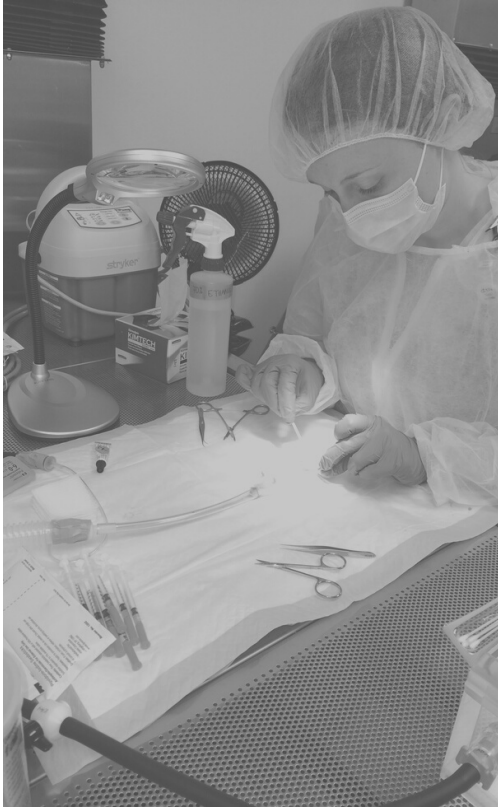

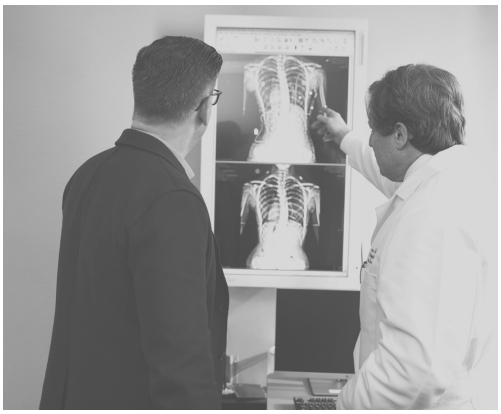

Patient Advocates' Role in Advancing Gene Therapy

Pre-Meeting Workshop

Monday, May 10

10 a.m. - 2 p.m. ET


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REGENXBIO is committed to developing gene therapies that improve treatment options for people with serious diseases. The personal stories of patients and families help guide our work. We earn their trust through our actions and our words.

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Patient Advocates' Role in Advancing Gene Therapy

10 - 10:30 a.m.: Overview of Gene Therapy, Research, and Development
Kevin Flanigan, M.D., Nationwide Children's Hospital

Session 1: Lessons Learned From Initiating Gene Therapy Research
Co-Chairs: Rachel Bailey, Ph.D. & Sharon King

Speakers share to their experience getting started and going through the process of pursing preclinical research or clinical trials for gene therapy including successes, challenges, and changes they would make.

10:30 - 10:42 a.m.
Gina Hann, The Rare Village Foundation

10:42 - 10:54 a.m.
Christine Waggoner, Cure GM1 Foundation

10:54 - 11:06 a.m.
Sue Wilson, The Children’s Medical Research Foundation, Inc.

11:06 - 11:18 a.m.
Amber Olsen, United MSD Foundation

11:18 - 11:30 a.m.
Matt Wilsey, Grace Science Foundation

11:30 a.m. - 12 p.m.
Panel Discussion

- Kevin Flanigan, M.D., Nationwide Children's Hospital
- Gina Hann, The Rare Village Foundation
- Christine Waggoner, Cure GM1 Foundation
- Sue Wilson, The Children’s Medical Research Foundation, Inc.
- Amber Olsen, United MSD Foundation
- Matt Wilsey, Grace Science Foundation

Patient Advocates' Role in Advancing Gene Therapy

Session 2:

Understanding the Research Process: Being Equipped for Success
Co-Chairs: Rachel Bailey, Ph.D. & Sharon King

12:15 - 12:30 p.m.
Preparing a Disease to Be Ready for Research
Steven Gray, Ph.D., University of Texas Southwestern

12:30 - 12:45 p.m.
Building Successful Relationships
Jill Wood, Phoenix Nest, Inc.

