Celebrate & honor

RARE. STRONG. TOGETHER.
40 YEARS
NORD®

THE RARE IMPACT AWARDS®

RARE. STRONG. TOGETHER. 40 YEARS NORD®

Welcome to

THE RARE IMPACT AWARDS®

NORD®

Program for the Evening

MUSICAL PERFORMANCE
CeeCee

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

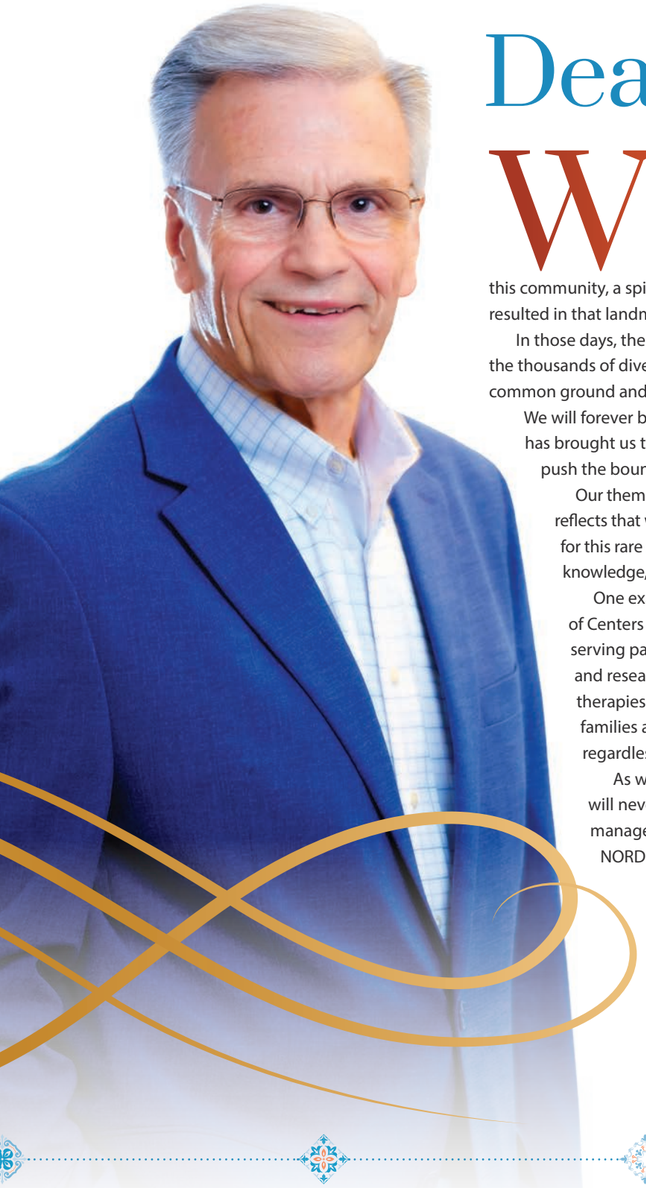
WELCOME
Peter Alexander, Emcee for the Evening

PRESENTATION OF AWARDS

DINNER AND
MUSICAL PERFORMANCE
Christian Guardino

PRESENTATION OF AWARDS

CLOSING



Dear Friends,

Welcome to NORD's 2023 Rare Impact Awards to honor and celebrate individuals and organizations for their accomplishments on behalf of the rare community. Our event has special significance this year because we are also celebrating the 40th anniversary of NORD and the Orphan Drug Act.

The stories you will hear this evening illustrate the remarkable spirit of this community, a spirit that first became evident in the events of the late 1970s and early '80s that resulted in that landmark legislation and the founding of NORD.

In those days, there was no Internet, no social media and no easy way for people affected by the thousands of diverse rare diseases to connect. Even so, the early advocates recognized their common ground and the importance of coming together to drive progress for all.

We will forever be grateful to those rare disease pioneers for the movement they began that has brought us to where we are today. We pledge to carry this cause forward and continue to push the boundaries of scientific inquiry, public policy and healthcare services.

Our theme for this special anniversary year — "Reimagining the Future of Rare Disease" — reflects that we are at a pivotal moment in time. Opportunities exist today to drive progress for this rare community in ways that were never before possible as a result of increased knowledge, public awareness and the incredible scientific advances of recent years.

One example of how NORD is reimagining the future of rare disease is our network of Centers of Excellence with its focus on sharing best practices and medical knowledge; serving patients closer to where they live; equitably improving access to diagnosis, care and research; and facilitating collaboration to promote development of innovative new therapies. Once the full capabilities of this amazing network are realized, patients and families affected by rare diseases will have access to the latest and best medical care, regardless of where they live.

As we explore new frontiers, we also want to assure our community that NORD will never betray its origins as an organization that is patient-focused, patient-managed and patient-governed. The reputation for integrity that has characterized NORD from its earliest years remains a guiding force today.

This evening, we celebrate our honorees for their outstanding contributions to the rare cause. We also pause to express gratitude to those who had the wisdom and foresight to come together to represent the "orphans" of the medical world.

PETER L. SALTONSTALL
PRESIDENT & CEO

EMCEE FOR THE EVENING

Peter Alexander

Emmy Award-winning journalist Peter Alexander is serving as host for the 2023 Rare Impact Awards.

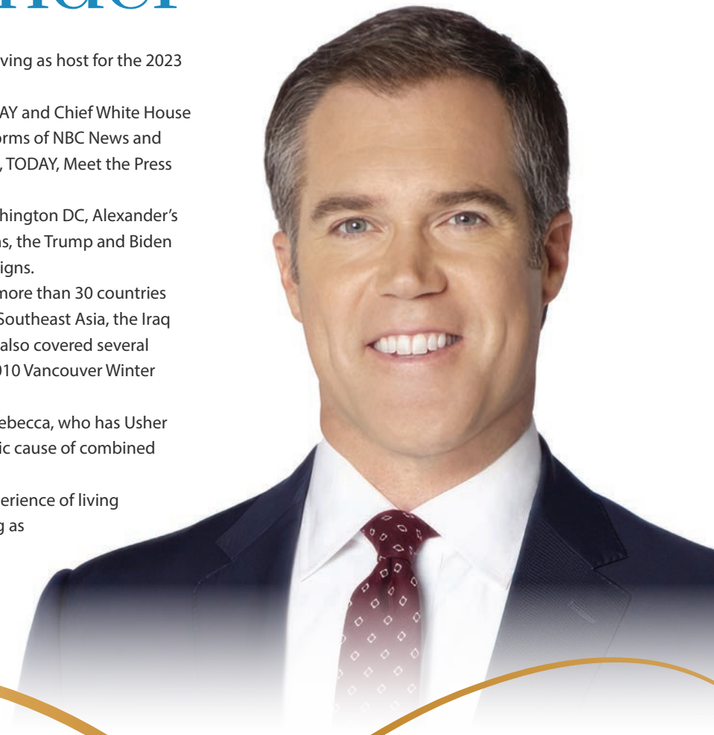
Peter Alexander is the co-anchor of Saturday TODAY and Chief White House Correspondent for NBC News. He reports for all platforms of NBC News and MSNBC, including NBC Nightly News with Lester Holt, TODAY, Meet the Press and NBC News Now.

