
RARE
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WELCOMES YOU TO THE

2022 RARE IMPACT AWARDS®

PROGRA
FOR
THE
EVENIN

WELCOME
Art Alexakis, Emcee for the Evening

OPENING REMARKS
Peter L. Saltonstall, President and CEO, NORD

PRESENTATION OF AWARDS

MUSICAL PERFORMANCE
Art Alexakis, Everclear

DINNER

PRESENTATION OF AWARDS

CLOSING
Dessert Reception

DEAR FRIENDS

Thank you for joining us for the 2022 Rare Impact Awards, presented live in Cleveland, Ohio at the Rock and Roll Hall of Fame. What a joy it is to reunite our community safely in-person for the first time since 2019. Despite the enormous challenges we have faced during the pandemic, the rare disease community has consistently uplifted one another – for that I salute each and every one of you.

And so, what a perfect opportunity we have here to honor not only the innovation, achievements, and advocacy of the rare community over the last year, but the resiliency and heroes among us from whom we can draw inspiration to keep our rare family moving forward into the future. The Rare Impact Awards are truly a celebration, and one that belongs to our entire community.

At the National Organization for Rare Disorders (NORD®), we are proud to honor a new group of individuals and companies each year for their achievements in the rare disease world. We're humbled to work alongside this group, and we show endless appreciation for an unparalleled tenacity and commitment to creating a better, more inclusive rare future. This year, we are proud to welcome back the Rare Impact Award honorees from 2020 and 2021, who deserve the in-person recognition of our community, as all the honorees before them have had.

NORD began as a collective of patients and caregivers advocating for the Orphan Drug Act back in the early 1980s, and we continue to make an impact for the nearly 30 million Americans and 300 million people worldwide affected by rare disease because we celebrate our past as we strive for a better future. I'd like to take a moment to thank the tireless rare disease advocates who continue to drive change that benefits the entire community, congratulate the companies that had a breakthrough therapy approved, and acknowledge the hard work of our federal partners at the US Food and Drug Administration (FDA) and the National Institutes of Health (NIH), who help make so much of this progress possible.

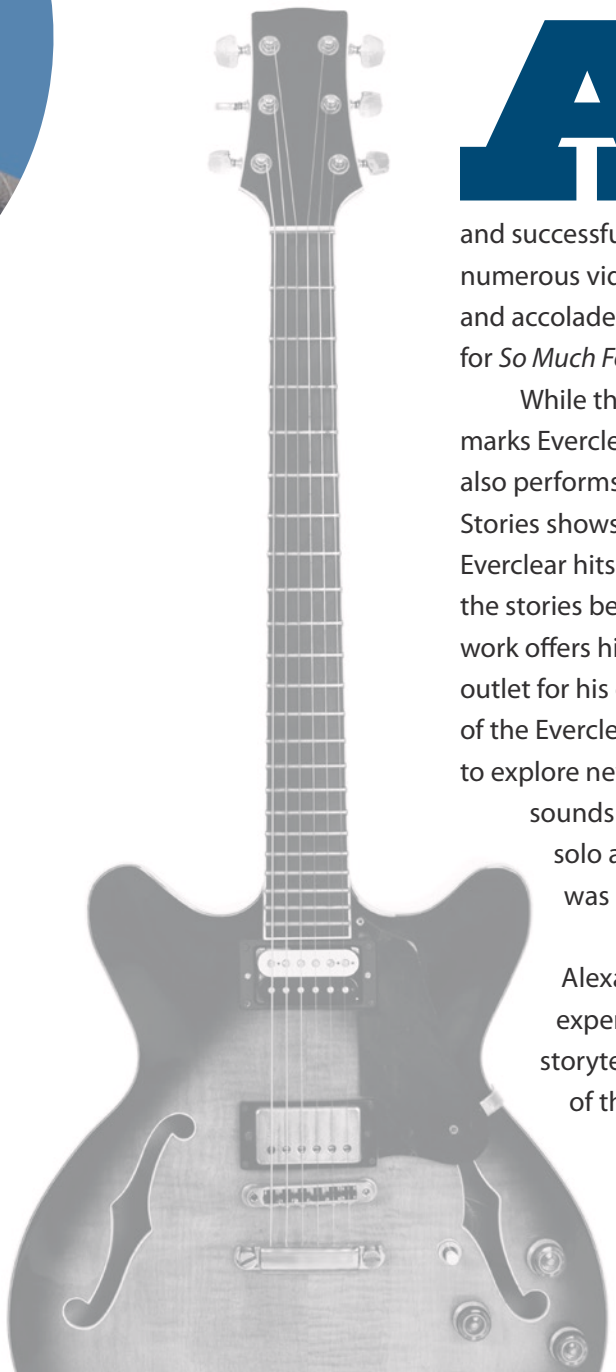
Our sincere thanks for joining us for this program to celebrate your contemporaries, colleagues, and family. Alone we are rare, together we are strong®. And tonight, we are certainly strong.

A heartfelt congratulations to all our Rare Impact Award and Industry Innovation Award honorees.

PETER L. SALTONSTALL
PRESIDENT & CEO

EMCEE FOR THE EVENING

ART ALEXAKIS



Art Alexakis is the master of ceremonies for the 2022 Rare Impact Awards.

As singer-songwriter and guitarist of rock band Everclear, Alexakis has enjoyed a lengthy and successful career, spanning 11 studio releases, numerous videos, thousands of live shows, and accolades including a 1998 Grammy nomination for *So Much For The Afterglow*.