12:45 - 1 p.m.
Managing Expectations and Being Prepared for Setbacks
Michelle Berg, Aldevron

1 - 1:15 p.m.
NIH Pathways to Gene Therapy Treatments for Diseases Lacking Commercial Interest
P.J. Brooks, Ph.D., National Center for Advancing Translational Sciences

1:15 - 2 p.m.
Panel Discussion

- Steven Gray, Ph.D., University of Texas Southwestern
- Jill Wood, Phoenix Nest, Inc.
- Michelle Berg, Aldevron
- P.J. Brooks, Ph.D., National Center for Advancing Translational Sciences

Patient Advocates' Role in Advancing Gene Therapy

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Preparing a Disease to Be Ready for Research

Steven Gray, Ph.D., University of Texas Southwestern

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Speakers

Rachel Bailey, Ph.D.
University of Texas Southwestern Medical Center

Dr. Rachel Bailey has a broad background in studying protein aggregation in neurodegenerative disorders, modeling neurological diseases in rodents, and developing gene therapies to treat those disorders. Dr. Bailey's lab focuses on the development of gene therapies for neurological disorders and facilitates the translation of these treatments for use in humans. Pediatric disorders that she is working on include SLC13A5 epileptic encephalopathy, Multiple Sulfatase Deficiency, Giant Axonal Neuropathy and ECHS1 Deficiency. Dr. Bailey's gene therapy research efforts to include more complex neurodegenerative disorders, including tauopathies, such as Alzheimer's disease. Her lab utilizes AAV vector engineering for both gene replacement and gene-silencing approaches, explores alternative methods of virus delivery, performs proof-of-concept studies in cell culture models, and performs IND-enabling studies in animal models. Dr. Bailey works closely with Patient Foundations and industry sponsors to facilitate the translation of these therapies for use in the clinic.

Michelle Berg
Aldevron

Michelle Berg brings over 20 years of experience in the biotechnology sector, 10 years of which have been spent in executive leadership roles. In her current position as President of Aldevron's GMP Nucleic Acids Business Unit, Berg oversees Aldevron's strategy to provide GMP plasmids and mRNA for gene editing, gene therapy, and cell therapy applications. She works closely with the company's operational team to meet the requirements of these growing fields, while supporting the clinical and commercial efforts of Aldevron's clients. Aldevron's GMP plasmid facility, located in Fargo, N.D., is the largest in the world. Berg earned a Bachelor of Science in Biotechnology from North Dakota State University (NDSU). She is a contributing author and speaker on patient-focused programming, rare disease advocacy, and accessible education on genetic medicines. In addition to her roles with Abeona and Aldevron, she has performed research on behalf of the Department of Plant Sciences at NDSU.

P.J. Brooks, Ph.D.
National Center for Advancing Translational Sciences

Dr. Philip John (P.J.) Brooks is a Program Director in the NIH National Center for Advancing Translational Sciences (NCATS) Office of Rare Diseases Research (ORDR). Dr. Brooks received his Ph.D. in neurobiology from the University of North Carolina at Chapel Hill. After completing a postdoctoral fellowship at the Rockefeller University, Brooks became an investigator in the NIH intramural program. He developed an internationally recognized research program focused on rare neurologic diseases resulting from defective DNA repair. Since joining NCATS and ORDR, Dr. Brooks is interested in accelerating clinical trials in rare diseases by moving beyond "one disease at a time" approaches. Examples include the development of therapeutics that target shared molecular mechanisms underlying multiple rare diseases, platform technologies for the delivery of nucleic acid therapeutics, and the implementation of recommendations regarding the acceleration of gene therapy clinical trials. He is also the coordinator of the NIH Common Fund's Somatic Cell Genome Editing (SCGE) program (https://commonfund.nih.gov/editing). Dr. Brooks was recently elected as the Interdisciplinary Scientific Committee Chair for the International Rare Diseases Research Consortium

Kevin Flanigan, M.D.
Nationwide Children's Hospital

Kevin Flanigan, M.D., is the Director of The Center for Gene Therapy at Nationwide Children's Hospital in Columbus, Ohio, where he holds the Robert F. & Edgar T. Wolfe Foundation Endowed Chair in Neuromuscular Research. He directs the NCH Neuromuscular Program as well as Nationwide Children's NIAMS P50-funded Center for Research Translation(CORT) in Muscular Dystrophy Therapeutic Development. He trained in Neurology & Neuromuscular Medicine at Johns Hopkins University, pursued a post-doctoral fellowship in Human Molecular Biology and Genetics at the University of Utah, and currently holds appointments as Professor of Pediatrics and Neurology at the Ohio State University. His research focuses on genotype/phenotype correlations in the muscular dystrophies, with a goal of understanding molecular mechanisms that lead to amelioration of symptoms in order to identify new therapeutic pathways. His laboratory works primarily on AAV-based approaches to gene therapy, including viral-vector based exon skipping and gene replacement.

