GENE THERAPY INITIATIVE SYMPOSIUM

3RD ANNUAL SYMPOSIUM

2026

A COLLABORATIVE EVENT | 2 DAYS • MORE INSIGHT • STRONGER CONNECTIONS

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DAY 1 PROGRAM

WEDNESDAY · SEPTEMBER 16TH

7:30 AM - 8:30 AM	***Registration & Breakfast***
8:30 AM - 8:45 AM	***Welcome Remarks*** **Stephanie Cherqui, PhD | UC San Diego** *Director of Gene Therapy Initiative* **Barbara Jung | UC San Diego** *Associate Vice Chancellor & Dean of School of Medicine* **Nancy Stack | Cystinosis Research Foundation** *Founding Trustee & President of Cystinosis Research Foundation*
Session 1	**Antisense Oligonucleotides for Rare Disorders**
8:45 AM - 9:05 AM	**Olivia Kim-McManus, MD | UC San Diego & Rady Children's Institute for Genomic Medicine** *Antisense Oligonucleotides for Rare Pediatric Neurological Disorders*
9:05 AM - 9:25 AM	**Laurence Mignon, PhD | n-Lorem Foundation** *From Hope to Reality: Turning Precision Medicine into Action for Nano-Rare Patients*
9:25 AM - 9:35 AM	**Leah Schust Myers | FamilieSCN2A Foundation** *From the One to the Many*
9:35 AM - 9:45 AM	**Q&A**
Session 2	**Industry Talk**
9:45 AM - 10:00 AM	**Beth Dlouhy & Natalie Marquez | Miltenyi** *From Discovery to Delivery: Overcoming Challenges in Translating Next-Generation Therapies*
10:00 AM - 10:15 AM	**Break**
Session 3	**Ex Vivo Cell Platform for Safety Studies and Treatments for Rare Diseases and Cancer**
10:15 AM - 10:35 AM	**Stephanie Cherqui, PhD | UC San Diego** *Clinical Translation of PPL-001, an Autologous, Gene-Edited Hematopoietic Stem Cell Therapy for Friedreich's Ataxia*
10:35 AM - 10:55 AM	**Alysson Muotri, PhD | UC San Diego** *Fast and Furious: Human Brain Organoids Reach the Clinic - A Translational Inflection Point*
10:55 AM - 11:15 AM	**John Bobby Goulding | Fate Therapeutics Inc.** *Off-the-Shelf and On-Target: How iPSC-Derived CAR T Cells Could Democratize Cell Therapy*
11:15 AM - 11:30 AM	**Q&A**

WIFI NETWORK: Public
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Session 4	Panel on Navigating Bench to Bedside
11:30 AM - 12:00 PM	**Dan Oliver, MBA | CEO & Co-founder of Rejuvenate Bio** **Nicole Paulk, PhD | CEO & Founder of Siren Biotechnology** **Karen Potter, PhD | Partner at Morrison Foerster**
12:00 PM - 1:00 PM	**Lunch**

Session 5	In Vivo Gene Editing as an Emerging Technology
1:00 PM - 1:40 PM	***Keynote | Kiran Musunuru, MD, PhD | University of Pennsylvania*** *Developing and Deploying Personalized Gene-Editing Therapies*
1:40 PM - 1:50 PM	**Q&A**
1:50 PM - 2:10 PM	**Alexis Komor, PhD | UC San Diego** *Development of Precision Genome Editing Methods for Disease Modeling and Treatment*
2:10 PM - 2:30 PM	**Brieana Buckley, PharmD, MS | Editas Medicine** *EDIT-401: A Potentially Transformative, Investigational In Vivo CRISPR Gene-Editing Medicine to Upregulate LDLR & Reduce Multiple Atherogenic Lipoproteins*
2:30 PM - 2:40 PM	**Q&A**
2:40 PM - 2:55 PM	**Break**

Session 6	AAV Gene Therapy: Improved Capsids and Groundbreaking Applications
2:55 PM - 3:05 PM	**Sharif Tabebordbar, PhD | Novartis** *AAV Optimization Overview*
3:05 PM - 3:25 PM	**Brian Head, PhD | UC San Diego** *Neuron-Targeted Caveolin Gene Therapy to Mitigate Neurodegenerative Proteinopathies*
3:25 PM - 3:45 PM	**Prashant Mali, PhD | UC San Diego** *A Nature-Guided Approach for Engineering Targeted Delivery*
3:45 PM - 4:05 PM	**Katherine Cygnar, PhD | Regeneron Pharmaceuticals, Inc.** *The CHORD Trial: An Update on the Efficacy and Safety of DB-OTO Gene Therapy for Profound Deafness in Children*
4:05 PM - 4:20 PM	**Q&A**
4:20 PM - 4:25 PM	***Closing Remarks***
4:30 PM - 5:30 PM	***Poster Session***
5:00 PM - 6:00 PM	***Networking Mixer with Food & Drinks***