During more than a decade covering the White House and Washington DC, Alexander's extensive reporting has included four Supreme Court confirmations, the Trump and Biden administrations' pandemic response, and three presidential campaigns.

Since joining NBC News in 2004, Alexander has reported from more than 30 countries covering numerous international stories, including the tsunami in Southeast Asia, the Iraq War and the death of Osama bin Laden in Pakistan. Alexander has also covered several Olympic Games, including the 2008 Beijing Summer Games, the 2010 Vancouver Winter Games and the 2016 Summer Games in Rio.

He regularly reports on the deeply personal story of his sister Rebecca, who has Usher syndrome, type III, a rare genetic disorder that is the leading genetic cause of combined deafness and blindness in the U.S. and around the world.

NORD is grateful to Peter for helping to shine a light on the experience of living with a rare disease and we are thrilled to welcome him this evening as our host for the 2023 Rare Impact Awards and NORD's 40th Anniversary Celebration!



CeeCee

Catherine "CeeCee" Castro is a singer-songwriter influenced by Mariah Carey, Whitney Houston and Selena, whose style is a unique blend of R&B and Soul with Latin influences. She has performed the national anthem at the famous Madison Square Garden, sung for Coca-Cola Latin America, and worked with Grammy Award-winning producers and artists. Lately, she has been performing with her band across the New York Tribeca area promoting her song "Sin Ti."

CeeCee is also a cancer survivor; at the young age of thirteen, she was diagnosed with acute lymphocytic leukemia. Performing for NORD is extremely special and dear to her heart because of this experience.

—

You can follow CeeCee on social and media listen to her music on:

Instagram: @Ceecee.music
Facebook: @CeeCeeSings
Youtube: @CeeCee8859

Christian Guardino

Christian Guardino is a 23-year-old singer-songwriter from Long Island, New York. Christian performs all over the country wowing crowds with his powerful and soulful vocals, as well as his miraculous story of overcoming blindness at thirteen years old after receiving a then, experimental, gene therapy.

Christian is no stranger to the spotlight, in fact, you may have seen him during his impressive run on American idol's 20th season, where he continually impressed judges, Lionel Richie, Katy Perry and Luke Bryan and ultimately finished in the Top 7 and performed a stunning duet of "Smile" with Michael Bublé in the season finale.

Christian also received Howie Mandel's Golden Buzzer on America's Got Talent Season 16, where he finished in the semi-finals. He won Amateur Night at The Apollo as well as appeared on Fox's "Showtime at The Apollo" with host Steve Harvey.

Since American Idol, Christian has been focused on writing and recording music and is soon to release his first original album. He has been performing in his own headliner show alongside his team of producers and band members and continues to touch lives everywhere he goes with his music, his humble nature and inspirational story.

Christian's dream is to become a global performer and to leave a lasting mark on music for generations to come and to inspire the hearts of all that experience his shows.

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You can keep up with Christian on his social media and on all major music streaming platforms:

Facebook: @christianguardinoofficial
Instagram: @christianguardino
YouTube: @christianguardinoofficial

2023 LIFETIME ACHIEVEMENT AWARD HONOREE

Abbey S. Meyers

Abbey Meyers is the "mother" of a movement. A movement that in 1983 became the National Organization for Rare Disorders (NORD), the first national nonprofit to represent all individuals and families affected by rare disease.

Like so many of us in the rare disease community, Abbey's crusade started as a determined parent for whom "no" has never been an acceptable answer, especially when searching for treatment for her son's Tourette's syndrome in the 1970's. She assumed she wasn't the only parent waging such a battle for their child.

Stronger together, Abbey built a coalition of caregivers and support groups. Their cause became the Orphan Drug Act, the world's first law incentivizing drug development for rare diseases. Their coalition became NORD. These two milestones, forever intertwined, marked a turning point for rare diseases.

Abbey became NORD's first president, and so began NORD's 40-year history of patient-centered stewardship and innovation to advance rare disease care, treatment and research. She served as NORD's president until 2008. Abbey also served as Honorary President of Rare Diseases Europe (EURORDIS), formerly known as the European Organisation for Rare Diseases.

Throughout her career, Abbey has held numerous roles focused on rare diseases. She served as the consumer representative on the National Commission on Orphan Diseases, the NIH Human Gene Therapy Subcommittee, the NIH Recombinant DNA Advisory Committee, the FDA Biological Modifiers Committee and the HHS National Human Research Protections Advisory Committee.

Her tireless advocacy has been deservingly recognized with the FDA Commissioner's Special Citation for Exceptional Dedication and Advancements on Behalf of All People Afflicted with Rare Disorders and the Department of Health and Human Services' Public Health Service Award for Exceptional Achievement in Orphan Drug Development. She also holds an Honorary Doctorate from Alfred University in New York.

Abbey has authored numerous articles and papers on rare diseases and has been a frequent speaker. She published her memoir, Orphan Drugs: A Global Crusade, in 2016. We are forever grateful for Abbey's years of dedication to leading and serving the rare disease patient community.

NORD is honored to present Abbey Meyers with the 2023 Lifetime Achievement Award.

2023 ABBEY S. MEYERS LEADERSHIP AWARD HONOREE

Alliance to Cure Cavernous Malformation

What started at Connie Lee's kitchen table in 2002 has grown into an essential organization that raises awareness, educates the public, drives research, and provides support for those affected by cavernous malformations.

Lee's personal journey with cavernous malformation began in 2000 when her infant daughter was diagnosed with the condition. Frustrated by the lack of support and resources for this rare community, Lee launched the Alliance to Cure Cavernous Malformation (ACCM) from her home, establishing a website that to this day serves as a hub to connect patients, families and advocates.

ACCM is leading efforts to advance diversity, equity and inclusion — dedicating 20-25% of its annual budget since 2017 to DEI and working to reach communities that have traditionally been underrepresented in its work.

