. Below it, in a smaller font, is "PHARMACEUTICAL DIVISION OF Johnson & Johnson". To the right is a blue, stylized icon.

The Pfizer logo, featuring a stylized, blue, "P" icon to the left of the word "Pfizer" in a blue, sans-serif font.

ADVOCATE

The Alexion logo, featuring a stylized, blue, "A" icon to the left of the word "ALEXION" in a blue, sans-serif font.

The Mallinckrodt logo, featuring a stylized, multi-colored icon (green, blue, yellow) to the left of the word "Mallinckrodt" in a black, sans-serif font.

The Neurocrine Biosciences logo, featuring a stylized, multi-colored icon (green, blue, yellow) to the left of the words "NEUROCRINE BIOSCIENCES" in a black, sans-serif font.

The Traverse Therapeutics logo, featuring a stylized, multi-colored icon (green, blue, yellow) to the left of the words "TRAVERSE THERAPEUTICS" in a black, sans-serif font.

GRASSROOTS

The Amicus Therapeutics logo, featuring a stylized, blue, "A" icon to the left of the words "Amicus Therapeutics" in a blue, sans-serif font.

The BridgeBio logo, featuring a stylized, blue, "B" icon to the left of the word "bridgebio" in a blue, sans-serif font.

The Chiesi logo, featuring a stylized, multi-colored icon (green, blue, yellow) to the left of the word "Chiesi" in a black, sans-serif font.

The Genentech logo, featuring the word "Genentech" in a bold, black, sans-serif font.

The Harmony Biosciences logo, featuring a stylized, multi-colored icon (green, blue, yellow) to the left of the words "HARMONY BIOSCIENCES" in a black, sans-serif font.

The Mitsubishi Tanabe Pharma America logo, featuring a stylized, blue, "M" icon to the left of the words "Mitsubishi Tanabe Pharma America" in a black, sans-serif font.

The Pharming logo, featuring a stylized, blue, "P" icon to the left of the word "Pharming" in a black, sans-serif font.

The RegenXBio logo, featuring a stylized, multi-colored icon (green, blue, yellow) to the left of the words "REGENXBIO" in a black, sans-serif font.



EVENT PROGRAM

★ Masters of Ceremonies

Mark Dant, Executive Director, Ryan Foundation

Ryan Dant, Director of Patient Advocacy, Grace Science LLC

★ Announcement of the RareVoice Award Recipients

Congressional Leadership Award

Federal Advocacy: Congressional Staff

Federal Advocacy: Patient Advocate Or Organization

Federal Advocacy: Federal Agency Staff

State Advocacy: State Legislator

State Advocacy: Patient Advocacy or Organization

Federal or State Advocacy by a Teen

Diversity Empowerment: Patient Advocate or Organization

Artist-To-Advocate



@RareAdvocates

#RareVoice2022 // RareAdvocates.org



★ ABOUT THE ABBEY ★

The RareVoice "Abbey" Award was named after Abbey Meyers, the founder of the National Organization for Rare Disorders (NORD). Mrs. Meyers received the Lifetime Achievement Award at the inaugural RareVoice Awards in 2012 for her vital role in the passage of the Orphan Drug Act.

The statue was commissioned for the RareVoice Awards from the renowned sculptor Nobe, who specializes in bronze. The Abbey represents the "rare voice" speaking on behalf of patients, especially children, who might not otherwise be heard. This award is presented to patient advocates and organizations.



*Abbey Meyers with EveryLife
Founder, Dr. Emil Kakkis.*



MASTERS OF CEREMONIES



MARK DANT

Executive Director, Ryan Foundation

Mark Dant is a parent advocate and retired Carrollton, Texas Police Department Assistant Chief of Police. Mark and his wife Jeanne are the parents of Ryan, who is 34 years old and the longest treated person in the world with MPS I. Mark and Jeanne spearheaded the funding for the first MPS Enzyme Replacement Therapy, Aldurazyme, through their Foundation, the Ryan Foundation. In partnership with Dr. Emil Kakkis, Mr. Dant and his family were also key advocates speaking to the FDA about the importance and significant impact of ERT for the treatment of mucopolysaccharidoses. In 2009, Mr. Dant and his family successfully championed the US House of Representatives to pass the Ryan Dant Health Care Opportunity Act, a bill designed to help those living on Medicaid assistance become

gainfully employed. The Dant's journey has been documented on The Today Show, CBS 60 Minutes, CNN, Biography Magazine, Readers Digest in 13 languages around the world, Golf Digest, the LA Times and numerous newspapers and news outlets across the US. Mark spends his time now volunteering for the EveryLife Foundation, the Ryan Foundation, and numerous other rare disease organizations to help empower the patient advocate through the understanding that all of us have the power to turn action to hope and hope to reality.



RYAN DANT

Director of Patient Advocacy, Grace Science LLC

Ryan Dant is a long-time patient advocate diagnosed at the age of three with mucopolysaccharidosis type 1 (MPS I), a rare lysosomal storage disorder. When doctor's predicted Ryan's life to be cut short due to the disease, Ryan's parents, Mark and Jeanne, spearheaded the funding for the first MPS Enzyme Replacement Therapy, Aldurazyme. Now 34 years old, Ryan is the longest treated person in the world with MPS I. His story has been featured in numerous media outlets and inspired people around the globe. Determined to apply his patient experience to benefit others, he participated in several medical trials and served as keynote speaker at physician, biotechnology, and nonprofit events around the U.S. and internationally. Ryan received a Bachelor of Applied Science, Sports

Administration from the University of Louisville. A sports enthusiast, Ryan served in equipment management support roles for the Texas Rangers Baseball club, Southern Methodist University and University of Louisville football teams, and the San Francisco 49ers. Recently, Ryan worked in patient assistance roles for healthcare providers, including Norton Healthcare and UT Southwestern Medical Center. Ryan is currently serving as director of patient advocacy for Grace Science, LLC, working with NGLY1 patients. Last summer, Ryan married Silvia, the love of his life who works as a fourth-grade bilingual teacher.

