Betsy Bogard, Head of Program and Alliance Management, Ensoma

Betsy Bogard works in the rare disease community to enable development of transformative therapies. Her younger brother Jud was born with a rare genetic disease, inspiring her to make a difference for patients and families facing debilitating conditions. She is currently the head of program and alliance management for Ensoma, an emerging gene therapy company. She also chairs the Research Committee for the International FOP Association, a non-profit patient organization for the rare disease fibrodysplasia ossificans progressiva (FOP). Ms. Bogard has over 20 years of experience in biotechnology in areas that include portfolio and alliance management, program leadership, real world evidence, registries, health economics and patient community engagement. She has a master's degree in health policy and management from the Harvard School of Public Health. Ms. Bogard lives in Somerville, Massachusetts with her two sons.

Nicole Boice, Founder and Executive Director, RARE-X

Nicole Boice is a committed champion of families affected by rare disease. She founded Global Genes in 2008 and grew it into one of the most notable rare disease advocacy organizations focused on educating and empowering patients/advocates throughout their journey. Impact includes a membership of over 750 patient organizations, collaborations with over 100 biopharma and academic research partners, numerous support programs and educational events. RARE-X grew out of the need to provide more tools and resources for families needing to become better equipped data stewards and participants in research. A board member of several patient organizations and advisory roles within Biopharma, Nicole remains an important voice for rare disease patients globally.

Bruce Bloom, Chief Collaboration Officer, Healx

In addition to his role at Healx, Dr. Bloom is the founder and former CEO of Cures Within Reach, a leading global non-profit saving lives by unleashing the untapped potential of human approved drugs, devices, diagnostics, and nutraceuticals, testing new uses for them to quickly deliver safe and affordable treatments and cures for diseases that have no currently effective therapy. Bruce envisioned and created CureAccelerator®, the first online redevelopment research collaboration platform, to bring together clinicians, researchers, funders, and industry to create and conduct proof of concept redevelopment clinical trials. Dr. Bloom is an Ashoka Social Entrepreneur Fellow, the Patient Advisory Board Chair for the Institute for Translational Medicine, board member of the Drug Discovery Center and Chair of the Cancer Center Advancement Advisory Boards at the University of Illinois Chicago, member of the Vanderbilt Institute for Clinical and Translational Research External Advisory Board, and is on the Science Advisory Boards of Rediscovery Life Sciences, the Dr. Ralph and Marian Falk Medical Research Trust Awards Programs, the Findacure Fundamental Disease Charity, the Rare Disease Research Hub of the Westchester
Bobby Bouthillette, Head of Partnerships, Vibe Bio

Bobby is a dynamic go-to-market leader who has spent the last 13 years in software & startups spanning disruptive, seed-funded organizations in ideation phase through, high-velocity growth organizations across various phases of funding and market maturity. His passion for empowering patients and driving health outcomes has focused the bulk of his work over the last 6 years within health tech and life sciences. Notable accomplishments include three successful exits via acquisition - Vertica (HP), Cloudant (IBM), and Twine Health (Fitbit/Google). Bobby holds a B.S. in Finance from Salve Regina University.

Stacie Calad-Thomson, Ph.D., Chief Strategy Officer, Head of Drug Discovery, BioSymetrics

Stacie is a pharmaceutical R&D and biotech leader with expertise in AI and machine learning applications for drug discovery. At BioSymetrics she guides the company on its drug discovery and partnering strategies, with a focus on translating human-relevant disease biology. Prior to BioSymetrics, Stacie led collaborations and partnerships in the AI-powered drug discovery space as Vice President and Head of AI Molecular Screening Partnerships at Atomwise. Previously she established and co-led the Accelerating Therapeutics for Opportunities in Medicine (ATOM) consortium, a public-private partnership developing machine learning tools to accelerate drug discovery. She worked at GSK for more than 13 years, starting as a process chemist and moving up into R&D strategy and operations roles where she led several change initiatives and teams. Stacie holds a B.S. in Chemistry from University of California Berkeley and a Ph.D. in chemistry from UC Irvine.