While the band remains active today—2022 marks Everclear's 30th anniversary—Alexakis also performs solo during his Songs & Stories shows, where he strips down Everclear hits and fan favorites and tells the stories behind the songs. Art's solo work offers him a new and different outlet for his creativity, where outside of the Everclear realm, Alexakis is free to explore new styles and different sounds as evident on his debut solo album *Sun Songs* that was released in 2019.

NORD is thankful Art Alexakis is lending his musical expertise and passion for storytelling to the role of emcee of the 2022 Rare Impact Awards.

"The Rare Impact Awards are a celebration of the heart, spirit, and collective strength of the rare disease community. This ceremony is about the amazing work that has happened and the progress that can happen, and NORD is proud to recognize the tireless commitment of all the nominees and companies who earned awards tonight."

PETER L. SALTONSTALL, PRESIDENT & CEO

2022 ★ HONOREES

RARE IMPACT AWARDS®

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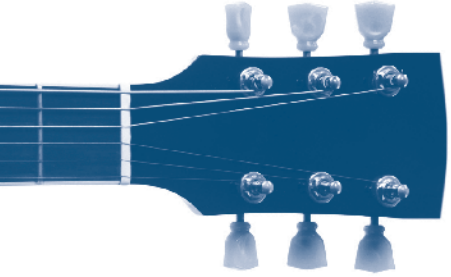
LIFETIME ACHIEVEMENT AWARD

MARSHALL L. SUMMAR, MD

Marshall L. Summar, MD, is Chief of the Division of Genetics and Metabolism at Children's National Hospital and holds the Margaret O'Malley Professorship in Genetic Medicine.

Dr. Summar has worked in the field of rare disease as a clinician since 1985. Since his training in pediatrics and then genetics, Dr. Summar's clinical work has focused on finding new and better ways to treat rare disease patients. His work has been inspired by the the strength and determination of the thousands of individuals with rare disease that he has met over the years. Dr. Summar has also worked with many organizations on issues around rare diseases and ways to provide care for patients, including American College of Medical Genetics (ACMG), the National Institutes of Health (NIH), NORD, Society for Inherited Metabolic Disorders (SIMD), and more.



Beyond his research and medical career, Dr. Summar has been a big champion of patient advocacy and has developed longstanding relationships with patient advocacy organizations in the United States and around the world. He has supported the recognition of rare diseases within the field of medical specialties, spearheaded the development of the Rare Disease Institute at Children's National Hospital, and oversees the largest clinical division of its kind in the world, treating more than 7,000 patients per year with rare diseases.

Dr. Summar served on the NORD Board of Directors for nine years, including six years as Chairman.

For decades of excellence and commitment to individuals, families, and organizations combatting rare diseases, NORD is proud to present Dr. Summar with the Lifetime Achievement Award.

"Working in this field gives one a distinct sense of purpose that you do not find elsewhere. No other field of medicine has the engagement that exists in rare disease. Get involved with patient advocacy groups. They will pick you up if you are feeling low, and they remind you of why you are there."

SICKLE CELL DISEASE ASSOCIATION OF AMERICA INC.

For more than 50 years, the Sickle Cell Disease Association of America Inc. (SCDAA) has been in the forefront of advocating, educating, and increasing awareness of the disease and the full spectrum of how it affects the body.

Formed from a coalition of 15 community sickle cell organizations and now an umbrella organization of 53 member organizations, SCDAA is a consistent global leader and voice for warriors, caregivers, providers, colleagues, coaches, teachers, friends – all those who support those living with sickle cell disease. The mission of SCDAA is to advocate for those affected by sickle cell conditions and empower community-based organizations to maximize quality of life and raise public consciousness while advancing the search for a universal cure.

> ***"Caregivers should be honored. Community-based organizations should be honored. Dedication should be recognized. The landscape for a universal cure looks bright. The goal will be to make it accessible and affordable to all who need it."***
>
> **REGINA HARTFIELD, CEO AND PRESIDENT, SCDAA**

Thanks to an emphasis that stretches far beyond just the wellness of sickle cell disease patients, SCDAA has elevated health equity for all rare diseases, specifically working to educate marginalized rare disease communities around gene therapy and CRISPR technologies. An organizational effort around inclusion for black and low-income Americans who have been historically underrepresented in registries and other research has led to partnerships between SCDAA and organizations including American Red Cross, NIH, and Pfizer. SCDAA has made identifying and including diverse populations who are often left behind in research, clinical trials, gene therapy and blood donations a priority nationally and internationally for the rare disease community.

SCDAA's accomplishments and leadership on Capitol Hill are also particularly noteworthy, including major legislative victories in Congress, such as the passage of the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act on February 26, 2018, and its companion bill in the Senate later that month, which elevated the disease to the national spotlight and sought to improve data collection on the prevalence of sickle cell disease.

NORD is proud to bestow the Abbey S. Meyers Leadership Award to the Sickle Cell Disease Association of America Inc.

PUBLIC HEALTH LEADERSHIP AWARD

MARIA ELENA BOTTAZZI, PHD & PETER HOTEZ, MD, PHD

Peter J. Hotez is a professor of pediatrics and molecular virology at Baylor College of Medicine, where he co-heads the Texas Children's Center for Vaccine Development. He is the author of the book "Preventing the Next Pandemic: Vaccine Diplomacy in a Time of Anti-Science". Dr. Hotez's daughter, who has autism, is a major inspiration and driver of his work. Maria Elena Bottazzi is co-director of the Texas Children's Hospital Center for Vaccine Development. She is a professor of pediatrics and molecular virology and microbiology at Baylor College of Medicine and associate dean of its National School of Tropical Medicine.