SPEAKERS/PANELISTS

OLIVIA KIM-MCMANUS, MD | 8:45 AM

Associate Clinical Professor of Neurosciences | UC San Diego &
Rady Children's Institute for Genomic Medicine
Antisense Oligonucleotides for Rare Pediatric Neurological Disorders

Olivia Kim-McManus, MD, is an Associate Clinical Professor of Neurosciences and a physician-scientist supported by the California Institute for Regenerative Medicine and the National Center for Advancing Translational Sciences. Board-certified in pediatric neurology, epilepsy, and clinical neurophysiology, her research focuses on precision therapeutics for rare genetic diseases, including antisense oligonucleotides. Dr. Kim-McManus has extensive IND regulatory experience and serves as principal investigator on multiple intrathecal and intraventricular clinical programs. She is also Vice Chief of the Neurology MSEC Section at Rady Children's Hospital and serves on several advisory and scientific review boards.

LAURENCE MIGNON, PHD | 9:05 AM

Vice President of Clinical Development | n-Lorem Foundation
From Hope to Reality: Turning Precision Medicine into Action for Nano-Rare Patients

Laurence Mignon, PhD, is Vice President of Clinical Development at n-Lorem Foundation. She has over 20 years of experience in academic research and clinical development focusing on rare diseases. She previously worked at Ionis Pharmaceuticals in Translational Medicine and Clinical Development, contributing to the development of Spinraza for spinal muscular atrophy and initiating the first clinical study in myotonic dystrophy type 1. She also held roles at Orexigen Therapeutics and the Neuroscience Education Institute. Dr. Mignon earned her PhD in Pharmacology and Experimental Therapeutics from Loyola University Chicago and conducted neurology research at the University of California, Los Angeles.

LEAH SCHUST MYER | 9:25 AM

Founder & Executive Director | FamilieSCN2A Foundation
From the One to the Many

Leah Schust Myers is Founder & Executive Director of the FamlieSCN2A Foundation, where she leads efforts to advance research, patient support, and therapeutic development for individuals affected by SCN2A-related disorders. With over 20 years of experience in healthcare administration, her expertise spans healthcare operations, strategic leadership, communications, and community engagement. Following her son’s diagnosis with an SCN2A-related disorder, she became a leading advocate for the rare disease community. In her current role, she has helped position SCN2A as a leading target for precision medicine while driving research, collaboration, and awareness worldwide.

BETH DLOUHY & NATALIE MARQUEZ | 9:45 AM

Clinical Applications & Technical | Miltenyi

From Discovery to Delivery: Overcoming Challenges in Translating Next-Generation Therapies

Beth Dlouhy, Clinical Applications Manager, and Natalie Marquez, Technical Sales Consultant, support academic and industry cell and gene therapy teams at Miltenyi Biotec. Their expertise spans translational research, workflow optimization, process development, GMP manufacturing, and advanced cell processing. Beth supports the implementation of Miltenyi Biotec's clinical cell and gene therapy portfolio, helping customers translate and manufacture advanced therapies from development through clinical production, while Natalie provides technical guidance to optimize workflows, evaluate technologies, and address technical challenges. Beth brings ten years of cell and gene therapy experience, while Natalie has over five years of biotechnology industry experience.

STEPHANIE CHERQUI, PHD | 10:15 AM

Professor of Pediatrics & Director of Gene Therapy Initiative | UC San Diego

Clinical Translation of PPL-001, an Autologous, Gene-Edited Hematopoietic Stem Cell Therapy for Friedreich's Ataxia

Stephanie Cherqui is a professor in the Department of Pediatrics, Division of Genetics at the University of California San Diego, Director of the UC San Diego Gene Therapy Initiative, and Ben and Wanda Hildyard Endowed Chair for Mitochondrial and Metabolic Diseases. Her research focused on developing hematopoietic stem cell gene therapy for metabolic and neurological disorders, with a strong track record of translating fundamental discoveries into clinical applications. Among her most significant achievements is the development of the first-in-human autologous hematopoietic stem cell gene therapy for cystinosis, an inherited metabolic disorder. She is the Chair of the Cystinosis Stem Cell and Gene Therapy Consortium and the Chair of the Scientific Review Board of the Cystinosis Research Foundation.