Wendy Chung, M.D., Ph.D., Kennedy Family Professor of Pediatrics and Medicine and Director of Clinical Genetics, Columbia University; Director of Clinical Research, Simons Foundation

Dr. Chung leads Simons Searchlight and directs NIH funded research programs in human genetics of pulmonary hypertension, autism, birth defects including congenital diaphragmatic hernia and congenital heart disease. She is a national leader in the ethical, legal, and social implications of genomics. She leads the National Organization of Rare Disorders Center of Excellence at Columbia University. She was the recipient of the NY Academy of Medicine Medal for Distinguished Contributions in Biomedical Science, the Rare Impact Award from the National Organization of Rare Disorders and is a member of the National Academy of Medicine and the American Academy of Physicians. Dr. Chung received her B.A. in biochemistry from Cornell University, her M.D. from Cornell University Medical College, and her Ph.D. from The Rockefeller University in genetics.
Ryan Colburn, Patient, Advocate and Innovator, Pompe Disease

Ryan Colburn is a human with some genetic variants, just like everyone else. Of note, 2(+) are in his GAA gene on chromosome 17. He has a professional background in engineering and operations management; spending portions of his career working on race cars, airplanes, rockets, and satellite communications. Diagnosed with Pompe disease in 2015, he has spent the time since learning about rare disease topics including research, advocacy, and development of treatments to better understand how to participate in the rare disease ecosystem. He is passionate about patient empowerment and engagement; actively developing relationships with other patients, advocacy groups, researchers, and pharmaceutical companies. He is driven to improve the health of the rare disease ecosystem by shifting the view from patients as “subjects” to one of participants, collaborators and partners who help find the most effective ways to tackle the challenges of rare disease and break down barriers to the acceleration of progress.

Lila Collins, Ph.D., Associate Director Therapeutics Development, California Institute for Regenerative Medicine (CIRM)

Dr. Collins joined CIRM in 2008 after a ten-year tenure in the biotechnology industry, including Collateral Therapeutics, where she was the assay group Lead, and most recently at Geron Corporation, where she championed the development and adoption of bioassays including single cell-based assays. Since joining CIRM, Lila has managed several discovery, translation, and clinical grants and represented CIRM at multiple state and national science conferences organized by CIRM as well as external groups. She contributed to building CIRM’s translational and clinical portfolio and accelerating its development. Lila currently manages Translation and Clinical grants in the neuroscience area such as Parkinson’s and ALS, as well as Ophthalmology, Oncology, and Infectious diseases. Lila also serves as the Therapeutics Development team’s lead for CIRM’s diversity, equity, and inclusion initiatives.

Robert Deans, Ph.D., Chief Scientific Officer, Synthego

Bob Deans brings more than 25 years of experience launching and developing stem cell and gene therapies. He brings in global experience from discovery to approval, as well as translational science and global regulatory expertise. Robert was the founding scientific leader and built the teams and pipeline at his previous executive roles at BlueRock Therapeutics and Rubius Therapeutics before joining Synthego. His experience includes the creation of next-generation therapies by harnessing stem cell biology and gene editing tools. He championed elevating industry positioning in standardizing regulatory practices through his participation in several stem cell and therapeutic societies, including ARM, ISCT, and ISSCR. Robert holds a Bachelor’s degree in Molecular Biology from MIT and a Ph.D. in Molecular Genetics from the University of Michigan. He did his postdoctoral work in immunology at the University of California, Los Angeles.
Scott Demarest, M.D., Associate Professor, Colorado Children’s Hospital

Dr. Demarest is an associate professor in the Department of Pediatrics, Division of Neurology. He is board certified in Child Neurology and Epilepsy. His clinical practice and research focus on the evaluation and treatment of neurogenetic conditions. This includes clinical trials for novel therapeutics, natural history studies and the development of improved outcome measures for neurogenetic conditions. He is the Clinical Director of Precision Medicine at Children's Hospital Colorado, co-director of the neurology complex drug program and medical director of the Batten and Neurogenetic Multi-disciplinary Clinics. He is also the director for the International CDKL5 Clinical Research Network.

Yssa DeWoody, Ph.D., Co-founder and Director, Ring14 USA

At Ring14 USA, a non-profit organization, Yssa focuses on improving the lives for those impacted by neurodevelopmental disorders on the 14th chromosome. This work is a labor of love motivated by her daughter, Marie, who was born with the ultra-rare Ring Chromosome 14 Syndrome. Yssa is true believer in collaboration as actualized by her commitment to several consortiums including Ring14 International (Co-founder and Past President), the Rare Epilepsy Network (ex-officio Chair), Epilepsy Leadership Council, Epilepsy Learning Healthcare Systems, and the Commission for Neurodevelopmental CNVs (founding partner).