The Baca Family Historical Project (BFHP) is one example of this effort. BFHP launched in 2017 as a collaborative campaign with the University of New Mexico that connects Hispanic families at risk for a founder mutation to their genealogy and provides free genetic testing and individual patient education to increase and encourage health self-advocacy.

Another example is ACCM's Breaking Barriers for Black Health Empowerment, an initiative that addresses the diagnostic and treatment disparities that impact Black patients with cerebral cavernous malformations (CCMs). By listening and understanding their experiences, ACCM works to develop ways to improve self-advocacy, care, and research opportunities.

The nonprofit also has driven innovation in research and clinical care through its many efforts, including the early establishment of a biobank and clinical database, in existence since 2006, and the development of a network of CCM Centers of Excellence to establish a research infrastructure for large multi-center clinical trials. Also launched was a 15-year consortium project — known as the Brain Vascular Malformations Consortium — through NIH's Rare Disease Clinical Research Network that allows researchers to document the natural history and investigate three rare diseases that cause brain vascular malformations.

While focusing its grant money to find a cure by 2030, ACCM is striving to meet its goal of having treatments available by 2025.

NORD is proud to bestow the Abbey S. Meyers Leadership Award to the Alliance to Cure Cavernous Malformation.

2023 RARE IMPACT AWARD HONOREE

Jaime Herrera Beutler

At 20 weeks pregnant with her first baby, then-Congresswoman Jaime Herrera Beutler and her husband received devastating news.

Doctors told them their baby had no chance of surviving due to a rare condition known as bilateral renal agenesis. Also known as Potter's Syndrome, the condition is caused when both kidneys are absent from the developing baby.

As only the ninth lawmaker in history to give birth while serving in Congress, the former congresswoman from Washington state faced the added challenge of dealing with a personal crisis in the public eye.

But Herrera Beutler is known for beating the odds. And this proved to be no exception.

Nine years later, her daughter Abigail is alive and well. She is not only the first child to survive this fatal condition but also, more importantly, no longer the only survivor. Her story offers hope for mothers who are facing the same challenge.

Herrera Beutler is committed to turning that hope into action.

Realizing that there is less research, development and innovation in the rare space, Herrera Beutler has used her platform — both in Congress and now as a private citizen — to influence and help direct where funding can go.

For example, she voiced support for the Renal Anhydramnios Fetal Therapy (RAFT) clinical trials at the Johns Hopkins Children's Center, which tests an experimental treatment for mothers and their babies affected by early pregnancy renal anhydramnios (EPRA).

The federal Advancing Care for Exceptional (ACE) Kids Act became law in 2019 — and Herrera Beutler was one of the driving forces behind it. This law allows children with medically complex conditions (CMC) who rely on Medicaid to cross state lines if they need to receive care from a specialty doctor who is not located in their home state.

Herrera Beutler continues to dedicate her time to bridging the gaps between policies and laws around healthcare and the families they impact.

In January 2023, Herrera Beutler joined the Children's Hospital Association as a strategic advisor, providing insight on key issues such as Medicaid investments, youth behavioral health crisis, the pediatric workforce and the care of children with rare medical conditions.

Because of her commitment to being a voice for those who are often overlooked, NORD is honored to present Jaime Herrera Beutler with a 2023 Rare Impact Award.

2023 RARE IMPACT AWARD HONOREE

Ada Hamosh, MD, MPH

Coming from a family of medical professionals — her mother was a biochemist, her father was a pulmonologist — Dr. Ada Hamosh always knew that she would pursue a career in medicine.

But she ended up taking a medical path less traveled — to the benefit of those with rare conditions.

A clinical geneticist, pediatrician, professor and mentor, Hamosh has committed her life's work to advocating for families in the rare community by researching genotype-phenotype correlations.

Dr. Hamosh is the Dr. Frank V. Sutland Professor of Pediatric Genetics and the Clinical Director of the McKusick-Nathans Department of Genetic Medicine at Johns Hopkins University School of Medicine.

She also serves as the Scientific Director of Online Mendelian Inheritance in Man (OMIM), a free, online catalog that provides information on all known Mendelian disorders. Under her direction, the website is updated daily with new information and attracts 1.2 million page views per week.

In collaboration with her colleagues, Dr. Hamosh co-developed PhenoDB, a web-based tool for the collection, storage, and analysis of standardized phenotype and genotype data for use in the Centers for Mendelian Genomic project, and GeneMatcher, a website that connects clinicians and researchers who share an interest in the same gene.

Throughout her career, Dr. Hamosh has authored more than 130 papers that discuss the genotype-phenotype correlations in cystic fibrosis, natural history studies in NKH (Nonketotic Hyperglycinemia), novel disease gene discoveries, and new treatments for rare Mendelian disorders.

She is an active member of the ClinGen Project, where she sits on panels and committees and provides her expertise on gene curation, disease naming, lumping and splitting, and syndromic disorders.

As a member of the Global Alliance for Genomics and Health (GA4GH), she collaborates with a diverse worldwide team to make high-level decisions about the organization's directions, values, and deliverables.

Dr. Hamosh became President of the Human Genome Organization (HUGO) in March 2023 and is excited to share the tools, resources and other benefits of the organization with people worldwide, particularly in countries that need them most.

With all of her contributions to making resources accessible and being a mentor and positive influence for professionals in medicine, NORD is proud to present Dr. Ada Hamosh with a 2023 Rare Impact Award.

2023 RARE IMPACT AWARD HONOREE

Shannon Killebrew

Shannon Killebrew is is a patient and the founder of Shannon's Hope for House Calls and Home Medical Care, a patient advocacy organization that provides support and resources to those that are homebound and bedbound. Like many who experience rare diseases, Shannon Killebrew knows firsthand what it's like to feel terrified and alone.

In 2006, she began feeling severe pain and within six weeks after experiencing her first symptoms, she lost her ability to walk.

Killebrew was ultimately diagnosed with a rare condition called Reflex Sympathetic Dystrophy (RSD). Nicknamed "the suicide disease," RSD is a chronic condition that typically brings severe burning pain and, in some patients, can cause extreme sensitivity to touch.

As the disease progressed, it took away Killebrew's ability to work and eventually her ability to even ride in a vehicle, which, in turn, made it impossible for her to travel to medical appointments.

But it didn't take away her will — nor did it stop her from wanting to make a difference and to advocate for those who need support.