ALYSSON MUOTRI, PHD | 10:35 AM

Professor of Pediatrics & Co-Director of Gene Therapy Initiative | UC San Diego

Fast and Furious: Human Brain Organoids Reach the Clinic - A Translational Inflection Point

Dr. Muotri is a professor at UC San Diego, Co-Director of the Gene Therapy Initiative, and Director at several university centers focused on stem cells, space, and evolutionary biology. His research focuses on brain evolution and modeling neurological diseases using human induced pluripotent stem cells and brain organoids. His awards include the prestigious NIH Director's New Innovator Award & the NIH Replication Award, two Telly Awards for Excellence in Science Communication, among several others. Dr. Muotri earned a BSc in Biological Sciences and a Ph.D. in Genetics in Brazil. As a Pew Latin America Fellow at the Salk Institute, he pursued postdoctoral training in neuroscience and stem cell biology.

JOHN BOBBY GOULDING, MPHIL, PHD | 10:55 AM

Director, Corporate & Scientific Strategy | Fate Therapeutics Inc.

Off-the-Shelf and On-Target: How iPSC-Derived CAR T Cells Could Democratize Cell Therapy

John (Bobby) Goulding, MPhil, PhD, is Director of Corporate Strategy & Business Development at Fate Therapeutics, where he leads external partnering strategy, informs clinical positioning across oncology and autoimmune indications, and aligns next-generation platform innovation with clinical and commercial objectives. His work centers on building innovative, accessible, and economically viable cell-based products for broad, community-based patient populations. Previously Fate's Director of Immunology, he led R&D of multi-edited iPSC-derived cell therapies. He holds a PhD from Imperial College London, an MPhil in Bioscience Enterprise from Cambridge Judge Business School, and trained at UC San Diego and the La Jolla Institute.

DAN OLIVER, MBA | 11:30 AM

CEO & Co-founder | Rejuvenate Bio

Daniel Oliver is the CEO and co-founder of Rejuvenate Bio, a startup out of George Church’s Lab pioneering gene therapy solutions for age-related chronic diseases. Rejuvenate Bio’s initial focus on cardiovascular health involves a groundbreaking "one-shot" treatment for heart disease and obesity. The company’s validation in companion animals has led to partnerships with major players in animal health. Prior to Rejuvenate Bio, Daniel was awarded a Blavatnik Fellowship, and he co-founded Voxel8, named one of the 50 most innovative companies by the MIT Technology Review. Daniel received his MBA from Harvard Business School and has degrees in Mechanical Engineering and Business from the California Institute of Technology.

NICOLE PAULK, PHD | 11:30 AM

CEO & Founder | Siren Biotechnology

Nicole Paulk, PhD, is the CEO and Founder of Siren Biotechnology and has dedicated her career to advancing gene therapy. Nicole is a pioneer in the development of next-generation AAV platforms for gene transfer and gene editing, directed evolution for engineered capsids, and multi-omic approaches to interrogate AAV biology for countless diseases. Before founding Siren, Nicole held various positions in academia and industry, and most notably was faculty in the UCSF Department of Biochemistry & Biophysics. Nicole has a BS in Microbiology, a PhD in Gene Therapy from OHSU, and completed her Postdoctoral Fellowship and Instructorship in Human Gene Therapy at Stanford University before starting her lab at UCSF.

KAREN POTTER, PHD, JD | 11:30 AM

Partner | Morrison Foerster

Karen Potter, PhD, JD, is a nationally recognized patent attorney and leader of the Cell and Gene Therapy Patent Practice at Morrison Foerster in San Diego, California. With over 20 years of experience in life sciences intellectual property law, her practice focuses on patent strategy, prosecution, portfolio management, and strategic counseling for biotechnology and pharmaceutical companies worldwide. Her expertise includes cell and gene therapies, immunotherapies, vaccines, diagnostics, and protein therapeutics. Dr. Potter earned her PhD in cellular and molecular biology from Duke University and completed postdoctoral research at the La Jolla Institute for Allergy and Immunology.