Wendy Erler, VP of Patient Experience STAR & Advocacy, Alexion Astra Zeneca

With a true passion for patients, and dedication to connecting people in need to resources, services and people, Wendy has built her patient advocacy career in many rare disease communities and oncology. At Alexion, she is responsible for leading the enterprise patient centered innovation model to gather and incorporate patient insights throughout the drug development and commercialization processes. In addition, she leads the Global Patient Advocacy function and has responsibility for the advocacy team. Previously, Ms. Erler was on the executive leadership team at Wave Life Sciences where she led Patient Advocacy and Commercial. During her tenure, she built both functions and developed the commercialization strategy as the organization initiated its rare disease clinical trials in Duchenne muscular dystrophy and Huntington’s disease. Prior to Wave, Wendy held roles of increasing responsibility at Biogen, starting in field sales and advancing through the commercial organization before serving as the patient advocacy lead for several rare disease programs including ALS, SMA, IPF, and Hemophilia. In these roles she gained experience in Newborn Screening, public policy, HTA’s, and bringing the patient voice into all aspects of drug development. Wendy serves on the Life Science Cares Board of Advisors, the Huntington Disease Society of America Board and the Board of the Jett Foundation. Wendy graduated from Miami University and earned her MBA from St. Joseph’s University.

Onno Faber, Co-founder and CEO, Rarebase

Onno Faber has been an entrepreneur his entire life. He started his first company in high school and built several others after receiving his architecture degree. After being diagnosed with the rare neurological disease NF2, Onno shifted his efforts to focus on entrepreneurship in the life sciences. Rather than accept that there were no NF2
treatments available, he took a personalized medicine approach to find a therapy. He identified leading experts in biomedical research, sequenced his DNA, and found an off-label drug that prevented his brain tumors from growing. Because of this experience, Onno was motivated to co-found Rarebase, a public-benefit precision medicine company whose mission is to discover and develop treatments for the millions of people worldwide living with a rare disease.

**Nasha Fitter, Head of Data Platforms, Invitae**

Nasha works closely with patients, advocacy groups and biopharma companies to build natural history studies and utilize real world evidence to accelerate treatments for rare diseases. She is also the mother of a child with the rare neurological condition, FOXG1 Syndrome, and co-founded and leads the FOXG1 Research Foundation. Nasha also serves on the board for the ACMG Foundation for Genetic and Genomic Medicine and received her MBA from the Harvard Business School.

**Sarah Glass, Ph.D., Chief Operating Officer, n-Lorem Foundation**

Sarah is a Molecular Geneticist by training who has extensive experience in precision medicine and drug development from academia, pharmaceutical companies, and CROs. She is acclaimed for forging key strategic partnerships across rare disease sectors and has driven efficiencies in clinical research. Most notably, Sarah combines her professional background with the perspective as a parent of her son who is an n of 1. This allows Sarah to not only personally understand the challenges faced by n of 1 patients and their families, but also to translate this understanding into n-Lorem’s paradigm shifting platform solution for the nano-rare community. Outside of n-Lorem, Sarah is the chair of the DYRK1A Medical and Scientific Advisory Board and is engaged in many rare disease organizations to drive collaboration to reach our common goals.

**Uta Grieshammer, Ph.D., Senior Science Officer, California Institute for Regenerative Medicine (CIRM)**

Uta was at CIRM from 2007 to 2015 and led the programs that created both the Genomics Initiative and the iPSC bank. She also organized several scientific conferences and workshops involving hundreds of CIRM-funded researchers. After leaving CIRM she became the Scientific Director of the California Initiative to Advance Precision Medicine at the University of California San Francisco where she created and managed the application and peer review process. Most recently she was the Program Officer at the University of California Office of the President’s (UCOP) Tobacco Related Disease Research Program where she focused on the neuroscience of nicotine addiction. She also helped develop a scholarship program to attract students from diverse backgrounds to pursue a career in science. Upon returning to CIRM in 2021, Uta now manages a portfolio of discovery and training grants and is leading the development of new concepts within the Scientific Programs portfolio at CIRM.
Rachel Groth, Ph.D., VP and Head of Neuroscience Research, BridgeBio