CHANGING POLICY, SAVING LIVES



The EveryLife Foundation for Rare Diseases is a nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.



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Rare Disease Legislative Advocates is a program of the EveryLife Foundation for Rare Diseases designed to support the advocacy of all rare disease patients and organizations.



@RareAdvocates

rareadvocates.org



RAREHUB

WASHINGTON D.C.

The Rare Hub is a community workspace created by the EveryLife Foundation for Rare Diseases to improve cross-disease collaboration and legislative advocacy in the heart of Washington, D.C.

SCAN FOR MORE INFO



Thank you to our RareHub Partners



Undiagnosed Diseases
Network Foundation



RARE DISEASE CONGRESSIONAL CAUCUS

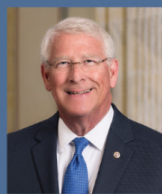
Thank you to our Rare Disease
Congressional Caucus Co-Chairs and Members



Representative
G.K. Butterfield (NC)



Representative
Gus Bilirakis (FL)

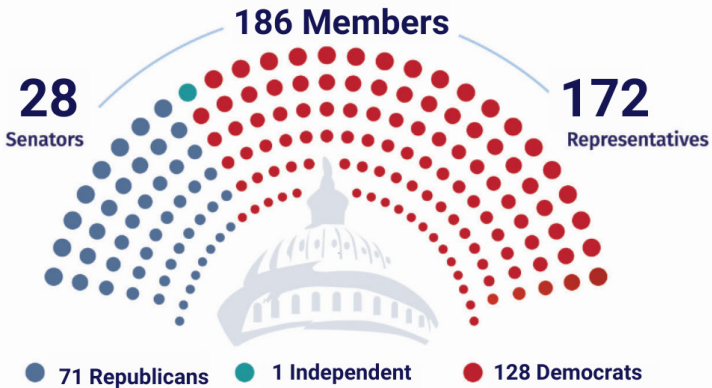


Senator
Roger Wicker (MS)



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



CONGRESSWOMAN ANNA G. ESHOO

Congresswoman Anna G. Eshoo was born in New Britain, Connecticut, of Assyrian and Armenian heritage. For over two decades in Congress, Rep. Eshoo has defended consumers, promoted American competitiveness and innovation, fought for access to health care for families and children, protected the environment, and encouraged development of clean energy technology.

Rep. Eshoo's work consistently earns the highest approval from a wide range of organizations, including the League of Conservation Voters, the Humane Society, the American Association of University Women, Iraq and Afghanistan Veterans, the Biotechnology Industry Organization, the American College of Emergency Physicians, and the Science Coalition. On January 15, 2019, the Democratic Members of the House Energy and Commerce Committee voted to elect

Congresswoman Eshoo as the first woman ever to serve as Chair of the Health Subcommittee. Rep. Eshoo helped draft portions of the Affordable Care Act to provide comprehensive, affordable health insurance for all Americans, including eliminating lifetime limits in health insurance plans, and she continues to work on strengthening the law. She has written landmark bipartisan legislation to promote the development of new lifesaving cures in biologic drugs; protect patient's genetic information from being used to discriminate against them; modernize how the Food and Drug Administration regulates and approves medical devices; and promote the development of drugs specifically for children.



SENATOR LISA MURKOWSKI

Senator Lisa Murkowski, Alaska's senior U.S. Senator, is a third generation Alaskan proudly serving as the first Alaskan born senator. Since joining the Senate in 2002, Senator Murkowski has worked tirelessly for Alaskans and earned a reputation in the Senate for her ability to work collaboratively and across the aisle to reach common sense solutions.

Senator Murkowski recognizes that sound national policy will promote not only job creation and economic growth, but also higher standards of living and greater global stability. She is a member of the Senate Appropriations Committee (Subcommittees –Commerce, Justice, Science, and Related Agencies; Defense; Energy and Water Development; Homeland Security; Military Construction, Veterans Affairs, and Related Agencies), Ranking Member of the Interior-

Environment Subcommittee, member of Senate Energy and Natural Resources Committee, member of the Senate Health, Education, Labor & Pensions Committee, and Vice Chairman of the Senate Indian Affairs Committee.



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MAKE THE
BREAKTHROUGH.**

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FEDERAL ADVOCACY FINALISTS CONGRESSIONAL STAFF



SARAH SHAPIRO

*Office of Representative Eric Swalwell
Precision Medicine Answers for Kids Today Act*

Sarah Shapiro is the Legislative Director for Congressman Eric Swalwell (D-CA) and staffs the Congressional Personalized Medicine Caucus. Sarah was previously Congressman Swalwell's policy advisor for health care after working as a legislative aide for Senator Chris Murphy who serves on the Senate HELP Committee. She previously worked for the Center of American Progress and attended the University of Southern California where she studied public policy.