The dynamic duo of Dr. Hotez, MD, PhD and Dr. Bottazzi, PhD have been partners since 2001, in part due to an interest in neglected tropical diseases and a long desire to help eradicate those tropical diseases through a multi-step approach and collaboration.

Dr. Hotez and Dr. Bottazzi are being honored by NORD for their innovative approach to public health and vaccine development and adoption that focuses on global equity, partnership, and access. Driven by a desire to develop vaccines for infectious diseases for those in need around the world, the two have spent decades focusing on parasitic disease vaccines for the poor. The development of their vegan COVID-19 vaccine was made possible through an inclusive model built upon partnership with ministries of health/science and vaccine producers, an emphasis on access, addressing vaccine hesitancy, and engaging with the disease community directly.

> ***"Collaboration is not a one way street, we have to find value with our partners but we also have to have accountability to align with the ultimate goal."***

With the world—and the rare disease community in particular—facing such stern challenges during and due to the pandemic, Dr. Hotez and Dr. Bottazzi developing a vaccine through partnership and a commitment to diversity has been a tremendous contribution to the greater health and safety of individuals with rare disorders.

NORD is honored to present Dr. Peter Hotez and Dr. Maria Elena Bottazzi with the Public Health Leadership Award.

2022 RARE IMPACT AWARD HONOREE

LESLEY BENNETT

Lesley Bennett is an advocate and rare mom. She has worked in healthcare for 50 years, has been a volunteer with NORD's Rare Action Network® (RAN) program since its inception in 2016 where she has developed a proven record of getting things done in Connecticut on issues impacting the rare disease community. For her leadership, stewardship, and exceptional advocacy efforts, Lesley now occupies the role of the RAN Connecticut Volunteer State Ambassador.

Lesley successfully spearheaded a campaign to create a rare disease task force within the state legislature and as of press time is hard at work trying to create a permanent Rare Disease Advisory Council (RDAC) in Connecticut. Lesley has worked on several important legislative issues, including step therapy, newborn screening, medical nutrition, and co-pay accumulators. She has built strong relationships with state legislators, hosted numerous in-state events and activities, and established an extensive network of rare advocates working alongside the Connecticut General Assembly in the last six years, all thanks to her tireless commitment and advocacy.

> ***"I talk with everybody, I work with all the rare disease groups. That's the key, working with everyone, trying to keep everyone together."***

This past summer, Lesley lost her daughter, who suffered from several rare diseases. Despite personal hardship, Lesley continues her efforts today and, in many ways, is even more motivated to help the rare community.

NORD is proud to present Lesley Bennett with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

BO BIGELOW

Bo Bigelow is a rare dad, nonprofit founder, film festival organizer, and advocate extraordinaire. His family's rare journey began with his young daughter and her unknown, ultra-rare genetic condition. Faced with a long diagnostic odyssey and no existing support group, Bo created the Foundation for USP7-Related Diseases (usp7.org). The Foundation for USP7 Related Diseases is now a NORD member and will soon establish a registry for patient data.

As the chair of his foundation, Bo built the support system, community, and the plan to fund and advance research on what has since been named Hao-Fountain Syndrome. Thanks to his tireless efforts and a commitment to storytelling, sharing and connecting with families across the globe, there are now more than 100 families in their community.

> ***"It isn't a zero-sum game – we all benefit the more people talk about rare. It's about the families that come next."***

Along with Daniel DeFabio, Bo later co-created DISORDER: The Rare Disease Film Festival, to shine a light on patient stories for all those battling a rare disease. In 2020, when the pandemic derailed plans for the next iteration of the in-person festival, he co-created the DISORDER streaming channel, which features short films dedicated exclusively to rare disease stories. Bo's dedication is to break down barriers and combat the isolation of rare disease families and patients. While he initially quit his job to become a rare advocate full-time, he has since returned to his former day job as a lawyer, fighting for families of special needs children when the terms of their Individualized Education Programs are not being met by their school system.

Recognizing his efforts to bring people together, tell stories, and advocate at the highest level, NORD is proud to present Bo Bigelow with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

MELINDA BURNWORTH, PHARMD, BCPS

Melinda Burnworth is a pharmacist educator. She currently fills the roles of professor at Midwestern University-College of Pharmacy Glendale Campus and as a clinical pharmacist. In addition to her incredible career paying forward her training to the next generation of clinicians, pharmacists and rare care providers, Melinda's calling is as a rare advocate, inspired by her late niece, Ella Paige.

Melinda has made a lasting impact on the rare disease community through local, state, and national efforts to advocate and bring awareness. She has held several leadership roles with the Arizona Pharmacy Association (AzPA) including Chair of the Health-System Academy from 2007–2009 and developing and implementing the inaugural AzPA Mentor Connection Program. In 2018, she joined NORD's Rare Action Network (RAN) and was soon appointed as the Arizona Volunteer State Ambassador. She was instrumental in organizing the inaugural Rare Disease Day in Arizona and the signing of the state proclamation officially recognizing Rare Disease Day in the state of Arizona. She is a firm believer in 'advocacy as a family affair' and building community to impact change.