Rachel Groth has more than 10 years’ experience leading drug discovery and early development efforts in industry across diverse therapeutic areas (e.g., neuroscience, immunology, renal, and oncology) and drug modalities (e.g., small molecules, monoclonal antibodies, and antisense oligonucleotides). Before joining BridgeBio, Rachel held roles of increasing responsibility at Pfizer and Biogen. Rachel earned her B.A. in Biology and Neuroscience from Macalester College, her Ph.D. in Neuroscience from the University of Minnesota, and completed her postdoctoral training in Molecular and Cellular Physiology at Stanford University.

Laura Hameed, Executive Director, Columbus Children’s Foundation

Laura Brod Hameed brings two decades of experience in the fields of bioscience management, non-profit leadership, higher education governance and state government to her role as Executive Director. Most recently, Ms. Hameed served as Chief Executive Officer of RoverMed BioSciences. Prior to that, she was Chief Executive Officer of GeneSegues Therapeutics. Additionally, Ms. Hameed is an Aspen-Rodel Fellow with the Aspen Institute and serves on the board of Givvor, a cloud-based platform for online giving, and Drake Bank, which specializes in small-business lending.

Christopher Hart, Ph.D., CEO and Co-founder, Creyon Bio

Chris is an experienced leader leveraging computational methods, ML/AI, and deep biological insights to solve problems. He has extensive experience in pharma and biotech, including building and leading the functional genomics department at Ionis where he oversaw the development of antisense oligonucleotide predictive analytics for lead identification and enhancement and was responsible for the company-wide genomics and bioinformatics efforts and execution and strategic leadership of translational ’omics and exploratory drug discovery programs in rare and common diseases. Prior to his work in the pharmaceutical industry, he was a faculty member at New College of Florida, worked on methods for single-molecule sequencing at Helicos Biosciences, and advised the White House Office of Science and Technology Policy on policy including personalized medicine. Chris earned his Ph.D. from Caltech and completed post-doctoral training at Yale University.

Tom Hume, Marketing and Communications Lead, RARE-X

Tom joined RARE-X in May 2020, bringing over 25 years of experience leading brand strategy and promotional campaigns for consumer and business brands including Toshiba, Qwest, Sage Software, Gateway Computers, Sprint, and Verizon. He has developed over 1,000 marketing campaigns in his career using a mix of TV, digital, radio, outdoor, public relations, social media, online videos, mobile, and websites. In 2003, Tom co-founded Cure JM Foundation to find a cure and better treatments of Juvenile Myositis (JM) and improve the lives of families affected by JM. Cure JM Foundation has raised over $16 million for medical research for a rare and life-threatening disease that strikes in childhood. Tom was inspired by...
his son’s determination and resilience in fighting JM when he created the vision to launch this organization.

Mustaqhusain Kazi, Global Informatics Strategy Leader, Roche

In his role at Roche, Kazi focuses on driving Roche’s overarching Informatics Strategy and Digital Innovation initiatives to realize game-changing opportunities that meet the dynamic needs of the company’s Pharma, Diagnostics, and Insights businesses. Most recently, Kazi led an internal, cross-functional effort to develop and implement a Roche-wide Data & Digital Backbone for high-impact transformative use cases that reach across the entire value chain from R&D, Commercial, Manufacturing, Real World Data, Finance, Human Resources, and others. Over the past decade, Kazi focused on growing Roche’s Personalized Healthcare (PHC) strategy, culminating in his appointment to Global Head of PHC Informatics in 2018. He assembled a high performing team that built and enabled Roche’s efforts to use multi-modal data (real-world data, genomics, clinical imaging, and digital health) to accelerate the discovery, development, & delivery of personalized medicines & diagnostics to patients.

Walt Kowtoniuk, Venture Partner, Third Rock Ventures; Chair, RARE-X Board of Directors

Walt is the father of two who spends his days playing with his kids, teaching them about the world, and having fun watching them grow up. Walt is also passionate about making a difference for patients. He spends his days focused on genetics and genomics and the insights they bring into disease biology. Walt works where science meets business and strategy, enabling insights from the lab to become the next generation of medicines that can change lives. He is enthusiastic about launching companies with emphatic, truly patient-centric cultures that effectively and efficiently execute drug discovery. He places priority on taking the time to listen to the patients whom we endeavor to help, recognizing that the value we aim to create is defined by the magnitude of the difference we can make in their lives.