DANIEL ZAWITOSKI

*Office of Representative Scott Peters
Precision Medicine Answers for Kids Today Act*

Daniel Zawitoski is the D.C. Chief of Staff for Rep. Scott Peters who represents California's 52nd District which includes San Diego, Coronado, and Poway. He received his undergraduate education at Colby College in Waterville, Maine and obtained a Master's Degree in Political Science from San Diego State University. He is an original member of Rep. Peters' office, previously serving as Legislative Correspondent, Legislative Assistant, and Legislative Director. In his roles he has been instrumental in Rep. Peters' legislative successes in a variety of issue areas, however, he has been most impactful in advancing Rep. Peters' healthcare and innovation portfolio, most recently securing the inclusion of the Precision Medicine Answers for Kids Today Act in CURES 2.0. He is married with two children (3 and 1), and resides in Silver Spring, MD.



FEDERAL ADVOCACY FINALISTS CONGRESSIONAL STAFF



CHRIS JONES

*Office of Representative Gus Bilirakis
Speeding Therapy Access Today Act and Rare
Disease Congressional Caucus*

Chris Jones is a Senior Policy Advisor for Rep. Gus Bilirakis (R-FL). Originally from Los Angeles, CA, Chris has lived in the DC area since 2001. He received his Bachelor's in Economics from George Mason University, and his Master's in Public Policy from the Schar School of Policy and Government. Chris has worked for Congressman Bilirakis since 2015, where he has worked his way up from an internship to senior legislative staff. In addition to health care policy, Chris also works to advance legislative priorities in Commerce and Consumer Protection portfolio. He currently oversees the Congressman's co-chairmanship of the Congressional Rare Disease Caucus, Parkinson's

Caucus, and Congenital Heart Caucus. Over this past Congress, he has helped the Congressman secure passage of the Oral Health Literacy and Awareness Act and enactment of the Restoring Brand USA Act and STANDUP Act into law. Chris and his wife live in Arlington, Virginia.



AISLING MCDONOUGH

*Office of Representative Anna Eshoo
Ensuring Lasting Smiles Act*

Aisling McDonough is the Chief of Staff for Congresswoman Anna G. Eshoo (CA-18). Prior to becoming Chief of Staff, McDonough served as Senior Health Policy Advisor to Eshoo since she became Health Subcommittee Chairwoman in 2019. From 2017 to 2019, McDonough was Health Legislative Assistant to Senator Brian Schatz (D-HI), and worked from 2010 to 2017 at the Centers for Medicare and Medicaid Services at the U.S. Department of Health and Human Services. She is a graduate of the University of Maryland where she earned a Bachelor of Arts and a Master of Public Policy.



FEDERAL ADVOCACY FINALISTS CONGRESSIONAL STAFF



GIDGET BENITEZ

*Office of Representative Jan Schakowsky
Lymphedema Treatment Act*

Gidget Benitez serves as Health Policy Counsel to U.S. Representative Jan Schakowsky, where she handles all legislative and committee work related to health policy. This includes Medicare, Medicaid, rare diseases, FDA/pharmaceutical and medical device regulations, disability and reproductive rights. She also serves as the Congresswoman's staff liaison to the House Democratic Task Force on Aging and Families and the Congressional Pro-Choice Caucus.



JAY EBERLE

*Office of Senator John Barrasso
Access to Genetic Counselor Services Act*

Jay currently serves as Senator Barrasso's health legislative assistant. Senator Barrasso is Chairman of the Senate Republican Conference and a member of the Senate Finance Committee. Jay joined Senator Barrasso's staff in 2011.

He began his career on Capitol Hill working for Senator Jim Bunning (KY). After Senator Bunning's retirement, he worked for Representative Chip Cravaack (MN-8), handling the Congressman's work on the House Science, Space and Technology Committee. He holds an undergraduate degree in government from Centre College and a master's degree in legislative affairs from George Washington University.

Jay is married to his wife Alison, a nurse practitioner at Johns Hopkins Hospital. They are the parents of three children.

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WE SEE HOPE FOR
PATIENTS AND FAMILIES



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At Neurocrine Biosciences, our purpose is simple: to relieve suffering for people with great needs, but few options.

We are proud to support the **EveryLife Foundation for Rare Diseases** and this year's **RareVoice Awards** recipients.

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FEDERAL ADVOCACY FINALISTS

PATIENT ADVOCATE OR ORGANIZATION



BECKY ABBOTT

Ensuring Lasting Smiles Act

Becky M. Abbott, MPH, is the Director of Research and Treatment Advocacy for the National Foundation of Ectodermal Dysplasias. She is also Co-Chair of the NFED Family Advocacy Committee where she leads efforts to move the Ensuring Lasting Smiles Act (ELSA) through Congress. She received her undergraduate degree in biology from Mount Mary College and her Master's in Public Health at Benedictine University. She resides in Wisconsin with her husband, Tom, and sons Aidan, diagnosed with x-linked hypohidrotic ectodermal dysplasia, Ryder, and Lachlen. The Abbott family advocates regularly to educate members of Congress on ectodermal dysplasias, skin disease, rare disease, and congenital anomalies. Becky is the NFED's representative on the EveryLife Foundation's Community Congress and a member of the Access and Public Policy workgroups. She encourages everyone to raise awareness for their disease and advocate on a daily basis. Patient's stories and voices are the most powerful tool to promote change.