> ***"Don't be afraid to care and share about rare. Keep talking. Don't be dissuaded by people who are unaware."***

With an eye on the future and the future of rare medicine, Melinda has developed a rare program for her pharmacy students. She created the only elective course on rare diseases—and won't stop there. Her innovative program requires students to host a Rare Disease Day event each year and assist in organizing the in-state RAN events. Melinda has grand aspirations for the future of rare, especially towards precision medicine, and helping healthcare professionals narrow the gaps that rare disease patients and families experience.

As a leader in promoting student mentorship, advocacy, and rare diseases education, NORD is thrilled to present Dr. Melinda Burnworth with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

THE HONORABLE G.K. BUTTERFIELD

Congressman G.K. Butterfield is a Member of the U.S. House of Representatives, representing North Carolina's first district.

Representative Butterfield has spent decades in public service, first as a lawyer and judge, and now in Congress, where he has represented North Carolina since 2004. Among Rep. Butterfield's many remarkable achievements has been his lasting impact on the rare disease community.

From his position on the House of Representatives Energy and Commerce Health Subcommittee, Rep. Butterfield has overseen and guided legislation that facilitated new treatments and improved the lives of rare patients. Rep. Butterfield was one of the original champions of the Creating Hope Act, which authorized the development of the rare pediatric disease priority review voucher (PRV) program at the FDA. The PRV program has generated significant investment in rare disease treatments and cures, and it has been authorized by Congress for an additional four years.

> ***"Be persistent. A lot of issues facing rare disease patients and researchers are complex and it takes time to educate members and their staff. But if you take the time to help folks understand they tend to be very receptive. No one wants to harm rare disease patients; they just may need a push to figure out how to best help."***

Rep. Butterfield has acted as the co-chair of the Rare Disease Caucus and the Childhood Cancer Caucus and helped ensure the voice of rare disease patients are heard in Congress. For his work with NORD to ensure that the rare disease patients are not left behind at the highest level, we are proud to honor Rep. G.K. Butterfield with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

RICH HORGAN

Rich Horgan is the Founder and President of Cure Rare Disease, a nonprofit biotech that is developing CRISPR-based gene therapies for people living with rare disease. His rare disease journey began with a personal connection but continues to be driven by his passion for building things and reducing the burden on patients in the drug development world.

Rich founded Cure Rare Disease in 2017 because he was not satisfied with the lack of treatment options for his brother, Terry, who has Duchenne Muscular Dystrophy. In the years since, Rich has built a collaborative network of top clinicians, researchers, and industry leaders who joined in his effort to develop a life-saving treatment for Terry and others living with rare genetic diseases that have no treatment or cure.

> ***"Collaboration is everything. And not just to say 'collaboration'. No one has the right answer, but we strive in the direction that is driven by the science."***

Rich has led a collaborative approach with the FDA and health insurers to create a supportive ecosystem of regulators and payors. Because ultra-rare diseases are often not too commercially interesting to companies, Rich has worked tirelessly with his team to develop drugs in academic clinical studies, to reduce risk and build partnerships with researchers and industry alike. This work will have broad impacts for all rare disease treatment and will continue to scale up in the next five years, addressing value-based reimbursement, partnership in technologies, and an expansion into research for other rare diseases.

Rich is passionate about assisting rare disease families as they navigate the healthcare ecosystem, including helping them to find clinicians and start research efforts. Rich is also a member of the Patient-Centered Outcomes Research Institute (PCORI) Rare Disease Advisory Panel.

For an innovative but real-world approach, an uncompromising vision of advancing into next generation technologies, and a sustainable community-centric outreach to combatting rare diseases, NORD is thrilled to present Rich Horgan with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

TAMMY JONES

Tammy Jones is a patient, advocate, rare mom and grandmother and leader in the rare disease community.

Tammy has been a stable fixture of the NORD Rare Action Network® (RAN) for many years, and for the last four years, she has acted as the Arkansas State Ambassador of the RAN program.

Tammy helped build a solid network of engaged local advocates, working tirelessly to develop contacts within the Arkansas community and state legislature in order to put the rare disease community front and center. From her position on NORD's Policy Steering Committee, she has been an influential figure driving local and national events and legislative initiatives. Most recently, Tammy has been on the frontline working to establish a Rare Disease Advisory Council (RDAC) in Arkansas.

Tammy and her family all suffer from a rare blood disorder, Hemophilia, but together they raise awareness and inspire hope for other families and communities like them. Tammy is always willing to share her family's story in hopes that others will not have to endure what her family did to gain access to adequate coverage for life-saving medications and treatments.

NORD is honored to present Tammy Jones with a 2022 Rare Impact Award.

"That's when I became an advocate—because I decided I don't want anybody to struggle and suffer the way I did for over 30 years by having all these symptoms that don't make any sense."

2022 RARE IMPACT AWARD HONOREE

THE KIDS CARING FOR KIDS (KC4K) YOUTH ADVISORY BOARD OF THE AVALON FOUNDATION

The Avalon Foundation is a collection of patients, caregivers, and advocates.

Formed in 2017, by kids and for kids, the structure of the organization is uniquely youth centric. The organization features two main boards working in tandem; the KC4K Advisory Board, consisting of eleven kids ages 10–18, who lead the foundation, and the adult board of directors who facilitate the advisory board's initiatives. This youth leadership program is uniquely structured on a Four Pillar model of Leadership, Compassion, Contribution, and Commitment. Every aspect of the KC4K Program is a facet of these pillars and actively benefits individuals with rare disease receiving painful medical treatments.