Kirk Lamoreaux, Founder, Cogenticity, LLC

Kirk has over 25 years of healthcare experience. He began his career at Pfizer Inc., where his roles included Director, eHealth Strategy, U.S. Planning & Business Development, and Director, Strategic Policy, Worldwide Policy. Kirk has a B.A. from Brigham Young University and an MBA from Saint Joseph’s University in Philadelphia. He currently resides in Phoenix, AZ.

Casey McPherson, Founder and President, To Cure a Rose Foundation; Founder and CINO, Everlum Bio

Casey spent most of his life as a professional singer-songwriter, performing all over the world with his bands Alpha Rev and Flying Colors. Casey’s music has been featured on VH1 and MTV, as well as topping the charts in the top 10 on U.S. radio. However, his world changed forever when his youngest daughter, Rose, was diagnosed with a rare
genetic disease with no cure: HNRNPH2. He founded To Cure a Rose Foundation to develop a therapeutic for Rose and scale a better model to develop rare therapeutics for children. Casey then built Everlum Bio, a proof-of-concept drug discovery lab, to serve family foundations in drug development. Now, he is a leader on a mission to save his daughter and change the way we treat children across the rare disease spectrum. Casey lives in Austin, TX with his two daughters, Weston and Rose, and their dog, Betsy.

**Glenn Morrison, VP of Clinical Development and Neuroscience/Rare Disease, Recursion**

Glenn has over 20 years’ experience in clinical development. Before joining Recursion, he led clinical development at both small and large bio-pharma companies. He spent six years at Genentech and Roche, where he led the Phase 3 implementation of global clinical development for two anti-amyloid antibodies in Alzheimer's disease. Subsequently, he was VP of Global Clinical Development at Zogenix, where he led global clinical development to deliver U.S. and European Medicines Agency approvals of fenfluramine for Dravet syndrome. Most recently, he was VP of Neurology Clinical Development at Alector, where he led the design and execution of development programs in neurology, with a specific focus on Alzheimer's disease and ALS. Glenn earned his B.S., M.S. and Ph.D. in neurobiology all from the University of Toronto and completed his post-doc at UCLA. He is an avid golfer and enjoys car detailing and sports cars.

**Indu Navar, CEO and Founder, EverythingALS**

Over the past 20 years, Indu has served as CEO, founder, board member, and investor of several Silicon Valley software companies, during which she led the development of innovative products resulting in successful exits. In 2019, after she lost her husband to ALS, a neurological disease, she has focused on bringing her skills in building scalable platforms and technology innovations to patient-driven health ventures. Since 2019, Indu has been CEO and founder of the Peter Cohen Foundation, which functions as EverythingALS.org, a 501c3 non-profit organization focused on technology-based solutions to the discovery of biomarkers for neurological diseases. EverythingALS brings together researchers and providers from esteemed institutions as well as patients to conduct research and openly share data for potential treatments and a cure for ALS, with implications for other neurological diseases. In addition, Indu is a board member of Global Genes and on the advisory board of Answer ALS. Indu is focused on bringing the technology framework from Peter Cohen Foundation into larger rare disease communities. She started as an engineer at NASA in Moffett Field. Indu has B.S Electrical Engineering from Bangalore University, India and M.S. in Computer Science from California State University, Chico.

**Lurong Pan Ph.D., Founder and CEO, Ainnocence Inc.**

Dr. Lurong Pan has extensive drug design and precision medicine research experience using structural biology, computational chemistry, and artificial intelligence technologies. She was previously a senior investigator at Global Health Drug Discovery Institute and a research scientist in structural biology and computational biology at the University of Alabama at Birmingham. Dr. Pan received her B.S. in Applied Chemistry from Nanjing University, M.S. in Computer
Science from Georgia Tech, and Ph.D. in Chemistry from the University of Alabama at Birmingham. She is also an IBM-certified big data architect.

