2nd Annual Gene Therapy Initiative Symposium

THURSDAY,
September 18th
8:30AM - 5:30 PM

Salk Institute for Biological Studies
The Conrad T. Prebys Auditorium

UC San Diego
GENE THERAPY INITIATIVE

Acknowledgments

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MASTER OF ADVANCED STUDIES IN PRECISION MEDICINE THERAPEUTICS IN ONCOLOGY

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.

cystinosis RESEARCH FOUNDATION

The Cystinosis Research Foundation's mission is to find better treatments and a cure for cystinosis by investing in bench, clinical and translational research. CRF has raised more than $72 million and awarded 251 grants to advance cystinosis research. Our strategic research approach has led to two FDA approvals and supported multiple clinical trials. We have accomplished milestones and given hope to the cystinosis community that a better quality of life and a cure are within reach.

French BioBeach

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.

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*Network with attendees + raffle entry opportunity *details below*

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*To participate, simply complete the following **THREE** tasks by Sept 18.*

1. *Post about the symposium on your LinkedIn page and tag the "Gene Therapy Initiative at UC San Diego"*
2. *Share a photo on the GTI Symposium LinkedIn group of what networking at the event looks like for you.*
3. *Deposit your completed ticket the Raffle Box located at TABLE #4 in the Networking Zone during the Poster & Netorking Session*

No purchase necessary. One Entry Per Person. Task completion will be verified before announcing winner. Winner notified after the symposium via email and/or phone.

Program

8:30AM ***Welcome | Stephanie Cherqui, PhD & Gabriel Haddad, MD***

8:40AM ***Award Presentation to Theodore Friedmann, MD***

CELL AND GENE THERAPY FOR RARE DISEASES AND CANCER

Chairs: Stephanie Cherqui, PhD & Nayla Mumneh, MD

8:50AM ***Donald Kohn, MD | Keynote***
Distinguished Professor of Microbiology, Immunology & Molecular Genetics Pediatrics and Molecular & Medical Pharmacology UCLA, Director of the UCLA Human Gene and Cell Therapy Program
Hematopoietic Stem Cell Gene Therapy for Blood Cell Diseases

9:25AM ***Q&A***

9:35AM ***Mitchell Weiss, MD, PhD***
Arthur Nienhuis Endowed Chair in Hematology, St. Jude Children's Research Hospital
Ex Vivo Gene Editing for Sickle Cell Disease

9:55AM ***Dan Kaufman, MD, PhD***
Professor of Medicine, Vice Chief of the Division of Regenerative Medicine, Co-Director of the Sanford Advanced Therapy Center and Scientific/Medical Director of the Advanced Cell Therapy Laboratory, UC San Diego
Targeted Virus-Like Particles for In Vivo Immune Cell Engineering to Mediate Anti-Cancer Activity

10:15AM ***Travis Young, PhD***
Vice President of Biologics at Calibr-Skaggs Institute for Innovative Medicines, Scripps Research Institute
Modular, Switchable CAR-T Cell Platform in Oncology and Autoimmunity

10:35AM ***Q&A***

10:50AM ***Break***

THE PROMISE AND CHALLENGES OF GENE THERAPY

Chair: Farah Sheikh, PhD

11:05AM ***Chamindra Laverty, MD***
Professor of Neurosciences, Director of Multidisciplinary Neuromuscular Clinics, UC San Diego
Safety in Gene Therapy-A Neuromuscular Perspective

11:25AM ***Barry Byrne, MD, PhD***
Professor & Associate Chair of Pediatrics, Director of the Powell Gene Therapy Center, University of Florida
AAV Vector Safety Considerations in Gene Therapy

11:45AM ***Q&A***

12:00PM ***Lunch***

AAV GENE THERAPY FOR NEURODEGENERATIVE DISORDERS

Chair: Alysson Muotri, PhD

1:00PM ***Afternoon Remarks | Alysson Muotri, PhD***

1:05 PM ***Mark Tuszynski MD, PhD***
Professor of Neurosciences, Director of the Center for Neural Repair, UC San Diego
A Clinical Trial of AAV2-BDNF Gene Therapy for Alzheimer's Disease and Mild Cognitive Impairment

1:25 PM ***Wonkyu Ju, PhD***
Professor in Ophthalmology and Bioengineering, Hanna and Mark Gleiberman Chancellor's Endowed Chair in Glaucoma Research, University of California San Diego
Restoring AIBP Expression in the Retina Provides Neuroprotection in Glaucoma

1:45PM ***Eric Kelsic, PhD***
CEO & Cofounder at Dyno Therapeutics
Machine-Guided Design of AAV Capsids for Highly Specific CNS-IV Gene Delivery

2:05PM ***Q&A***

RARE DISEASE PATIENT PANEL

Chair: Betty Cabrera, MPH

Terry Pirovolakis
CEO & Founder of Elpida Therapeutics

2:20PM ***Caitlyn Barrett, PhD***
Director of the Milken Institute Science Philanthropy Accelerator for Research and Collaboration (SPARC)

James A. Levine, MD, PhD, MBA
Professor of Medicine, Mayo Clinic & President Fondation Ipsen Paris