Despite being unable to leave her home due to her condition, Killebrew founded Shannon's Hope for House Calls and Home Medical Care. The mission of Shannon's Hope is to open the hearts, ears and minds of the medical community to ensure that equality of care becomes the norm rather than a rarity.

Using social channels, she connects homebound patients who need care with physicians, in-home medical care providers, and other caregivers. Since starting Shannon's Hope, Killebrew says she has met some of the bravest rare disease warriors, medical professionals and caring family members of loved ones who are battling with a rare disease — all of whom have inspired her to continue her work.

NORD is honored to present Shannon Killebrew with a 2023 Rare Impact Award.

2023 RARE IMPACT AWARD HONOREE

Phillip Maderia

Known as the "Father of Running for Rare," Phillip Maderia brought three seemingly unrelated worlds together to make a significant impact on the rare disease community.

First, while working as a young engineer for Genzyme — a biomanufacturing company in Cambridge, Mass. — he gave tours to patients being treated by Genzyme programs for Gaucher, Fabry and Pompe diseases. During these tours, he experienced the magic of working for a company that was making such a significant difference in the lives of these patients. He connected with patients on a personal level and was motivated by their stories.

In 2007, after running his first Boston Marathon, the sense of accomplishment he felt after completing the grueling race inspired him to do more. When he decided to run the marathon the following year, he reached out to his colleagues at patient advocacy and suggested using the marathon as a platform to connect employees and raise money and awareness for NORD.

In 2008, he created the Running for Rare program and began selecting passionate runners to raise awareness and fundraise to support patients with rare diseases as members of the Boston Marathon team.

Between 2008 and 2010, more of Maderia's colleagues were encouraged by his commitment to support the rare community and to run the Boston Marathon. They too wanted to join and find ways to support the cause.

The program evolved in 2010 when it began to pair members of the rare disease community directly with the running team members. Through this program, lifelong relationships have formed between those impacted and those with a passion for helping.

In 2016, the Running for Rare program came under NORD's leadership to contribute to the organization's mission to drive public policy, accelerate research and improve care for people living with rare diseases, including those who are undiagnosed.

Because of this program, there has been an increased quality of life for those impacted by rare diseases as well as those who are simply looking to be physically active.

NORD is proud to present Phillip Maderia with a 2023 Rare Impact Award.

To join volunteers like Phillip in making an impact on those living with rare diseases, visit runningforrare.org.

2023 RARE IMPACT AWARD HONOREE

Bernie Williams

Baseball fans know Bernie Williams as a stellar centerfielder who helped lead the New York Yankees to four World Series championships. But those in the rare community also know him as a voice, advocate, and champion for patients and families who are impacted by idiopathic pulmonary fibrosis (IPF) and other forms of interstitial lung disease (ILD). Williams was first introduced to rare diseases when his father was diagnosed with IPF and unfortunately passed away years later in 2001. Williams dealt with his father's passing the best he could, but it wasn't until he was approached by Boehringer Ingelheim Pharmaceuticals in 2017 that he was really able to grieve — and heal.

Partnering with the company on its Breathless campaign, Williams uses his platform to raise awareness and encourage those who may have IPF to seek early diagnosis and care. When the COVID-19 pandemic hit in 2020, Williams and Boehringer Ingelheim — like much of the world — had to find creative ways to continue engaging with the community. With his undergraduate degree in jazz performance and love for playing the guitar, the campaign evolved to use music as a way to reach people and help them cope with the symptoms.

In 2021, Williams teamed up with singer-songwriter and "American Idol" winner Jordin Sparks to perform a tribute to his father and others who are coping with IPF. What made this even more unique was that the song was part of The Breathless Ballad Challenge, a competition to raise awareness for IPF. The winner of the challenge was the son of a mother who died of lung disease.

Most recently in 2022, Tune In To Lung Health became the next chapter in the Breathless story. The program explores how music and breathing may help people affected by IPF and other types of ILD cope with the physical, mental, and emotional burden of living with or caring for someone with the disease through educational resources like breathing and vocal exercises. Williams found this new way of reaching people to be therapeutic and it allowed him to properly grieve the loss of his father.

Amazed by the great strides and progress that has been made thus far, Williams hopes his work in this rare community sends a message to patients and families that they don't have to fight this fight alone.

Bernie William's father surely would be proud — just as NORD is honored to present Bernie Williams with a 2023 Rare Impact Award.

2023 INDUSTRY INNOVATION AWARD HONOREE

bluebird bio for SKYSONA®

Cerebral adrenoleukodystrophy (CALD) is a rare, progressive, neurodegenerative disease that primarily affects young boys. It causes irreversible, devastating neurologic decline, including major functional disabilities such as loss of communication, cortical blindness, total incontinence, wheelchair dependence or complete loss of voluntary movement.

Without treatment, about half of those affected die within five years of symptom onset. This condition occurs as a result of a defective or missing ABCD1 gene located on the X chromosome. That gene controls the production of an enzyme that breaks down fatty acids in the body.

In September 2022, the FDA granted Accelerated Approval to SKYSONA, a gene therapy, to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active CALD. The treatment is designed to add functional copies of the ABCD1 gene into a patient's hematopoietic stem cells. Early identification of adrenoleukodystrophy is possible with newborn screening.

Prior to the approval of SKYSONA, effective treatment options were limited to blood stem cell transplantation utilizing cells from a genetically matched donor.

SKYSONA is the first approved therapy shown to slow the progression of neurologic dysfunction in boys with this devastating disease.

In recognition of the hope this milestone brings to a community struggling with a terribly devastating disease, NORD is presenting a 2023 Industry Innovation Award to bluebird bio.

bluebirdbio

CSL Behring and uniQure for Hemgenix®

Hemophilia B is a genetic bleeding disorder resulting from missing or insufficient levels of a blood protein called factor IX, which is needed to for blood clotting, the process by which blood seals a wound to stop bleeding and promote healing. Symptoms of hemophilia B can include prolonged or heavy bleeding after injury, surgery or a dental procedure. In severe cases, bleeding episodes can occur spontaneously without a clear cause.

Prolonged bleeding episodes can lead to serious complications, such as bleeding into joints, muscles or internal organs, including the brain. Approximately 15% of the individuals affected by hemophilia have this particular type (hemophilia B).

Hemophilia B is caused by pathogenic variants in the F9 gene on the X chromosome. It is expressed mostly in males but some females who carry a pathogenic variant in F9 may have mild or, rarely, severe symptoms of bleeding.

In November 2022, the FDA approved the first gene therapy for hemophilia B. It is a product known as Hemgenix® and it's a one-time gene therapy given as a single dose by IV infusion.