Maria Picone, Co-founder and CEO, TREND

Maria and her husband Chris started TREND together after their daughter was born with a rare disease called Prader-Willi syndrome. TREND is a digital health analytics company with a bold vision to revolutionize the world's understanding of diseases. TREND’s technology analyzes social media conversations to understand diseases from patient and caregiver perspectives. These valuable insights can shed light on aspects of our health conditions that even our most qualified doctors and scientists couldn’t possibly know.

Luke Rosen, Founder, KIF1A.ORG

Luke Rosen and Sally Jackson founded KIF1A.ORG in 2016 following their daughter Susannah’s KIF1A diagnosis. In 2017 Luke left his career in film and television to accelerate discovery of treatment for Susannah and children like her living with neurodegenerative conditions. Luke’s mission is to support biotech innovation and forge efficient collaborations to rapidly discover treatment for KIF1A Associated Neurological Disorder. Luke has a Bachelor of Fine Arts in Classical Theater, and a Master of Science in bioethics. He has written extensively on the family impact of rare genetic diseases and is an award-winning playwright.

Morrie Ruffin, Managing Partner, Adjuvant Partners; Co-founder, AAIH; Board Member, RARE-X

Mr. Ruffin has more than 25 years of experience in the Biotech and Healthcare industries. Prior to founding Adjuvant Partners, he was CEO of LifeTech Innovations, LLC, and was an original employee of the Biotechnology Innovation Organization (BIO), serving as VP of Capital Formation and Business Development. He also worked for U.S. Senator Arlen Specter for five years as his senior legislative assistant. In addition, over the past 15 years Mr. Ruffin has founded and led/leads several key industry organizations in the life sciences sector with a primary focus on cell and gene medicine and the application of artificial intelligence in healthcare. These organizations include the Alliance for Artificial Intelligence in Healthcare (AAIH), the Alliance for Regenerative Medicine (ARM), the Standards Coordinating Body for Regenerative Medicine (SCB), the Foundation for Cell & Gene Medicine, and the Interoperable Informatics Infrastructure Consortium (I3C). He also currently serves as a member of the Board of Directors for the SCB, the Foundation for Cell & Gene Medicine, and RARE-X. Mr. Ruffin received his MA from the Johns Hopkins School for Advanced International Studies (SAIS) and his BA from the University of Virginia.

Bina Shah, Founder and CEO, Project 8p Foundation

In 2018, Bina founded Project 8p Foundation to accelerate research with the goal of treatment for chromosome 8p heroes like her daughter Karina. Since then, she has fully committed herself to the advancement of clinical and research understanding of rare disorders, specifically neurodevelopmental chromosomal disorders, such as the...
8p rearrangements. Shifting perspectives from disease specific efforts to innovative approaches that are gene-agnostic and cross disorder is a long-term scalable vision of hers. As a result, she is the founding partner of the Commission on Novel Technologies for Neurodevelopmental Copy Number Variants (CNVs). Bina earned her B.S. from NYU Stern Business School and her M.B.A. from Columbia University. She has over 15 years of experience in finance in the areas of mergers and acquisitions, private and public equity investing, real estate development, and property management. Bina leverages her business and entrepreneurial background and network to build bridges to improve efficiencies in a Collaborative Impact model for Team Science.

**Charlene Son Rigby, CEO, RARE-X**

Charlene Son Rigby has spent her career building organizations at the intersection of data, technology, and life sciences. Charlene was previously Chief Business Officer at Fabric Genomics and held executive roles at enterprise software and genomics companies, including Oracle and Doubletwist. She started her career in neuroscience research at Roche. When Charlene’s daughter was diagnosed with a rare genetic disease, she co-founded the STXBP1 Foundation. She is committed to finding a cure for her daughter’s disorder. Charlene's unplanned connection between her personal life and profession has helped push forward the search for a cure for her daughter and kids like her and given her work deeper meaning. Charlene joined RARE-X because she firmly believes that a platform approach is crucial to transforming and accelerating therapy development across rare diseases. She holds a B.A. in Human Biology from Stanford University and an M.B.A. from the Haas School of Business at UC Berkeley.