SARAH CHAMBERLIN

Medical Nutrition Equity Act and National Formula Shortage

Sarah Chamberlin is the Executive Director of National PKU News and the parent of two daughters, one with PKU born in 2013. She has a degree in English from Wesleyan University and studied nonprofit management as a Jane Addams Fellow at the Indiana University Center on Philanthropy. In 2013, she designed what would become HowMuchPhe.org and obtained a grant to develop the app for PKU News. She represents PKU News as a member of the Patients & Providers for Medical Nutrition Equity and the NPKUA Advocacy Committee, and was a member of the working group to establish a rare disease advisory council in New Jersey. Sarah developed and maintains NutritionEquity.org and MetabolicFormula.org, key resources for advocacy for medical nutrition access for those all those who require medical nutrition. Her priorities at PKU News focus on using patient-generated data to improve health and care in all IEM and guide research priorities to support a whole-health approach to IEM management.



FEDERAL ADVOCACY FINALISTS

PATIENT ADVOCATE OR ORGANIZATION



KELLY HELM

New Era of Preventing End Stage Kidney Disease Act

Kelly Helm serves as NephCure's Executive Director of Patient Engagement, working directly with NephCure's regional communities, volunteers and patients. She started with NephCure as a volunteer in 2010 after her daughter, Macy, was diagnosed with FSGS. After working as a regional coordinator for several years, she joined NephCure as a full-time employee in 2014.

As a patient parent, Kelly has had 13 years of hands-on experience with the patient journey. She's also spent the last eight years advocating for patient families, helping them make their voices heard, and advocating for better treatments and care. Her favorite part of working for NephCure is providing hope for patients and their families and helping them feel less alone.

Kelly works from her home office in Colorado Springs, Colorado. In her free time, she enjoys spending time with her husband, Brandon, her children, Macy and Max, and her dogs, Bentley, and Dash.



NATIONAL MPS SOCIETY

RUSP nomination of MPS II

The National MPS Society is a 48-year organization that exists to cure, support, and advocate with MPS and ML. Their mission serves individuals, families, and friends affected by advocating, through family support programs, increasing research, and raising public awareness. They have funded almost \$20 million in research around the world, advocated for patients for 25 years on Capitol Hill, and served families from the time of diagnosis through loss and beyond.



FEDERAL ADVOCACY FINALISTS PATIENT ADVOCATE OR ORGANIZATION



AMY OLIVER

FEHBP Coverage of Medical Foods

Amy J. Oliver is the mother of three children, two of whom have Phenylketonuria (PKU). A seasoned advocate for PKU and rare diseases, she has testified multiple times before the Utah State Legislature and has met with hundreds of Congressional offices in Washington, D.C.

Amy is the President and Founder of the Intermountain PKU and Allied Disorders Association, a non-profit patient organization based in Salt Lake City, Utah. She served as the President of the non-profit National PKU Alliance for seven years, after serving for five years on the board of directors. She was a founding Charity Trustee of the Global Association for PKU, the first international PKU patient organization. Amy also served for more than a decade as a member of the Utah Newborn Screening Advisory Committee and the Utah Newborn Screening Research Review Panel.

Thank you!

The EveryLife Foundation is grateful for the decades of leadership, commitment, and support from Representatives G. K. Butterfield, Jaime Herrera Beutler, Lucille Roybal-Allard, and Fred Upton. They have been consistent and engaged allies to the rare disease community, working with patient advocates to promote policies that expedite regulatory decision-making, research and drug development, and earliest, equitable access to treatments and cures.



REP. G. K.
BUTTERFIELD



REP. JAIME
HERRERA BEUTLER



REP. LUCILLE
ROYBAL-ALLARD



REP. FRED
UPTON



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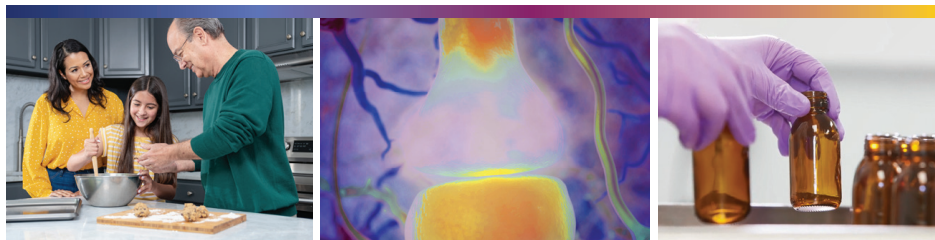
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FEDERAL ADVOCACY: FEDERAL AGENCY STAFF



JONI RUTTER, PH.D.

National Center for Advancing Translational Sciences

Joni L. Rutter, Ph.D., is the Director at the National Center for Advancing Translational Sciences (NCATS). She plans and executes the Center's preclinical and clinical translational science programs. Before joining NCATS, she served as the Director of Scientific Programs within the All of Us Research Program, and prior to that as Director, Division of Neuroscience and Behavior at the National Institute on Drug Abuse.

Dr. Rutter received her Ph.D. from Dartmouth Medical School and completed a fellowship at the National Cancer Institute. She is recognized for her work in basic and clinical research in human

genetics and in the study of genetic and environmental risk factors focusing on the fields of rare disease, cancer, and addiction. Her primary scientific objective is to bring more treatments to all people more quickly. She received a 2022 Leading for Impact, Women in Leadership Impact Award from the Federal IT sector.



2021 RAREVOICE AWARDEES



Alena Galan



Bob Graham



Christina
McCauley



Christine Brown



Dr. Francis
Collins



GA Rep. Sharon
Cooper



Hannah Yale



Kirby Miller



Rep. Frank
Pallone



Senator Burr



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STATE ADVOCACY FINALISTS STATE LEGISLATOR



SENATOR ANTHONY BUCCO

New Jersey

S. 3724, New Jersey Pediatric Cancer Fund

Anthony M. Bucco has served New Jersey's 25th Legislative District for 12 years, representing parts of Morris and Somerset counties in both the Assembly and later the Senate beginning in 2019. During his time in the Legislature, Sen. Bucco has sponsored laws to fund pediatric cancer research, fight human trafficking, improve school security, and help support small businesses impacted by the COVID-19 pandemic. He earned the Legislative Champion Award by the NJ Commission on Cancer Research in 2021.