Steven Gray, Ph.D.
University of Texas Southwestern

Dr. Steven Gray earned his Ph.D. in molecular biology from Vanderbilt University in 2006, after receiving a B.S. degree with honors from Auburn University. He performed a postdoctoral fellowship focusing on gene therapy in the laboratory of Jude Samulski at UNC Chapel Hill. He is currently an Associate Professor in the Department of Pediatrics at the University of Texas Southwestern Medical Center. Dr. Gray is the director of the UTSW Viral Vector Facility and maintains affiliations with the Department of Molecular Biology, the Department of Neurology and Neurotherapeutics, the Eugene McDermott Center for Human Growth and Development, and the Hamon Center for Regenerative Science and Medicine at UT Southwestern. Dr. Gray's core expertise is in AAV gene therapy vector engineering, followed by optimizing approaches to deliver a gene to the nervous system. His research focus has also included preclinical studies to development AAV-based treatments for neurological diseases, some of which have translated into clinical trials.

Gina Haan

The Rare Village Foundation

Gina Hann is a semiconductor marketing engineer by profession, as well as a rare disease parent and advocate. Gina founded Batten Hope Foundation in 2017, when her son Joseph was diagnosed with Batten disease (CLN7). She has worked closely with UT Southwestern and Children's Health based in Dallas, Texas in the development of an AAV vector for use in a gene therapy clinical trial - in 2020, the drug developed received FDA approval for use. Gina has partnered with another rare mom to co-found the Rare Village Foundation - a nonprofit organization focused on empowering families to build treatment for rare childhood disease. In 2020, the Rare Village launched their fiscal sponsorship fund for rare disease research, as well as the first episode of a documentary series titled, "One Shot to Live" in the hopes of inspiring newly diagnosed families to initiate the work needed to change the outcome for rare childhood diseases. Gina received her BSEE from University of Arizona, and an MBA from the Eller School of Management, University of Arizona.

Amber Olsen
United MSD Foundation

Amber Olsen is the mother to three daughters. In 2016, her youngest daughter Willow was diagnosed with Multiple Sulfatase Deficiency(MSD), an ultra rare, terminal genetic condition. Since the diagnosis, Amber formed the United MSD Foundation (501c3),assisted in forming an independent Scientific Advisory Board of experts, joined together with three global sister organizations, and is pushing forward the first ever Clinical trial for MSD. Olsen is the Executive Director and chief fundraising officer for the organization and will not stop until every child with MSD has an opportunity to live a healthy life without limitations. As a rare disease advocate, Amber has met with the Federal and State Legislators, been selected as a speaker at Rare Disease and Leadership conferences and served on leadership committees for the United Leukodystrophy Foundation and various other rare disease organizations. Amber and her husband, Tom Cannan, have resided in the beautiful and giving community of South Mississippi for the last 23 years with their three wonderful daughters, Kylee (18), Jenna (14), and Willow (7). In her pre-MSD career, Amber founded Nextaff Gulf Coast in Ocean Springs, MS in 2007 to offer recruiting services for businesses in South Mississippi. Amber is certified by the Human Resource Certification Institute as a Senior Professional in Human Resources(SPHR). Amber is originally from Missoula, Montana where she earned her Bachelor's Degree in Business in 1998 from University of Montana. Amber has held various community leadership positions, Mississippi Gulf Coast Chamber Board of Directors, former President of the Ocean Springs Rotary Club and former member of the board of Directors of the Mississippi Center for Medical Fragile Children, a collaborative initiative with former First Lady of Mississippi, Deborah Bryant.

Sharon King
Aldevron

From her experience caring for her daughter with a rare disease, Sharon King understands the unique challenges and social and emotional needs of patients and caregivers. The experience informs her role as manager of Advocacy and Community Engagement at Aldevron where Sharon educates, connects, and improves understanding of the patient communities potentially benefitting from our clients' innovations. While she is blazing a new trail in this part of industry, Sharon is not a stranger to creating new initiatives. She co-founded and serves as President of Taylor's Tale, the public charity named for her late daughter to raise awareness and funding for CLN1 disease (Batten disease)research and advocate for the needs of rare disease patients. Sharon is a state-appointed member of the N.C. Advisory Council on Rare Diseases and chair of the N.C. Rare Disease Coalition. She served on the Rare Disease InfoHub steering committee, funded by a N.C. University System grant to leverage artificial intelligence and analytics with medical systems to improve identification, diagnosis, treatment and support for rare disease patients and families. Sharon is a tireless advocate for patient-centered, integrated, comprehensive care networks. Her patient-focused approach is the foundation for her work at Aldevron.