**Alok Tayi, Ph.D., Co-founder and CEO, Vibe Bio**

Alok Tayi, Ph.D., has 15 years of experience as a scientist, including a postdoc at Harvard and 12 years as a serial entrepreneur. During this time, he started several notable life sciences focused software companies that have employed over 300+ and raised 100M+ in funding. Alok founded Vibe Bio after his daughter was hospitalized at birth and he learned first-hand that the biggest obstacle to developing treatments isn’t finding potential therapies — it’s funding them. Vibe Bio is building a community of patients, scientists, and partners to identify promising treatments for overlooked disorders and funding their development through the creation of a DAO via cryptocurrency token sales.

**Karmen Trzupek, Senior Director of Scientific Programs, RARE-X**

Karmen Trzupek is a passionate and innovative leader in rare disease programming, with experience in academia, telemedicine, advocacy, and industry. At RARE-X, Karmen develops collaborative ecosystems and partnerships to maximize the RARE-X data platform for the advancement of patient advocacy groups and industry-supported therapeutic pipelines. Previously, Karmen served as the Director of Clinical Trial Services at InformedDNA, supporting patients with genetic diseases to navigate clinical trial eligibility and enrollment through partnerships between biotech companies, patient advocacy groups, and academic and community clinics. As a clinical genetic counselor, Karmen developed the first nationwide telemedicine program for rare disease genetic counseling and testing, and co-developed the first pharma-sponsored genetic testing program in the U.S.
Winston Yan, M.D., Ph.D., Organizing Committee, N=1 Collaborative; Co-founder, Arbor Biotechnologies

Winston is a genome engineer and organization builder motivated by bringing genetic medicines to treating patients with serious disease. He has a BS in Physics from Harvard College and then joined the Harvard/MIT M.D.-Ph.D. program, where he completed his Ph.D. with Feng Zhang at MIT/Broad Institute, working to enable therapeutic applications of CRISPR genome editing technologies. Following his Ph.D., in 2017 he co-founded Arbor Biotechnologies with David Scott, David Walt, and Feng Zhang to bring the genetic cures to a wider array of diseases by scaling the discovery and therapeutic development of new genome editing tools. At Arbor, he served as Head of Operations, helping drive the development of Arbor’s technology platform and company strategy, while building out key aspects of early culture and operational efficiency. In October 2020, Winston returned to Harvard Medical School to complete his M.D. but remains actively engaged as a consultant with Arbor. Having had the incredible opportunity to be an early part of the CRISPR revolution, build a thriving biotech, and learn in cutting-edge clinical environments, Winston is currently utilizing his scientific, strategic, and operational experience to help build the N=1 Collaborative with the care and urgency that rare disease patients need.

Julia Vitarello, Mila’s Mom; Founder & CEO, Mila’s Miracle Foundation

Julia founded Mila’s Miracle Foundation in 2016 upon learning that her 6-yr-old daughter, Mila had Batten disease, a fatal genetic condition with no cure. In a race to save Mila, Julia’s collaboration with Dr. Timothy Yu from Boston Children’s Hospital led to the first ever drug tailored to just one person, affectionately named milasen. After showing great promise in the first year of treatment, Mila's disease slowly progressed. In February 2021, Mila’s big spirit left her little body. Driven by a sense of hope and responsibility, Julia is on a mission to open up the field of individualized medicines for many more.

Vanessa Vogel-Farley, Research and Data Governance Lead and Principal Investigator, RARE-X

In addition to her work at RARE-X, Vanessa is a co-founder of the Commission on Novel Technologies for Complex Copy Number Variants and serves on the Coordinating Committee for the Rare Epilepsy network as well as the Epilepsy Leadership Council and the Alliance for Genetic Etiologies in Neurodevelopmental Disorders and/or Autism. Vanessa served as Executive Director of the Dup15q Alliance from 2016 – 2021 and joined the Dup15q Alliance Board in 2021 to lead Science and Research Strategy. She also served as Director of Operations of ACEing Autism from 2008 through 2015, and prior to that was at the University of Minnesota, Center for Neurobehavioral Development. Vanessa also served as the Clinical Research Coordinator for the Division of Developmental Medicine Laboratory of Cognitive Neuroscience, Boston Children’s Hospital, working on collaborations with scientists from MIT and Harvard examining several clinical populations, including autism. She possesses 20 years of experience in data collection methods as well as expertise in non-profit and research operations, patient advocacy and support, non-profit management, and broad knowledge of child development neuroscience/psychology research and administration.