KIRAN MUSUNURU, MD, PHD, MPH, ML, MRA | 1:00 PM

Professor for Translational Research | University of Pennsylvania
Developing and Deploying Personalized Gene-Editing Therapies

Kiran Musunuru, MD, PhD, MPH, ML, MRA, is the Barry J. Gertz Professor for Translational Research at the Perelman School of Medicine and Co-Director of the Penn Medicine/CHOP Orphan Disease Center. A practicing cardiologist and dedicated educator, his research focuses on developing gene-editing therapies for cardiovascular and metabolic diseases. Dr. Musunuru also serves as Editor-in-Chief of The American Journal of Human Genetics. In 2025, he co-developed a breakthrough, personalized CRISPR therapy for an infant with a rare metabolic disease. He was named among the 100 Most Influential People of 2026 by TIME magazine.

ALEXIS C. KOMOR, PHD | 1:50 PM

Associate Professor of Chemistry and Biochemistry | UC San Diego
Development of Precision Genome Editing Methods for Disease Modeling and Treatment

Alexis Komor, PhD, is an Associate Professor in the Department of Chemistry and Biochemistry at the University of California, San Diego. She received her BS in Chemistry from the University of California, Berkeley and her PhD from the California Institute of Technology. She completed postdoctoral training with David Liu at Harvard University, where she co-developed base editing, a genome editing technology that avoids double-stranded DNA breaks. Her research focuses on advancing precision genome editing technologies, understanding their mechanisms, and applying them to study disease-associated mutations.

BRIEANA BUCKLEY, PHARMD, MS | 2:10 PM

Senior Vice President, Development and Program Leadership | Editas Medicine

EDIT-401: A Potentially Transformative, Investigational In Vivo CRISPR Gene-Editing Medicine to Upregulate LDLR & Reduce Multiple Atherogenic Lipoproteins

Brieana Buckley, PharmD, MS, is Senior Vice President of Development and Program Leadership at Editas Medicine, where she leads program strategy, planning, and development activities across its therapeutic pipeline. With over 20 years of experience in the healthcare and biotechnology industries, her expertise spans all phases of clinical development. She has contributed to ten successful commercial product launches and has extensive experience navigating global healthcare systems to improve access to innovative therapies designed to address unmet medical needs and transform patient outcomes. She holds a PharmD from the University of Utah and an MS in Pharmacoeconomics from the University of Florida.

BRIAN P. HEAD, PHD | 3:05 PM

Professor of Anesthesiology & VA Research Career Scientist | UC San Diego & VA San Diego Healthcare System

Neuron-Targeted Caveolin Gene Therapy to Mitigate Neurodegenerative Proteinopathies

Brian Head, PhD, is a Research Career Scientist at the VA San Diego Healthcare System and Professor of Anesthesiology at the University of California San Diego. He has over two decades of experience in research focused on AAV-mediated gene therapies for neurodegenerative diseases, including Alzheimer's disease, amyotrophic lateral sclerosis, and TDP-43–related disorders. He was elected a Senior Member of the National Academy of Inventors in 2026. A gene therapy program he helped develop for ALS, which was acquired by Eikonoklastes Therapeutics received Investigational New Drug clearance from the U.S. Food and Drug Administration in 2025.

PRASHANT MALI, PHD | 3:25 PM

Professor of Bioengineering | UC San Diego

A Nature-Guided Approach for Engineering Targeted Delivery

Prashant Mali, PhD, is a Professor of Bioengineering at the University of California San Diego. His research lies at the intersection of synthetic biology and regenerative medicine, with a focus on developing gene- and cell-based therapeutic technologies. Dr. Mali earned his B.Tech. and M.Tech. degrees in Electrical Engineering from the Indian Institute of Technology Bombay, his PhD in Biomedical Engineering from Johns Hopkins University, and completed postdoctoral training in Genetics at Harvard Medical School. He has received numerous honors and was elected to the American Institute for Medical and Biological Engineering in 2022 and the National Academy of Inventors in 2023.

KATHERINE CYGNAR, PHD | 3:45 PM

Executive Director, Sensory and Lysosomal Biology | Regeneron Pharmaceuticals, Inc.

The CHORD Trial: An Update on the Efficacy and Safety of DB-OTO Gene Therapy for Profound Deafness in Children

Katherine Cygnar, PhD, is Executive Director of Sensory and Lysosomal Biology at Regeneron, where she leads research across auditory sciences, ophthalmology, and lysosomal disease. Her expertise spans multiple therapeutic modalities, including antibodies, siRNA, gene editing, AAV gene therapy, and combination approaches. Dr. Cygnar leads the development of therapies for sensory and lysosomal disorders, as well as immunomodulation tools and strategies for genetic medicines aimed at improving the safety and efficacy of gene therapies and increasing patient access.