> ***"Inspiration is just being able to make a direct impact on patients and being able to know that even a small act can largely impact."***

The Avalon Foundation is cultivating future leaders while serving and supporting the pediatric rare disease community in critical chapters of their lives. In addition to support during critical treatment for the Hypophosphatasia pediatric community, the KC4K has initiated campaigns and educational efforts to the broader public.

Members of the youth advisory board take their messages to families and the community in a variety of ways, including in media and podcasts, schools, and publications and through events, presentations, fundraisers and more. Their efforts to start an honest, accessible conversation in which all are welcome to ask questions has been instrumental in raising awareness about inequity in the rare disease field.

For representing the future leaders in the fight against rare diseases, NORD is thrilled to present The Kids Caring for Kids (KC4K) Youth Advisory Board of the Avalon Foundation with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

MICHAIL LIONAKIS, MD, SCD

Dr. Michail Lionakis is an internal medicine and infectious disease trained physician. After completing his clinical training in Internal Medicine at Baylor College of Medicine and Infectious Diseases at the National Institute of Allergy and Infectious Diseases (NIAID), Dr. Lionakis joined the Laboratory of Molecular Immunology (LMI) in 2008 to work on fungal immunology.

Throughout his journey, Dr. Lionakis has always made patient care a priority. As a physician scientist, he has always been interested in helping patients and working at the research level. For the past 10 years, Dr. Lionakis has examined and assembled APECED/APS Type 1 patients, the largest patient cohort for this rare disease in the world. By building a team of 15 specialists at NIH to examine this population in a thorough and comprehensive manner, Dr. Lionakis remains a reliable expert and steadfast beacon of hope for rare disease research, evaluation, and treatment.

"When it comes to APS-1, our long-term dream is that we leave behind approved treatments and prevention strategies to change the natural history of the disease."

His latest work has centered around understanding how COVID-19 affects individual patient groups and how to protect them against COVID-19. Dr. Lionakis has worked hard to emphasize information sharing to keep the rare disease community informed throughout this current health crisis.

NORD is honored to present Dr. Michail Lionakis with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

KATHLEEN MURPHY

Kathleen Murphy is a District Delegate who has represented Virginia's 34th District for six years.

In 2021, Delegate Murphy sponsored Virginia's Rare Disease Advisory Council (RDAC) bill that passed through Virginia's General Assembly unanimously. Del. Murphy worked alongside numerous rare disease advocates in Virginia to advocate for the establishment of Virginia's RDAC, which is currently helping to give the rare disease community a stronger voice in Virginia's state government.

Del. Murphy started the Rare Disease Caucus in Virginia to help families who deal with the challenges of a child or family member who has a rare disease. She has supported Virginia's rare disease community by speaking at Virginia's Rare Disease Day events and sponsoring legislation that support rare disease patients and their families.

> ***"There are special needs for those who have rare diseases. There are financial challenges and the challenge of getting the needed medications... If people aren't diagnosed early, they can have horrible consequences."***

Del. Murphy continues to act as a fierce and compassionate advocate for Virginia's rare disease community. For years of dedication, support, and leadership on behalf of the rare disease community locally and nationally, NORD is proud to present District Delegate Kathleen Murphy with a 2022 Rare Impact Award.

2022 RARE IMPACT AWARD HONOREE

JACK TIMPERLEY

Jack Timperley is a rare patient and advocate, living with Fanconi Anemia, a rare genetic disease which mainly affects the bone marrow resulting in decreased production of all types of blood cells. But you may know Jack better as his superhero alter ego, Captain Marrow.

At the age of three, a bone marrow donor gave Jack a second chance at life, and he has set out to make every moment count. His alter ego, Captain Marrow, fights to empower others to be the captain of their own story. Jack is the living embodiment of full commitment, enduring hardship, and assisting others. His mission is to help people living with rare diseases become their own superheroes by recognizing their own unique origin stories and igniting their will to fight on every day.

Jack partners with nonprofits, schools, businesses, and families to educate communities, support patients undergoing medical procedures, and advocate on behalf of individuals with medical illnesses on a national and global scale. Through public speaking and his first-person comic book, Jack shares inspiring stories of other heroes he's encountered, and the journey ahead of him. By bringing together the medical field with the world of superheroes, his message resonates with all in the public sphere: individuals with medical conditions are worth saving because life is worth living.

For being a constant source of inspiration, education, and advocacy, as well as a strong connection within the global rare disease community through the Fanconi Anemia Research Fund, NORD is proud to present Jack Timperley with a 2022 Rare Impact Award.

"Put in the time and effort to really get to know the people behind the disease. My work has made me feel like I'm a part of something so much bigger than myself, given me the strength and courage to keep advocating for others among us and those who will come after us, while honoring those who are no longer with us."

2022 RARE IMPACT AWARD HONOREE

NORA WONG, PHD

Nora Wong is a rare mom, caregiver, and advocate. In 2013, Nora's healthy 22-year-old son Daniel was suddenly afflicted with continuous seizures and later died, following three months in the hospital in a medically induced coma. Nora was determined to find the cause of his death and united clinicians, scientists, and families behind a common name for Daniel's illness—New Onset Refractory Status Epilepticus (NORSE)—and to identify its causes and potential treatments.

In 2014, Nora encouraged doctors to file the first report of NORSE as a rare disease to NORD's Rare Disease Database and NIH's Genetic and Rare Diseases Information Center. This report later became the first report of NORSE on the Epilepsy Foundation's website. After driving efforts to arrive at a consensus definition of NORSE, Nora endeavored to create a community around this rare disease. In 2015, she founded the NORSE Institute, an organization focused on increasing awareness of NORSE, supporting research, and developing a shared community of researchers and families. Nora established the NORSE Institute's website as a resource for scientists, clinicians and families seeking to learn more about the disease, and as a portal for the International NORSE Family Registry, a study that Nora helped design and fund.