Bucco is a pediatric cancer survivor first diagnosed at 8 years old. The external radiation treatment and tumor removal in his thyroid severed

one of his vocal cords and later caused permanent loss of vision in his right eye. He understands the challenges of families with serious health complications and has spent a career fighting for the most medically vulnerable populations. A partner in the law firm of Murphy McKeon, P.C., Bucco is a 40-year volunteer fireman who resides in Boonton Township with his wife, Amy. They have six grown children and five grandchildren.



REPRESENTATIVE JARVIS JOHNSON

Texas

S. 3672, Medicaid eligibility for persons diagnosed with sickle cell disease

In the effort to help change the world State Representative Jarvis Johnson has served in many capacities that would make a difference in the lives of many. As an entrepreneur, consultant and developer, motivational speaker, strategist, community organizer, and trainer, he speaks on new opportunities and ideas centered on empowerment and change. For over 30 years he has worked to create harmony and self-sufficiency through community involvement, neighborhood organizing and business development. Since 2016, Johnson has served in the Texas House of Representatives as the State Representative for District 139. While in office, he has filed and

passed legislation focused on improvements of public and higher education programs, the criminal justice system, the relationship between the community and law enforcement, and child protective services. State Representative Jarvis Johnson is a native Houstonian, from 5th ward, a former Houston City Councilman. He is the father of two beautiful children, Nalyah and Jarvis II.



STATE ADVOCACY FINALISTS STATE LEGISLATOR



ASSEMBLYMEMBER BRIAN MAIENSCHIEIN

California

AB 114, Rare Disease Sequencing for Critically Ill Infants Act

Brian Maienschein has represented California's 77th Assembly District since 2012. He currently serves as Chair of the Rare Disease Caucus and sits on the Health, Judiciary, and Communications and Conveyance Committees. Before joining the Assembly, Brian served eight years on the San Diego City Council, and as the city's first Commissioner on Homelessness.

Brian was named the March of Dimes California Legislative Champion for his work to improve Maternal Mental Health. He also received the Easter Seals California Assembly Champion Award for his work with the developmentally disabled community. A graduate of the University of California, Santa Barbara and California Western School of Law, he teaches an Election Law course at USD School of Law. Brian lives in the 77th Assembly District with his wife, Elly, and his two daughters, Taylin and Brenna, who are his proudest accomplishments.

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STATE ADVOCACY FINALISTS PATIENT ADVOCATE OR ORGANIZATION



SHANNAH HUDSON

Newborn Screening RUSP alignment in Mississippi

Shannah Hudson is a rare disease and newborn screening advocate, and mom to Emmalyn who has Glutaric Aciduria/Acidemia Type 1 (GA-1). She founded a registered 501(c)(3) nonprofit organization, Mississippi Metabolics Foundation (MMF), to raise awareness, educate, support, and advocate for patients in Mississippi with rare diseases, specifically Inborn Errors of Metabolism. She currently serves as the CEO and board President for MMF and is a member of the EveryLife Foundation's Community Congress and a founding member organization with the Global Genes Global Advocacy Alliance (GAA). She is on the Patient Experience Bureau for the Child Neurology Foundation, the Patient/Consumer Representative for Mississippi for the Southeast Regional Genetics Network (SERN), and

a Rare Genetic Disease Patient Ambassador with Illumina, Inc. Shannah also serves in advocacy efforts with various other patient organizations including the National Organization for Rare Disorders (NORD), NORD's MS Rare Action Network (RAN), Genetic Alliance, Baby's First Test, and the Organic Acidemia Association (OAA).



COLIN MCEWEN

Newborn Screening RUSP alignment in Oklahoma

Colin McEwen is a fifth generation Oklahoman who worked in nonprofit development and communications until he decided to pursue a career in nursing. However, chronic illness and misdiagnosis wreaked havoc on his ambitions. After 6 years of searching for answers, he was nearly quadriplegic when his wife wheeled him into the Mayo Clinic where he was finally diagnosed with acute hepatic porphyria. He spent the next five years on disability until a groundbreaking new treatment gave him a new lease on life. After the birth of his son in 2020, Colin decided to pursue a career teaching middle school science to help inspire the next generation of research scientists and he works with his hematologist to find, diagnose and support people suffering from AHP. Most importantly, Colin uses his story to advocate for the undiagnosed and those fighting rare disease.



STATE ADVOCACY FINALISTS

PATIENT ADVOCATE OR ORGANIZATION



NATHAN PECK

Georgia Rare Disease Advisory Council

Mr. Nathan Peck is the co-founder and CEO of Cure VCP Disease, Inc., a patient advocacy organization dedicated to driving a cure for valosin-containing protein (VCP) associated multisystem proteinopathy (MSP-1). The disease is an adult-onset, autosomal dominant disease caused by a mutation of the VCP gene on chromosome 9. It can affect any combination of a patients' muscles, bones and brain.

Mr. Peck is a patient with the disease. He has three relatives deceased from the disease and one relative still living with the disease. Mr. Peck is retired, on long-term disability, and has quickly grown the awareness and influence of Cure VCP Disease among the research and patient community since 2018.