Christine Waggoner
Cure GM1 Foundation

Christine Waggoner and her husband Douglas Dooley founded the Cure GM1 Foundation in April 2015 in honor of their daughter Iris and all those affected by GM1 Gangliosidosis. Christine graduated from Brown University where she studied Visual Art and Computer Science. The combination of studies in art and technology served as a basis for her career in computer graphics and 3D feature film animation. With more than five years in rare disease patient advocacy, Cure GM1 has contributed to multiple gene therapy efforts, patient registries, the development of animal models, biomarkers, and to newborn screening. Cure GM1 is the only 501(c)(3) nonprofit entirely dedicated to GM1 Gangliosidosis and the GM1 patient community. The founding and running Cure GM1 is a true labor of love to help advance possible treatments to all those affected by GM1 Gangliosidosis.

Matt Wilsey
Grace Science Foundation

Matt is a Silicon Valley entrepreneur, angel investor, and start-up advisor. In addition to consumer products and services, Matt invests in and advocates for biomedical research, drug development, and genetic sequencing technologies. Before moving to the investment side, he spent many years as a front-line operator. Most recently, Matt was Co-founder and Chief Revenue Officer of CardSpring, a payment infrastructure company that was acquired by Twitter. Previously, Matt ran West coast sales and business development for Howcast.com. Before Howcast, Matt worked for Kohlberg Kravis Roberts (KKR) on the Capital Markets team focused on new product development, capital raising, and investor relations. Prior to that, Matt spent five years as Co-founder and Vice President of Business Development at Zazzle.com. He started his career serving in various roles at the White House and the Department of Defense. Matt became a "rare disease hunter" and advocate after his daughter, Grace, was born with NGLY1 Deficiency. He has since funded over 150 scientists at 20 medical centers in 5 countries with the sole purpose of treating the disease. Matt holds a BA from Stanford University and a MBA from Stanford's Graduate School of Business. In addition to the Grace Science Foundation, Matt is also a Board member at the Charles and Helen Schwab Foundation.

Sue Wilson

The Children's Medical Research Foundation, Inc.

Prior to co-founding The Children's Medical Research Foundation, Inc. with her husband, Brad, in 1995, Sue had more than 17 years of business experience, growing from administrative assistance to office management and finally to supervision of the structure and management of two office locations for a spinoff of a major corporation. All of these experiences helped her in forming the Foundation (www.curekirby.org) and moving it toward its mission of a cure for Sanfilippo Syndrome. Sue is president of the not-for-profit corporation based in suburban Chicago, which also has brought together dozens of other Sanfilippo families and their fundraising efforts. To date, the Foundation has granted $4.4 million to ten research groups and has assisted in the funding of human trials for Sanfilippo Syndrome, the disease that afflicted the Wilson's daughter Kirby. Sadly, Kirby passed away on October 22, 2019. But, Sue's resolve to find a cure remains unwavering. Inspired by Kirby's fortitude and will for happiness, she and the Foundation now strive to ensure Kirby's legacy of unbounded spirit and tenacity. The Foundation's mission remains - A Cure for Kirby.

Jill Wood
Phoenix Nest, Inc.

Jill Wood Co-Founded Jonah's Just Begun-Foundation to Cure Sanfilippo with her husband Jeremy Weishaar in May of 2010. After their son Jonah was diagnosed with Sanfilippo Syndrome type C. JJB's mission was to foster a treatment for Sanfilippo Syndrome type C; by connecting researchers, funding science, and mobilizing the patient population. Once this was accomplished Jill dissolved JJB and Co-Founded Phoenix Nest Inc., PN's priority is to bring the treatments that JJB initiated to the clinic and onto commercialization.

Disclosures

K.M. Flanigan
Apic Bio; Honorarium; Advisory board

4D Molecular Therapeutics; Stock options; Advisory board

Audentes; Royalty, research support; Licensing agreement, contracted research

S.J. Gray
Taysha Gene Therapies; Possible royalties; Inventor

Asklepios Biopharma; Royalties; Inventor

Abeona Therapeutics; Royalties; Inventor

Neurogene; Royalties; Inventor

Sarepta; Consulting fee; Advisory board membership

Vertex Pharmaceuticals; Consulting fee; Advisory board membership

Lysogene; Consulting fee; Advisory board membership

Opsin; Consulting fee; Advisory board membership