> ***"It's not just sorrow that you feel when someone dies, there's a lot of anger, guilt and confusion, so I tried to write, and encourage people to think about what they're feeling and thinking and then communicate to others."***

Nora has always recognized the importance of clinician and scientist collaboration regardless of organization affiliation. In addition to her work in NORSE, Nora is a strong advocate of better communication between medical professionals and patients' families. She emphasizes the power of family networks and how much can be done when clinicians, scientists, and families work together.

Through her efforts to raise awareness and help families who have experienced loss, Nora has made a difference for so many, and opened a conversation about grief, loss, and openness that has struck a powerful chord with the entire rare disease community.

NORD is honored to present Nora Wong with a 2022 Rare Impact Award.

2022 INDUSTRY INNOVATION AWARD HONOREE

APELLIS PHARMACEUTICALS FOR EMPAVELI™

Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare, chronic, life-threatening blood disease characterized by red blood cell destruction, anemia, blood clots and impaired bone marrow functions.

Many PNH patients are seeking choices in their treatment, so the approval of Empaveli™ brings new promise for the PNH community. Empaveli™ is the first and only approved PNH treatment that binds to compliment protein C3, and as a therapy, it has the potential to improve the lives of patients with PNH by increasing hemoglobin and reducing blood transfusion requirements. The treatment received priority review, fast track, and orphan designation from the FDA.

Apellis is committed to helping patients with treatment access and support, and their ApellisAssist™ program was specifically designed to provide comprehensive product support to patients throughout their treatment journey.

NORD is proud to present Apellis Pharmaceuticals with a 2022 Industry Innovation Award for bringing this important new treatment option to patients.

Apellis

2022 INDUSTRY INNOVATION AWARD HONOREE

BRIDGEBIO PHARMA FOR NULIBRY™

Molybdenum cofactor deficiency type A (MoCD) is an ultra-rare, life-threatening genetic disorder that progresses rapidly, results in severe and largely irreversible neurological injury, and has a high infant mortality rate. Until recently, the only treatment options for MoCD were supportive care and therapy for complications.

Then, on Rare Disease Day 2021, BridgeBio Pharma, Inc. and its affiliate Origin Biosciences, Inc. announced that the FDA approved NULIBRY™ (fosdenopterin) for injection as the first therapy to reduce the risk of mortality in patients with MoCD.

The development and approval of this treatment is a huge win for the patient community. As the first approved therapy for this disease, Nulibry will directly contribute to new hope and saved lives. The Nulibry treatment was granted Priority Review and Breakthrough Therapy status, as well as orphan drug designation, by the FDA. Since 2015, BridgeBio and its team of experienced drug discoverers, developers and innovators have worked to create life-altering medicines that target well-characterized genetic diseases at their source, such as MoCD.

NORD is excited to present BridgeBio with a 2022 Industry Innovation Award for bringing this important new treatment to patients.

bridgebio

2022 INDUSTRY INNOVATION AWARD HONOREE

BRISTOL MYERS SQUIBB AND 2SEVENTY BIO FOR ABECMA®

Multiple myeloma is an incurable cancer that forms in the white blood cells known as plasma cells.

Abecma® is the first FDA approved cell-based gene therapy for multiple myeloma and is approved for the treatment of adult patients with relapsed or refractory multiple myeloma.

Developed collaboratively by Bristol Myers Squibb and 2seventy bio, the journey to approval of Abecma started nearly a decade ago and has been driven by a hope and mission to provide multiple myeloma patients with a new approach to fight this relentless disease. This achievement was made possible by a massive network and community, featuring patients, caregivers, investigators and healthcare staff who participated in clinical studies, as well as a tremendous collaboration with the FDA. Abecma was granted orphan drug and breakthrough therapy designations.

Following approval of the treatment, a network has been established to support rapid and dependable manufacturing of Abecma and ensure capacity to accommodate patient demand. Abecma will be manufactured for each individual patient using the patient's own T cells at Bristol Myers Squibb's state-of-the-art cellular immunotherapy manufacturing facility in New Jersey.

NORD is thrilled to present Bristol Myers Squibb and 2seventy bio with a 2022 Industry Innovation Award for bringing this important new treatment to patients.

2022 INDUSTRY INNOVATION AWARD HONOREE

ENZYVANT THERAPEUTICS FOR RETHYMIC®

Pediatric congenital athymia is an ultra-rare immune disorder with an estimated incidence of about 17 to 24 live births each year in the United States. Children who have this condition are born without a thymus and face profound immunodeficiency and potentially fatal infections. With only supportive care until this year, children with congenital athymia typically die by age two or three.

Rethymic® is new, unique and potentially transformative therapy; this one-time regenerative tissue-based therapy is designed to regenerate the thymic function in children and does not require donor-recipient matching. Through FDA approval, the treatment was granted a Priority Review Voucher (PRV) under the Rare Pediatric Disease Program.

This therapy is the result of more than 25 years of research aimed at increasing survival for patients who previously had very little hope. Enzyvant's research program was inspired every day by the possibilities that exist for children who have ultra-rare and devastating conditions.

NORD is proud to present Enzyvant Therapeutics with a 2022 Industry Innovation Award for bringing this important new treatment to patients.