In announcing the approval, Dr. Peter Marks of FDA's Center for Biologics Evaluation and Research said that it "provides a new treatment option for hemophilia B and represents important progress in the development of innovative therapies for those experiencing a high burden of disease associated with this form of hemophilia."

The multi-year clinical development of Hemgenix® was led by uniQure and sponsorship of the clinical trials transitioned to CSL Behring after it licensed global rights to commercialize the treatment.

Hemgenix® was awarded priority review and breakthrough therapy status, in addition to orphan drug designation. NORD is presenting a 2023 Industry Innovation Award to CSL Behring and uniQure for this important new treatment option for those affected by hemophilia B.

CSL Behring uniQure

Biotherapies for Life®

Mallinckrodt Pharmaceuticals for Terlivaz®

Hepatorenal syndrome (HRS) is a form of impaired kidney function that occurs in individuals with advanced liver disease. Symptoms may include fatigue, abdominal pain and a general feeling of ill health.

There are two distinct types. One type progresses quickly and may lead to kidney failure within days. The other type progresses more slowly, over weeks or months, and the symptoms are less severe.

Those affected by HRS do not have any identifiable cause of kidney dysfunction and the kidneys are not structurally damaged. Therefore, diagnosing and treating HRS can be challenging, and the condition can result in rapid reduction in kidney function, an acute and life-threatening condition requiring hospitalization.

HRS involving rapid reduction in kidney function is estimated to affect between 30,000 and 40,000 Americans annually. If left untreated, it has a median survival time of approximately two weeks and greater than 80% mortality within three months.

In September 2022, FDA approved Mallinckrodt Pharmaceuticals' Terlivaz® as the first product to improve kidney function in adults with HRS who experience rapid reduction in kidney function. In addition to orphan drug designation, Terlivaz® was awarded priority review and fast track status.

To acknowledge development of this important first treatment for a potentially life-threatening condition, NORD is presenting a 2023 Industry Innovation Award to Mallinckrodt Pharmaceuticals.

Mallinckrodt Pharmaceuticals

Marinus Pharmaceuticals for Ztalmy®

CDKL5 deficiency disorder (CDD) is a rare developmental and epileptic encephalopathy caused by pathogenic variants in the CDKL5 gene. This is a gene that provides instructions for making a protein that is essential for brain and neuron development.

The hallmarks of CDD are the onset of seizures at a very early age — typically at about three months but sometimes as early as the first week of life — and severe neurodevelopmental delay impacting cognitive, motor, speech and visual function. The seizures tend to be severe and difficult to control with medication. For CDD patients and their families, seizure control is a lifelong concern that must be managed alongside a constellation of other symptoms and comorbidities.

In March 2022, the FDA approved Ztalmy® (ganaxolone) oral suspension CV for the treatment of seizures associated with CDD in patients two years of age and older. This is the first treatment specifically for CDD-related seizures. This achievement was made possible thanks to the collaboration of a dedicated network of CDD patients, caregivers, advocates, investigators, and the FDA.

In addition to orphan designation, Ztalmy® received priority review status as well as Rare Pediatric Disease Designation. Upon approval, Marinus was awarded a Rare Pediatric Disease Priority Review Voucher. Marinus Pharmaceuticals is committed to continuing to advance innovation for individuals affected by rare epilepsies and seizure disorders.

NORD is presenting a 2023 Industry Innovation Award to Marinus Pharmaceuticals in recognition of the company's development of this important first treatment for CDD patients and their families.

MARINUS

Novartis for Vijoice®

PIK3CA-Related Overgrowth Spectrum (PROS) is a group of genetic disorders leading to overgrowth of various body parts and blood vessel abnormalities due to mutations in the PIK3CA gene. This gene is involved in making a protein that helps regulate cell growth, division and survival. The spectrum includes a broad array of disorders, with some overlap of symptoms.

Genetic mutations that cause these disorders are not passed down from parent to child but, rather, result from changes to genes during embryonic development. Symptoms associated with these disorders can be present at birth or appear later in early childhood. Overgrowth may stop in childhood or continue into adulthood.

Since PIK3CA-related mutations in these disorders are not present in all cells, only certain areas of the body are overgrown, ranging from isolated digits to whole limbs, the torso or the brain. Different tissues may be involved individually or in combination, such as fat, muscle, bone, nerve, brain and blood vessels.

Conditions associated with PROS include CLOVES syndrome, FAVA (fibroadipose vascular anomaly), Klippel-Trenaunay syndrome and several others.

In April 2022, FDA granted Accelerated Approval to Vijoice® for the treatment of adults and pediatric patients two years of age and older with severe manifestations of PROS who require systemic therapy. Vijoice® is the first FDA-approved treatment for PROS, which affects an estimated 14 people per million.

NORD is presenting an Industry Innovation Award to Novartis in recognition of the company's development of the first approved treatment for PROS.

NOVARTIS

Sanofi for Xenpozyme™

Acid sphingomyelinase deficiency (ASMD) is a rare progressive genetic disorder that results from a deficiency of an enzyme required to break down a fatty substance called sphingomyelin that accumulates in various tissues of the body. The disorder is highly variable and the age of onset, symptoms and severity can vary dramatically from one person to another.

At the severe end of the ASMD spectrum is a fatal neurodegenerative disorder that presents in infancy (Niemann-Pick disease type A). At the mild end, affected individuals have no or only minimal neurological symptoms and may survive into adulthood (Niemann-Pick disease type B). There are also intermediate forms of the disorder.

Patients with ASMD have enlarged abdomens that can cause pain, vomiting and feeding difficulties. They also have abnormal liver and blood tests. The most severely affected have profound neurologic symptoms and rarely survive beyond two to three years of age.

There was no approved treatment for ASMD until August 2022, when Xenpozyme™ was approved for pediatric and adult patients with ASMD to treat symptoms that are not related to the central nervous system. Xenpozyme™ is an enzyme replacement therapy that replaces the missing enyme with a genetically engineered form and helps reduce sphingomyelin accumulation in the liver, spleen and lung.

Xenpozyme™ had received fast track, breakthrough therapy and priority review designations, in addition to orphan designation. When it was approved, the sponsor, Sanofi, was awarded a rare pediatric disease priority review voucher.

In honor of this first treatment for a challenging rare disease, NORD is presenting a 2023 Industry Innovation Award to Sanofi.

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The NORD Board of Directors includes representatives of NORD Member Organizations as well as individuals who have demonstrated leadership in serving the rare disease patient community.