Mr. Peck possesses a Bachelor of Industrial Engineering from the Georgia Institute of Technology and MBA and Masters of Civil and Environmental Engineering from the Massachusetts Institute of Technology. He lives in Georgia, USA and is the proud husband of 22 years to Allison and father of three teenage boys.



DEREK ROBERTSON

Funding for Sickle Cell Disease Services in Maryland

Derek Robertson is president of the Maryland Sickle Cell Disease Association. Derek and his wife, Shantá, have three sons, two of whom have sickle cell disease (SCD). He is a member of The National Academies of Sciences, Engineering and Medicine Forum on Regenerative Medicine and a past member of the NIH's National Heart, Lung, and Blood Institute's SCD Advisory Committee. He served on the federal Maternal and Child Health Bureau's Newborn Screening Expert Panel.

Derek is an attorney specializing in the federal 340B Drug Pricing Discount Program and has worked extensively with various institutions on grants management and program income.

Derek holds an MBA from City University of New York - Baruch College and his JD from the University of Houston. He is licensed to practice law in Texas (currently inactive) and Washington, DC. He is certified in Health Care Compliance and is a volunteer mediator in Howard County, Maryland.



TEEN ADVOCACY FINALISTS



CANNON SITTIG

Ensuring Lasting Smiles Act

Cannon Sittig is a Senior at Madison High School in Madison, New Jersey. He was diagnosed with hypophosphatasia (HPP) when he was 18-months old and was the initial face of the disease since his mother founded Soft Bones, the leading patient group for HPP in the United States. He has grown into his role as an advocate as he has grown up, attending patient meetings, traveling to Washington, DC to advocate for rare disease legislation, and most recently, joining the Teen Advisory Council to raise awareness and give a voice to the HPP teenage population. In addition to his advocacy work, Cannon enjoys spending time on the golf course, landing a spot on the Varsity team and earning a Tournament of Champions ring this past year. He lives with his family, 3 dogs and 3 chickens, in New Vernon, New Jersey.



KRTEYU RAMANATHAN

Speeding Therapy Access Today Act

Krteyu was born on July 27, 2006. He moved to the USA from India in 2008. He has always worked to help different causes pertaining to different struggles that are often underrepresented on the main stage. In fourth grade, he moved back to India when his grandpa got brain cancer. Through this experience, he noticed severe issues when it came to immigration, healthcare, and therapy access in India and America. As a result, in fifth grade, he advocated for immigration reform, which then led him to develop a passion for advocacy and rare diseases. Krteyu was inspired by the memory of his grandfather, who despite not having a rare disease struggled with finding therapies and assistance, like the millions of people in India and America who struggle with diseases that don't even have therapies.



TEEN ADVOCACY FINALISTS



AVERY ROBERTS

Speeding Therapy Access Today Act and BENEFIT Act

Avery Roberts is a high school student and rare disease advocate with Congenital Muscular Dystrophy (CMD). She works in community outreach and engagement for Cure CMD, has established a Cure CMD Webinar Series, and expanded Cure CMD's young adult programming by creating a mentorship program and resource page. At twelve years old, she began participating in Rare Disease Week on Capitol Hill. She is very passionate about giving back to the rare disease community. She has raised over \$4,000 as part of Living in the Light & Cure CMD's "I Stay Home for Rare" fundraising campaign, which ultimately raised almost \$100,000 for rare disease families that suffered financially during the pandemic. She volunteers as the social media coordinator for the Hudson Valley Chapter of Canine Companions. She is also a dancer, is a part of two advanced NYC companies, & was in NBC's Annie Live! She wants to encourage more young people to use their voices.



OWEN MAXFIELD AND CLAIRE OLIVER

Medical Nutrition Equity Act and Utah PKU Formula Program

Owen Maxfield is a 15 year-old multi-sport student athlete. He plays competitive basketball, tennis, golf and pickleball. He has a 4.0 grade point average and is part of a service club with his church. He is an entrepreneur and spends his extra time running a successful lawn mowing business, called "Owen's Mowin". He is also an excellent low protein chef!

Claire Oliver is a 15 year-old high school sophomore. She is fluent in French, a talented dancer, and competes for her high school drama team, advancing to the state finals as a freshman. She is an honors student and is taking her third and fourth Advanced Placement courses this year as well as a course at the University of Utah. Claire spent her summer as a youth sports camp counselor. She loves to cook low protein dishes and enjoys traveling with her family.



TEEN ADVOCACY FINALISTS



KRISHANGI SHROFF

Texas Dyslexia Handbook

Krishangi is a 12-year-old disability rights advocate diagnosed with Usher Syndrome, a rare genetic disorder that causes deafness, blindness and balance issues. As a deaf-low vision child with dyslexia, her experience made her realize that we are a long way from having equal education experience for children with disabilities. "I dream that children with disabilities will get access and equal opportunities to succeed in all fields, including higher education," says Krishangi.

Because of her and her mother's advocacy efforts, the Texas Education Agency issued a guidance document that recognizes SLD in children with sensory disabilities.

As a Disability Book Week panelist, she reviews books that promote disability inclusion in literature. She highlights books written by authors with disabilities or books that show real depiction of disabilities; and review accessibility and ease of reading experience for individuals who are low vision or have dyslexia.



Interested in engaging the next generation of advocates?

YARR **Speakers Bureau**

Invite a Young Adult Speaker to Your Next Event

Members of the YARR Speakers Bureau are excited to discuss their experiences and rare disease community priorities.