ENZYVANT

2022 INDUSTRY INNOVATION AWARD HONOREE

KEDRION BIOPHARMA FOR RYPLAZIM®

Plasminogen deficiency type 1, or C-PLGD, is a lifelong rare disorder that can impair normal tissue and organ function and may lead to blindness. This ultra-rare condition affects fewer than 2,000 people in the US and most severely impacts children.

Ryplazim® is the first treatment used to treat patients with this condition. In addition to orphan designation, the treatment received fast track designation, priority review and a rare pediatric disease priority review voucher from the FDA. Kedrion Biopharma—which specializes in the development, production, and distribution of plasma-derived therapeutic products for treating serious diseases—is currently ramping up Ryplazim production capacity to meet patient demand.

Kedrion Biopharma has made it their core mission to improve the lives of people with rare and serious diseases. Their newly developed and released treatment has received strong support from the patient community and will address an unmet medical need for individuals affected by this rare genetic disease.

NORD is proud to present Kedrion Biopharma with a 2022 Industry Innovation Award for bringing this important new treatment to patients.

KEDRION
BIOPHARMA

2022 INDUSTRY INNOVATION AWARD HONOREE

ON TARGET LABORATORIES, INC. FOR CYTALUX™

Ovarian cancer is the number one cause of gynecologic cancer death in the United States. Cytoreductive surgery is a well-established treatment for ovarian cancer but is not a foolproof procedure for detecting all tumors or disease.

Cytalux™ serves as a new tool for surgeons to improve the ability to locate additional ovarian cancerous tissue that is normally difficult to detect during surgery. By supplementing current methods of detecting ovarian cancer during surgery, Cytalux offers health care professionals an additional imaging approach for patients with ovarian cancer. For enabling the detection of more cancer during surgery, the treatment was designated first-in-class by the FDA for ovarian cancer.

On Target Laboratories, Inc. is fighting for a brighter future for those combatting cancer. Now with Cytalux on the market, the goal is to establish the groundwork that can be used to explore the use of this technology in identifying and treating other cancers.

NORD is honored to present On Target Laboratories, Inc. with a 2022 Industry Innovation Award for bringing this important new treatment to patients.

ON TARGET LABORATORIES

2022 INDUSTRY INNOVATION AWARD HONOREE

PHARMAESSENTIA CORPORATION FOR BESREMI®

Polycythemia vera is a rare blood disease in adults that causes the overproduction of red blood cells, which increases the chance of blood clots and can lead to heart attack or stroke. This rare disorder affects approximately 6,200 Americans each year.

Besremi® is the first FDA-approved medication for polycythemia vera that patients can take regardless of their treatment history, and as such, there has been a lot of positive support from the patient community. The treatment is noted as first in class by the FDA and received orphan drug designation for this indication.

Founded in 2003, PharmaEssentia is a rapidly growing innovator, focused on redefining possibilities in the treatment of myeloproliferative neoplasms (MPNs). PharmaEssentia has shown a strong commitment to setting up patient support services and medical information channels to help both patients and healthcare providers navigate the choice to prescribe, enable access, and support patients through their rare journey.

NORD is thrilled to present PharmaEssentia Corporation with a 2022 Industry Innovation Award for bringing this important new treatment to patients.

PharmaEssentia

Better Science . Better Lives.

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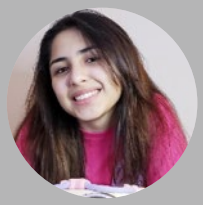

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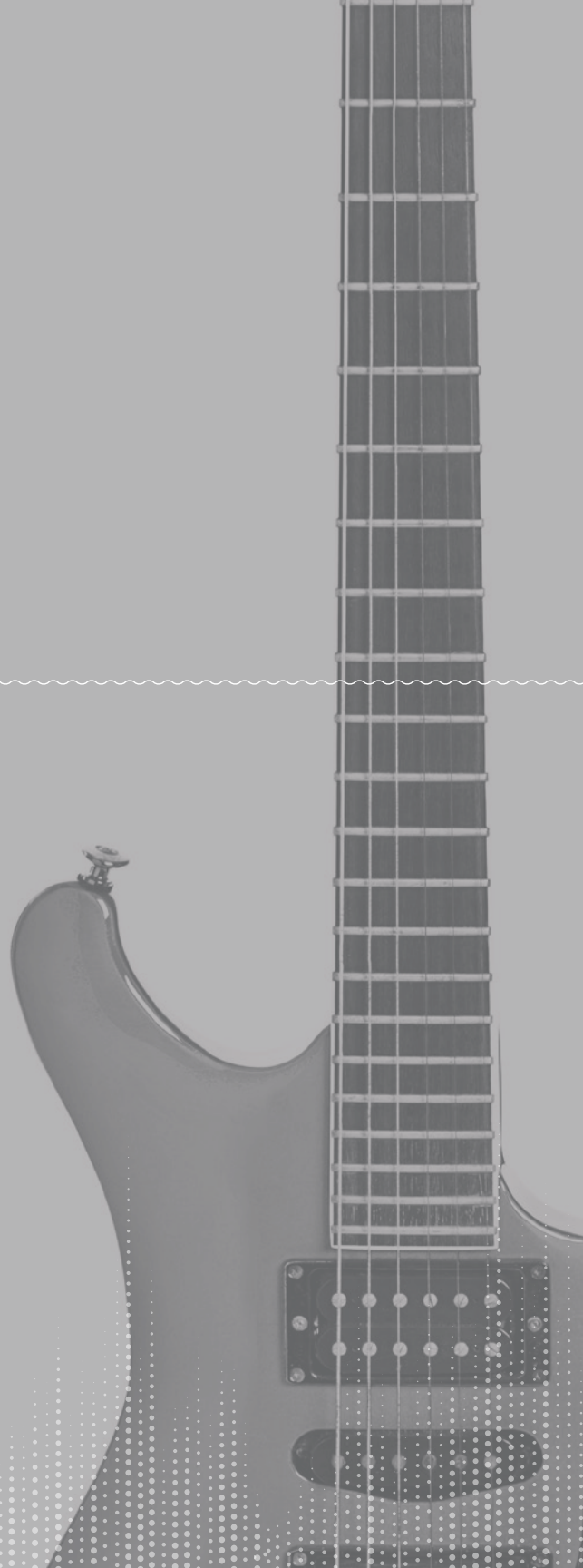