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CSL Behring

PURPOSE-DRIVEN TO PROTECT AND IMPROVE THE LIVES OF PEOPLE ACROSS THE GLOBE THROUGH THE PROMISE OF GENE THERAPY

At CSL Behring we are driven by our promise to turn scientific innovation into meaningful and life-saving treatments for patients with rare diseases and previously unmet medical needs around the world. That's why we are proud to support the 2023 NORD Rare Impact Awards and honored to be selected as a Rare Impact Award winner.

Our deepest thanks to uniQure for their partnership and tireless commitment to innovation and gene therapy.

Learn more about our promise to support innovation, like gene therapy, that aims to transform the treatment paradigm for patients living with rare diseases by visiting our website: **CSLBehring.com.**

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Amgen harnesses the best of biology and technology to make people's lives easier, fuller, and longer. We draw upon our deep knowledge of science to push beyond what's known today. With roots in the biotech revolution, we are one of the world's leading independent biotech companies – fighting the toughest diseases and helping millions of people globally. www.amgen.com

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Going to incredible lengths

Horizon is proud to support the 2023 Rare Impact Awards.

Horizon is a global biotechnology company focused on the discovery, development and commercialization of medicines that address critical needs for people impacted by rare, autoimmune and severe inflammatory diseases. Our pipeline is purposeful: We apply scientific expertise and courage to bring clinically meaningful therapies to patients. We believe science and compassion must work together to transform lives.

HORIZON®

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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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Better Health, Brighter Future

Takeda is a global, R&D-driven biopharmaceutical company committed to discovering and delivering life-changing treatments and vaccines that have a lasting impact on society.

Since our founding in 1781 in a market stall in Osaka, Japan, our values endure by putting patient needs first, building trust with society, strengthening our reputation, and developing the business - in that order.

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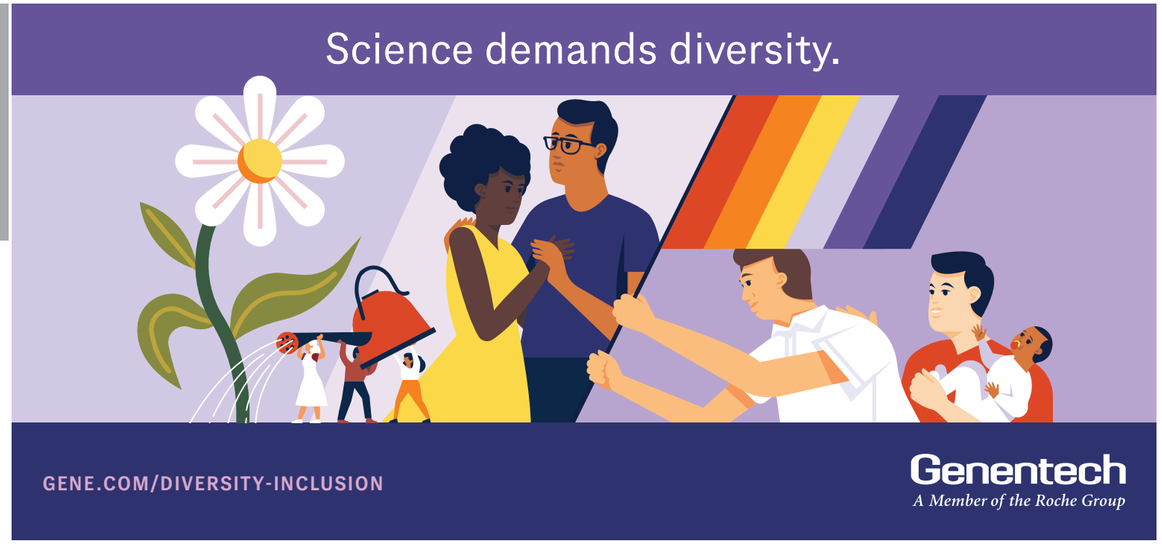

Novartis is pleased to celebrate NORD's 40th Anniversary and the 2023 Living Rare, Living Stronger Forum.

NOVARTIS | Reimagining Medicine

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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TRAVERE THERAPEUTICS

WHERE OTHERS SEE COMPLEXITY, **WE SEE HOPE FOR PATIENTS AND FAMILIES**

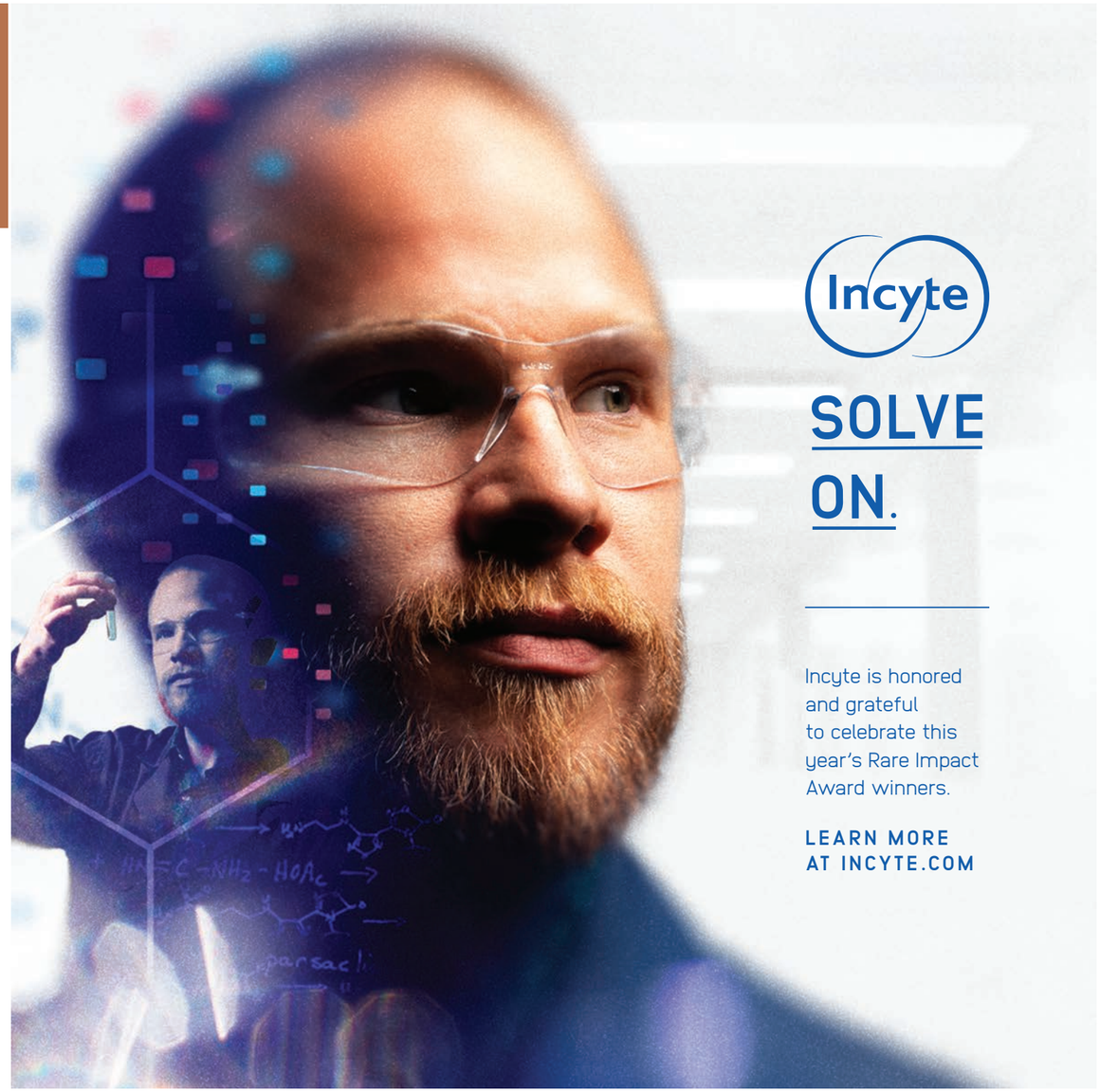
At Mallinckrodt, our focus is to improve the lives of patients worldwide. Making a difference is what drives us every day as we work to develop innovative therapies and cutting-edge technologies for patients with severe and critical conditions.