Join the Young Adult Rare Coalition

This quarterly, no-cost coalition brings together patient organizations supporting young adults with rare diseases to promote cross-collaboration.

*Scan for
more info*



Contact Lindsey Cundiff at lcundiff@everylifefoundation.org.



DIVERSITY EMPOWERMENT FINALISTS PATIENT ADVOCATE OR ORGANIZATION

Award exclusively sponsored by Travers Therapeutics



CONNIE MONTGOMERY

Connie L. Montgomery is a retired Occupational Therapist who is currently serving as a Family Faculty member for the Medical University of South Carolina and a Patient and Family Advisor for PFCC partners in Long Beach, California. She is a consultant with the National Hemophilia Foundation and speaks frequently for the organization. Ms. Montgomery is a national consumer scholar for the Camden Coalition of New Jersey. She is the author of a children's book entitled "Letta Forgives". She has Factor Seven deficiency— a rare bleeding disorder and Pemphigus Vulgaris, an autoimmune disorder alongside insulin dependent diabetes. She is an international motivational speaker. Ms. Montgomery is also the Diversity, Equity, and Inclusion Chair for the nSpiration Foundation, an organization which helps patients and families with chronic, rare

disorders with financial and diagnoses specific education and support. All of her National conference engagements for 2021 have been virtual for the National Organization for Rare Disorders, The Schwartz Center for Compassion in Action conference, and the National Hemophilia Foundation. When she is not busy with those programs she is serving as South Carolina's Public Service Ambassador for Covid Vaccinations with the American Hospital Association. Ms. Montgomery has been married to Dwayne Graham for 32 years and they have two adult children: Blake and Connor and two fur grand cats: Sir Germany and Oscar.



LEAH CAMPBELL

Leah Campbell was diagnosed with Neuromyelitis Optica Spectrum Disorder in May 2006 at 27 and after a 17-year diagnostic odyssey. Because of the rarity of this disorder and having it 15 years before a diagnostic test existed, she incurred severe and multiple disabilities including blindness and paralysis.

She serves on the Patient Advocacy Council for the Guthy Jackson Charitable Foundation. She is a co-group leader for the Oklahoma Support Group of the Siegel Rare Neuroimmune Association. Because of her disabilities she is a board member on both the Central Oklahoma Chapter and the Oklahoma Affiliate of the National Federation of the Blind and a co-chair of the legislative committee in Oklahoma. She is also Chapter Advocacy Coordinator of the Oklahoma City Chapter of the United Spinal Association.



DIVERSITY EMPOWERMENT FINALISTS PATIENT ADVOCATE OR ORGANIZATION

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FRANK RIVERA

Frank Rivera is the Co-Founder/President of Stronger Than Sarcoidosis since August 2020. He is also the Founder/President of Sarcoidosis of Long Island and an advocate, Navigator and Mentor with the Foundation for Sarcoidosis Research. Frank is a two-time published author: an autobiography called "Walking in Silent Pain" and a continuation biography "I Have Sarcoidosis but it Doesn't Have Me", a book about strength and resilience. In December 2017, Frank was named People of the Year in the newspaper organization TBR News Media.

WHAT'S YOUR STORY?

More than 30 million Americans are living with one or more rare diseases

Each one of us has a story. We want to hear yours.

"I was diagnosed with Narcolepsy when I was sixteen years old and I fell asleep at the kitchen table. As a patient advocate, I am enthusiastic about making sure that the home health and telemedicine options that have been expanded due to the pandemic continue to remain in place."

– Nicole E., Texas



SCAN FOR
MORE INFO



40TH ANNIVERSARY OF THE ORPHAN DRUG ACT

Dear Rare Advocates,

Early 2023 marks the 40th anniversary of the Orphan Drug Act, landmark legislation that has saved patient lives and proved the remarkable power of the rare disease patient advocacy movement to influence action on Capitol Hill.

A bipartisan, bicameral success story, the Orphan Drug Act of 1983 (ODA) was sponsored originally by legislative champions Henry Waxman and the late Orrin Hatch. The ODA incentivizes drugmakers to develop drugs for rare diseases. Because of the small communities that comprise rare conditions, research and therapy development of rare diseases had long been considered unprofitable. However, under ODA, pharmaceutical companies were offered tax and marketing incentives to spark research and development for safe and effective therapies for rare diseases.

Since ODA was enacted, FDA has granted approximately 1100 orphan designated approvals, including approvals for approximately 700 novel drugs and biologics. That reflects a massive effort and a sea change in comparison to when only ten drugs brought to market between 1973 and 1983 would have qualified as orphan drugs under today's ODA designation guidelines.

Still, more work remains to be done as there are more than 10,000 known rare diseases and only about five percent that have FDA-approved therapies.

The EveryLife Foundation joins the rare disease community in 2023 in marking the anniversary of this lifesaving legislation. At the same time, we will be calling on advocates not only to celebrate this historic win for rare disease patients but also, to protect the incentives provided under ODA and expand its impact for this and future generations of people living with rare disease.

Follow the EveryLife Foundation for news, events and resources so that we can protect and expand the benefits of ODA to even more patients and families.