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TODAY WE CELEBRATE

compassion, creativity and courageous science – three elements that, together, can lead to profound change.

Congratulations to our fellow 2022 Rare Impact Award winners and thank you National Organization for Rare Disorders for the many ways you improve our world.

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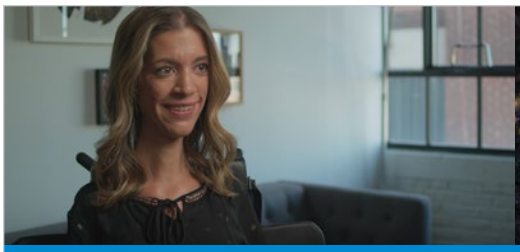

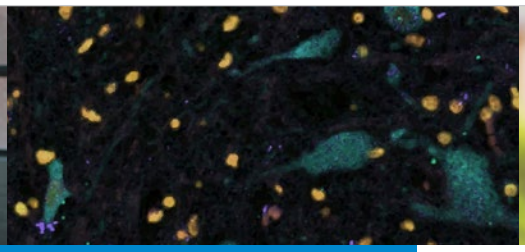

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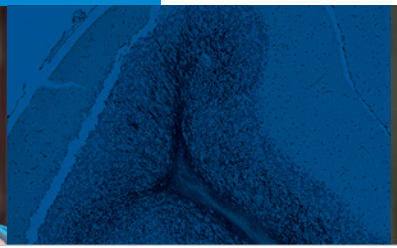

At Travere Therapeutics, we are in rare for life.

We come together every day to help patients, families and caregivers of all backgrounds as they navigate life with a rare disease. On this path, we know the need for treatment options is urgent — that is why our global team works with the rare disease community to identify, develop and deliver life-changing therapies. In pursuit of this mission, we continuously seek to understand the diverse perspectives of rare patients and to courageously forge new paths to make a difference in their lives and provide hope — today and tomorrow.

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Congratulations to our honorees!

The National Organization for Rare Disorders (NORD®) would like to congratulate and thank all of our Rare Impact Award® honorees, past and present, for their commitment to improving the lives of those impacted by rare disease.

Thank you for joining us this evening. It is a joy to reunite in-person with our community!

NORD®

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Together we are strong.®

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Ionis understands that what most may consider an ordinary moment is sometimes truly extraordinary, especially for those who are battling a devastating illness. This is why Ionis remains steadfast in its pursuit of delivering future creating RNA-targeted therapies to the patients who depend on them.

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At Mallinckrodt, our focus is to improve the lives of patients worldwide. Making a difference is what drives us every day as we work to develop innovative therapies and cutting-edge technologies for patients with severe and critical conditions.

We see challenges as opportunities to change lives.
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Congratulations to all the 2022 Rare Impact Award Honorees.

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By providing peer support to pediatric patients and their families during life-changing and critical transitions, we help shape a healthier & more positive experience that encourages compliance with treatments as well as family unity.

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NORD®

Rare Summit

RARE DISEASES & ORPHAN PRODUCTS

BREAKTHROUGH SUMMIT®

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The **2022 NORD® Rare Diseases & Orphan Products Breakthrough Summit®** will be held on **October 17 and 18 in Washington, DC**. The NORD Summit offers an unparalleled opportunity to learn from all rare disease stakeholders – medical and academic experts, regulators and policymakers, innovators, patients, caregivers, and industry leaders.

Topics of discussion often include: public policy issues in rare disease advocacy, advancements in rare disease research, and best practices patient advocacy organizations can adopt to provide optimal support to rare communities.

NORD®

To learn more and stay up-to-date on Summit happenings, visit: **nordsummit.org.**

NORD® was founded almost 40 years ago by a group of parents advocating for their children, who were struggling with rare diseases. From day one, we have fought to improve the lives of over 25 million Americans impacted by rare diseases and continue that important work today and every day.

- Helping those with rare diseases access resources and financial assistance to improve their quality of life.
- Providing learning opportunities through conferences, continuing medical education, and webinars for clinicians.
- Raising awareness for the issues facing the rare community and working together with you to make a difference.

NORD®

DONATE TO NORD!

NORD is leading the fight to improve the lives of those impacted by rare disease. Scan the QR code or visit **livingrare.org/donate** to support NORD today!

The National Organization for Rare Disorders (NORD®) is leading the fight to improve the lives of patients with rare diseases. We do this by supporting patients and organizations, accelerating research, providing education, disseminating information, raising public awareness and driving public policy.

ALONE TOGETHER
WE ARE RARE. WE ARE STRONG®

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