We see challenges as opportunities to change lives. It is our passion. It is Mallinckrodt.

Learn more at **Mallinckrodt.com.**

Mallinckrodt is honored to receive a Rare Impact Award and congratulates all 2023 Honorees.

Mallinckrodt Pharmaceuticals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Incyte is honored
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Amicus congratulates
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Thank you for
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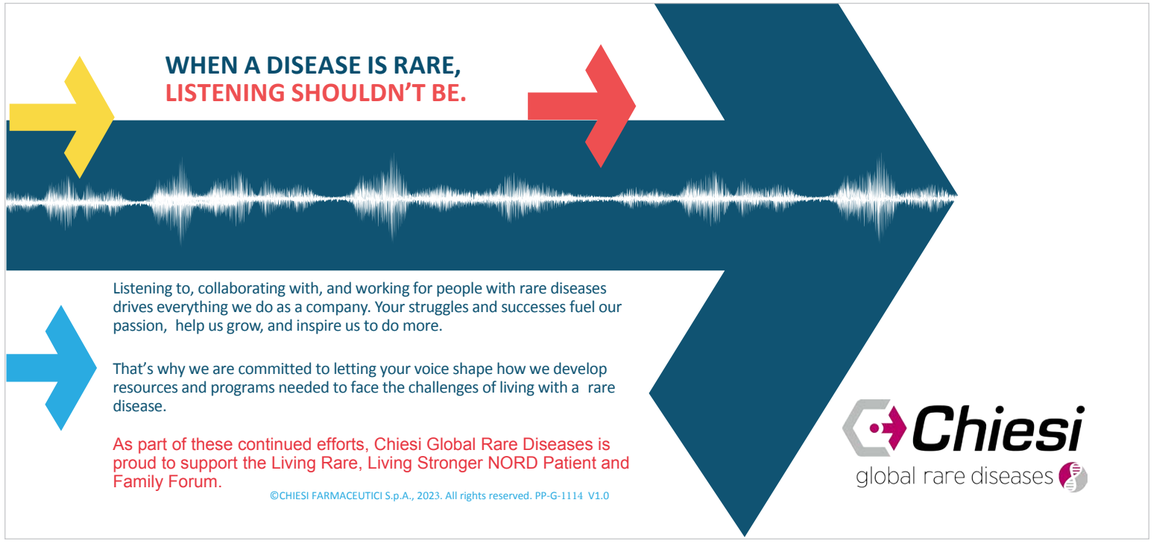
WHEN A DISEASE IS RARE,
LISTENING SHOULDN'T BE.
Listening to, collaborating with, and working for people with rare diseases drives everything we do as a company. Your struggles and successes fuel our passion, help us grow, and inspire us to do more.
That's why we are committed to letting your voice shape how we develop resources and programs needed to face the challenges of living with a rare disease.
As part of these continued efforts, Chiesi Global Rare Diseases is proud to support the Living Rare, Living Stronger NORD Patient and Family Forum.
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global rare diseases

Enzyvant is accelerating transformative medicines urgently needed by the rare disease community.

Enzyvant is a commercial-stage biotechnology company focused on advancing life-altering therapies in immunology and cardiopulmonology.

Equipped with a full range of clinical development, commercialization and manufacturing capabilities, Enzyvant is collaborating with the rare disease community to drive bold science forward and address some of the most pressing medical challenges.

Because in rare diseases, the communities are small, and the need is immense.

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www.enzyvant.com

Regeneron is proud to support the National Organization for Rare Disorders and congratulates the 2023 Rare Impact Award Winners

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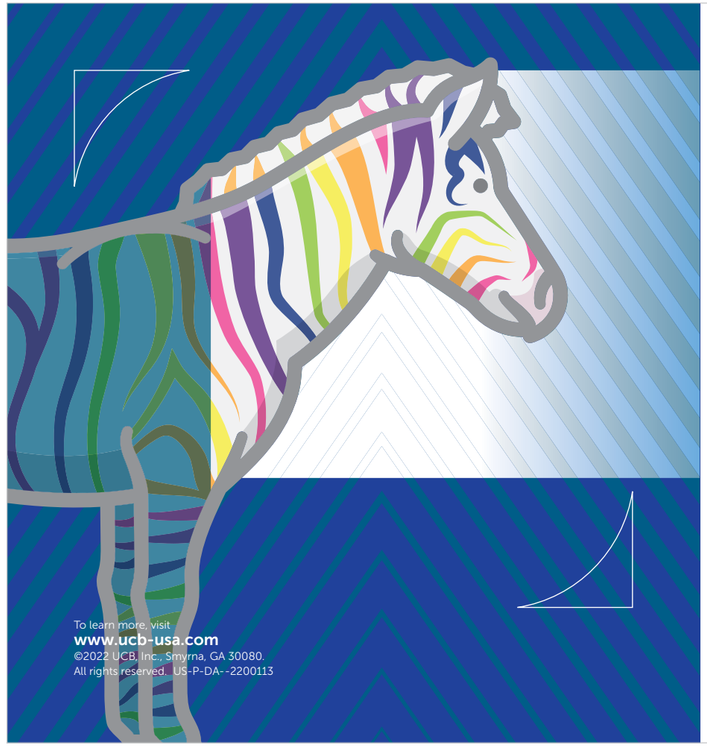

To learn more, visit
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Shining a light on **Rare Disease Communities**

UCB is committed to supporting rare disease communities. We will seek out scientific innovations that have the greatest impact on the lives of people with those diseases.