SESSION CHAIRS

DONALD CLEVELAND, PHD

Distinguished Professor of Cellular and Molecular Medicine | UC San Diego

Session 1: Antisense Oligonucleotides for Rare Disorders

MASAYUKI KAI, PHD

Therapeutic Area Head (Hem/HemOnc), Research Strategy | Kyowa Kirin

Session 3: Ex Vivo Cell Platform for Safety Studies and Treatments for Rare Diseases and Cancer

BETTY CABRERA, MPH

Director of Operations, Gene Therapy Initiative | UC San Diego

Session 2: Industry Talk

Session 4: Panel on Navigating Bench to Bedside

NORIKO KANAYA, DVM, PHD

Principal Scientist | Nitto BioPharma

Session 5: In Vivo Gene Editing as an Emerging Technology

SHARIF TABEBORDBAR, PHD

Global Head of Cell and Gene Therapies | Novartis

Session 6: AAV Gene Therapy: Improved Capsids and Groundbreaking Applications

Acknowledgments

COMMUNITY PARTNERS

The **Friedreich's Ataxia Research Alliance (FARA)** is a non-profit dedicated to curing Friedreich's ataxia (FA) through research. FA is a genetic, progressive neuromuscular disease that affects an estimated 5,000 individuals in the U.S. and 15,000 worldwide. FARA grants and activities provide support for FA research, pharmaceutical/biotech drug development, clinical trials, and scientific conferences. FARA also serves as a catalyst between the public and scientific community to create worldwide exchanges of information that drive medical advances.

Connect is a mission-driven nonprofit that helps San Diego's innovation ecosystem work more like a true ecosystem. Founded in 1985, Connect supports life science and technology companies with curated programs, advising, visibility, and access to capital without taking equity. It convenes founders, investors, universities, corporations, and partners to strengthen pathways, proof, and platform across the region for long-term shared momentum.

French BioBeach is a San Diego-based biotech cluster. It connects American, French, and Francophone life science professionals, entrepreneurs, researchers, and companies working across the biotechnology and pharmaceutical sectors. Established in 2006, it bridges the San Diego biotech hub and France's innovation landscape, facilitating scientific exchange and collaboration. Our network comprises members from across industry and academia, with strong representation in gene therapy, rare diseases, and early-stage therapeutic development.

The Science and Technology Office promotes scientific and technological cooperation between France and the United States. Its mission is to foster researcher mobility, support academic and industrial partnerships, and strengthen collaborations between universities, research organizations, companies, and innovation ecosystems on both sides of the Atlantic.

UC San Diego
SANFORD STEM CELL INSTITUTE

The **Sanford Stem Cell Institute's Strategic Advisory Research Team (START)** supports researchers during the preclinical development of regenerative medicine and gene therapies. The START helps investigators by providing strategic, technical, and regulatory compliance guidance during the steps leading up to an initial human study. The START is also extremely proficient in advising the preparation multi-million dollar preclinical grant funding applications, including those of the California Institute of Regenerative Medicine. START activities directly facilitate FDA drug trial approval and transition to clinical trials at the UC San Diego Alpha Clinic.

UC San Diego
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The **Master's Program in Precision Medicine Therapeutics in Oncology** offered by the UC San Diego School of Medicine is designed to meet the growing demand for precision medicine training in the health care, research, and life sciences industry. This interdisciplinary program equips you with the skills to advance your career, personalize oncology care and drive innovation in precision medicine. Gain in-depth knowledge in: Genomics, Cancer Biology, AI, Bioinformatics, Regulatory Aspects, Drug Development and more.

ENJOYED TODAY'S EVENT?

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JOIN US AGAIN FOR DAY 2!

CLS x CIRM Cell & Gene Therapy Symposium
Thursday, September 17, 2026
The Conrad T. Prebys Auditorium @ Salk Institute

Visit the CLS exhibitor table or check-in table for more information or to register onsite

UPCOMING COMMUNITY EVENTS

Saturday, February 27, 2027 | 4-7 PM | Farmer & the Seahorse

The **San Diego Rare Disease Day** event is a special gathering designed to build community, make connections, and have fun. We unite patients and families, advocates, healthcare providers, and researchers to spread awareness, share stories, and support one another in the rare disease journey.

This event is 100% donor-funded, so we welcome any organization or individual interested in sponsoring to contact us. Please email us at **info@raresandiego.com** for more information.

How You Can Help:

- Empower Your Patients: Share this event with the families you serve so they can find local support and connection.
- Become a Sponsor: Support our 100% donor-funded initiative. to keep this event free for the rare disease community.

Thank You

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