Sincerely,

Julia Jenkins
Executive Director,
EveryLife Foundation for Rare Diseases

***Let's hear your voice
February 28-March 2
at Rare Disease Week
on Capitol Hill.***



Senator Orrin Hatch with EveryLife Board Member Cristina Casanova Might



Parent advocate and former EveryLife Board Chair Mark Dant with Rep. Henry Waxman



ARTIST-TO-ADVOCATE FINALISTS

Award exclusively sponsored by WCG Clinical



LAURA ROMANO

Laura Romano (They/Them) is a 25-year-old patient advocate from Groton, MA. They were diagnosed with Classical-Like Ehlers-Danlos Syndrome (cLEDs) in 2021 at the age of 23 following a 13-year diagnostic odyssey. Laura is a graduate of the YARR Leadership Academy and a member of the YARR Speakers Bureau. Laura recently launched their podcast, *Sharing Our Stripes*, on Spotify. Laura holds a B.S. in Neuroscience from Simmons University and is currently pursuing a M.Ed. in Early Childhood Education from Fitchburg State University while working as a Pre-K teacher.



ANNELIESE WILLIAMS

Anneliese Williams studies public health at Purdue University and conducts global pediatric oncology research to improve access to care in low- and middle-income countries. During her junior year, she was diagnosed with Guillain-Barré Syndrome, a rare condition hallmarked by rapid ascending paralysis. With her recovery, she discovered a passion for advocacy and has become involved with YARR. She has participated in *Rare Across America*, the YARR Leadership Academy, and *Rare Artist* to spread awareness of issues faced by the rare disease community and to promote legislative change. She was featured as a guest on the podcast, *Making Our Way*, where she shared her rare disease advocacy journey through spoken word and has become actively involved in fighting for disability rights and access in her community.



ARTIST-TO-ADVOCATE FINALISTS

Award exclusively sponsored by WCG Clinical



ALI SP

Ali is a rare disease advocate, self-taught artist, writer and board-certified endodontist. She was diagnosed with hypocomplement urticarial vasculitis in 2019. Ali has used art not only as a coping mechanism but also as a tool to spread awareness and advocate. Her preferred medium of choice is acrylic. As a writer, she enjoys writing fiction and poetry. In 2021, her poem *Painting Me*, was awarded an honorable mention in Writer's Digest's 90th annual writing competition. She was also one of the 2021 rare artist award recipients for her painting, *Intensity*. Ali has spent the last year using art and writing to share her story while advocating for changes in legislation.

RAREARTIST

POWERED BY THE EVERYLIFE FOUNDATION



Congratulations to the 2022 Rare Artist Awardees:

Adults: Wes Burian, Carina Imborno, Christina Schulz

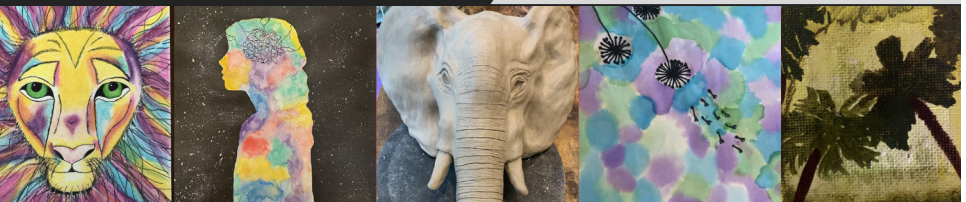
Young Adults: Laura Romano, Miranda Walker

Teenagers: Rylie Erbacher, Aanya Khattar, Nell Choi

Children: Lonnie Lu Rivera, Brenna Bruck

2023 Rare Artist Contest will open for submissions July 2023

All Rare Artist Finalists will be invited to participate in Artist-to-Advocate individualized coaching with the EveryLife Staff in October 2023.



RAREARTIST.ORG

CONGRATULATIONS TO ALL RARE DISEASE PATIENT ADVOCATES NOMINATED THIS YEAR

Jonathan Alvarez

Sylvia Anspach

Felicia Bannon

Melodie Blackwell

Lesa Brackbill

Carol Cross

Sarita Edwards

Gina Glass

Cynthia Howard

Andre Harris

Keely Harris

Brian and Tia Jones

Lynn Julian

Taylor Kane

Stephanie Kennedy

Angelica Lamas

Dorothea Lantz

Demeshia Montgomery

Ambre Minty

Mary Mecham

Mary Morlino

Mia Maysack

Hannah Mehta

Beth Nguyen

Doug Passow

Jeremy Penn

Deborah Rice

Cristina Rosa

LaShardae Scott

Bonnie Schneider

Katie Stevens

Melissa Tumblin

THANK YOU TO THE RAREVOICE NOMINATIONS COMMITTEE

Dr. David Eckstein, Office of Clinical Research, NIH

Dr. Stephen Groft, EveryLife Foundation Board Member

Sarah-Lloyd Stevenson, Amazon

Lisa Schill, Patient Advocacy and Engagement Consultant

Wendy Erler, Alexion Pharmaceuticals

Shayne Woods, Congressional Staff

RARE DISEASE WEEK ON CAPITOL HILL



POWERED BY THE EVERYLIFE FOUNDATION

FEBRUARY 28 – MARCH 2, 2023



**REGISTRATION FOR RARE DISEASE ON CAPITOL HILL 2023
WILL OPEN ON JANUARY 4, 2023!**

[RAREADVOCATES.ORG/RDW](https://rareadvocates.org/rdw)

@RAREADVOCATES

#RAREDC2023

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



We are an innovative global healthcare company, driven by one purpose: we chase the miracles of science to improve people's lives.

In Specialty Care, our mission is to help people with debilitating and complex conditions in rare diseases, rare blood disorders, neurology, oncology, and immunology. These conditions are often difficult to diagnose and treat. But we aren't afraid of challenges. They just push us to work harder, to chase new potential therapies that help patients to live their lives.

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