Inspired by **patients.**
Driven by **science.**

Going beyond every day.™

Ultragenyx is proud to sponsor NORD's 2023 Rare Impact Awards. Congratulations to this year's award honorees for your contributions to the rare disease community. Ultragenyx is grateful to be in the company of parents, protectors, and proud partners in care.

To learn more visit **UltraRareAdvocacy.com**

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At Moderna, we are working to deliver the greatest possible impact to people through mRNA medicines.

Learn more and see what mRNA could do at aboutmRNA.com

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While we are grateful that bluebird's persistence in developing gene therapies is being acknowledged, we humbly share this award with people living with CALD and the families, researchers, and clinicians who partnered with us at every step of the way to give patients hope for more bluebird days.

Congratulations to all the award winners being recognized for their work advancing rare disease treatments for patients.

Please visit **www.bluebirdbio.com** to learn more.

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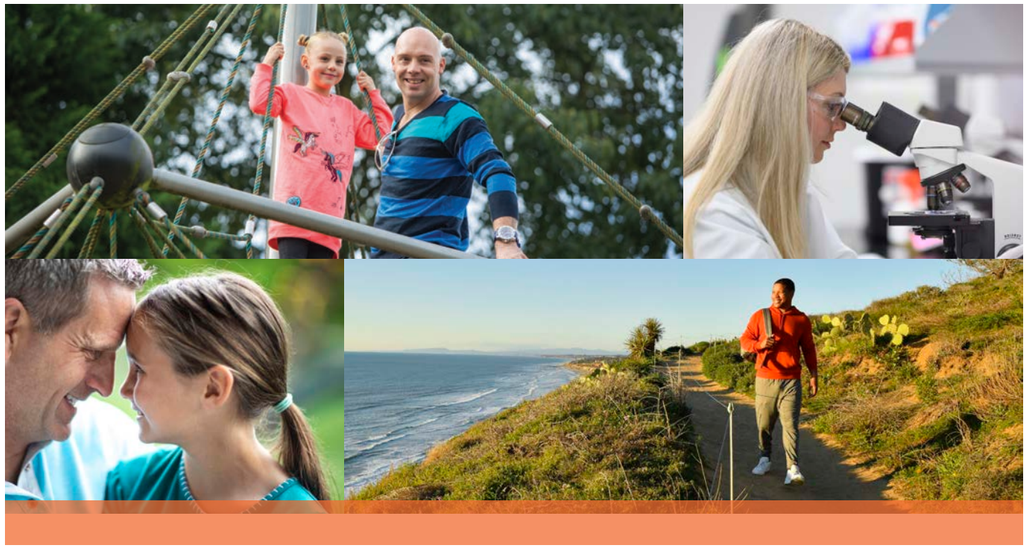
bluebirdbio



Delivering on the promise of gene therapy

As uniQure celebrates its 25th anniversary as a pioneer in gene therapy, we are enormously proud to have developed the world's first approved gene therapy for people living with hemophilia B.

Congratulations to our partner, CSL Behring, and all the other 2023 Rare Impact Award winners.

uniQure

Innovating to transform the lives of patients

uniQure.com

THANK YOU

to **Patients** for your courage and contributions to create a better future for others; to **Caregivers** for being a voice for those whose cannot speak for themselves; to **Member Organizations** and **Patient Advocacy Groups** for banning together to establish a united front, representing the needs of your communities; to **Government Agencies and Leaders** for your dedication to patient safety, research and improving access to care; to **Biopharma and Life Sciences** companies for investing in orphan therapies; to **Researchers** for tackling scientific and medical challenges with resolve; to **Physicians and Healthcare professionals** for your tireless commitment to patient care; to **Donors** for giving generously to our life-saving work; to our **Staff and Volunteers** for bringing your "A" game every day to our mission and cause; to our **Founder, Abbey Meyers** for pioneering the movement that brought us all together.

Thank you for 40 years of progress, collaboration, and innovation.

Alone we are Rare. Together we are Strong.

Together, We Are Reimagining A Brighter Future For People with Rare Diseases

Forty years of patient advocacy has taught us that not only is our collective voice powerful, but we know that every person in the rare community has the power to shape the future and make a lasting impact.

As NORD continues its mission to improve the lives of millions living with a rare disease, we are asking YOU - our community members, partners and colleagues to help us better understand what would make a difference in your rare journey.

Alone We are Rare. Together We Are Strong.

Share & Join The Conversation

We welcome you to join our community discussion to share what changes you would like to see made to help individuals and families impacted by rare disease. Share your story, successes and help us define what everyday progress means to you.

Scan the QR code or visit:
rarediseases.org/nord40

Dinner Menu

SPRING DELIGHTS

First Course

Burrata & Pan-Roasted Zucchini Salad (NF, Veg)
Fennel, Carrot, Mixed Greens, Olive Migas,
Parsley Mint Salsa
Mint Gastrique

Entree

Peach-Glazed Chicken Breast & Seared Mahi-Mahi Duo (GF, NF)
Kale, Smoked Potato Hash, Red Cabbage & Fennel Slaw
Roasted Corn & Mango Salsa
Red Pepper Coulis

Vegan Entree

Chili-Rubbed Broccoli Steak with Cauliflower & Chick Peacake (GF, NF, V)
Braised Leeks, Sweet Pepper & Herb Gremolata
Golden Tomato Emulsion

Dessert

Caramel Macchiato Pavê (GF, NF)
Espresso Blondie, Coffee Mousse, Caramel Cremeaux,
Coffee Caviar

Coffee service by request

We improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.

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Join us as we meet the challenge to create a better future for people living with rare diseases. Scan the QR code or visit **rareimpact.org/donate**.

Alone we are Rare.
Together we are Strong.®

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