2:50PM ***Q&A***

EMERGING BIOTECHS

Chair: Chris Mason, MD, PhD

3:05PM ***Ana Moreno, PhD***
Founder & CEO, Navega Therapeutics
Developing Epigenetic Gene Therapies for Intractable Chronic Pain Indications

3:15PM ***Courtney Young, PhD***
CEO & Co-Founder, MyoGene Bio
Development of a Gene Editing Therapy for Duchenne Muscular Dystrophy

3:25PM ***Q&A***

POSTER & NETWORKING SESSION | 3:35 PM

SOCIAL HOUR | 4:40PM

Speakers

Keynote Speaker | Donald Kohn, M.D. UCLA

Donald B. Kohn, M.D. is a Distinguished Professor at University of California Los Angeles in Microbiology, Immunology & Molecular Genetics; Pediatrics; and Molecular & Medical Pharmacology. A board-certified pediatrician with over 35 years of experience in pediatric bone marrow transplantation, his research focuses on gene therapy for blood cell diseases, including SCID and sickle cell disease, using autologous hematopoietic stem cells. Dr. Kohn has sponsored six investigator-initiated INDs for gene therapy clinical trials and has received numerous honors, including the Doris Duke Distinguished Clinical Scientist Award, the American Society of Gene and Cell Therapy's Outstanding Achievement Award, and the Society for Pediatric Research's Maureen Andrew Mentoring Award. He is a past president of the American Society of Gene and Cell Therapy and the Clinical Immunology Society and currently serves on the FDA Cellular, Tissue, and Gene Therapies Advisory Committee.

Caitlyn Barrett, Ph.D. MILKEN INSTITUTE

Caitlyn Barrett, Ph.D. is a Director on the Science Philanthropy Accelerator for Research and Collaboration (SPARC) team at the Milken Institute, where she partners with philanthropists to accelerate impact in biomedical research, including rare diseases. She has expertise in grant management, stakeholder engagement, and program analysis. Previously, she was Senior Director of Research and Programs at CureSearch for Children's Cancer and serves on the Board of the Coalition Against Childhood Cancer. Dr. Barrett earned her Ph.D. in Cancer Biology from Vanderbilt University and completed postdoctoral training in the Institute of Neurodegenerative Disease at the University of Pittsburgh

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Barry Byrne, M.D., Ph.D. UF UNIVERSITY of FLORIDA

Barry Byrne, M.D., Ph.D. is Associate Chair of Pediatrics and Director of the Powell Gene Therapy Center and Child Health Research Institute at the University of Florida. A clinician scientist and pediatric cardiologist, his work focuses on gene therapy for inherited muscle diseases such as Pompe disease, Friedreich's ataxia, and Duchenne muscular dystrophy. His team has pioneered AAV-based therapies and scalable AAV manufacturing methods. Dr. Byrne earned his M.D. and Ph.D. from the University of Illinois, trained at Johns Hopkins, and holds the Earl and Christy Powell University Chair in Genetics at the University of Florida. He also serves as Chief Medical Advisor and board member of the Muscular Dystrophy Association.

Wonkyu Ju, Ph.D. UC San Diego

Wonkyu "Daniel" Ju, Ph.D. is Professor of Ophthalmology and Bioengineering at the University of California San Diego holding the Hanna and Mark Gleiberman Chancellor's Endowed Chair in Glaucoma Research. His research explores mitochondrial dysfunction, neuroinflammation, and neuroprotection in glaucoma and Alzheimer's disease. He is a recipient of a 2024 Gene Therapy Initiative seed grant for an AAV gene therapy approach to glaucoma. Dr. Ju earned his Ph.D. in Anatomy from the Catholic University of Korea and completed a postdoctoral fellowship at Washington University in St. Louis where he investigated cellular and molecular mechanisms of cell death and neuroprotection in retinal ischemia and glaucoma.

Dan Kaufman, M.D., Ph.D.

Dan Kaufman, M.D., Ph.D. is Professor of Medicine, Vice Chief of the Division of Regenerative Medicine, and Clinical Director of the Cell and Regenerative Medicine service at the University of California San Diego. His lab pioneered the development of human iPSC-derived natural killer cells for cancer therapy now in clinical trials. He is a recipient of a 2024 Gene Therapy Initiative seed grant for developing in vivo immune engineering approaches as a novel cancer treatment. Dr. Kaufman also co-directs the Sanford Advanced Therapy Center and leads the Advanced Cell Therapy Laboratory translating cell-based therapies to patients. He earned his M.D. and Ph.D. from the Mayo Clinic Alix School of Medicine.

Eric Kelsic, Ph.D.

Eric Kelsic, Ph.D. is CEO of Dyno Therapeutics, where he has raised over $100M in VC financing, and built partnerships with leading gene therapy developers and technology companies such as Astellas, Roche, and NVIDIA. Previously, at Harvard's Wyss Institute, he led the development of Dyno's AI-powered capsid engineering platform in George Church's lab. There he measured the first comprehensive fitness landscape of the adeno-associated virus (AAV) capsid protein and co-discovered the AAV MAAP gene. Dr. Kelsic earned his Ph.D. in Systems Biology from Harvard and a B.S. in Physics from Caltech.

Chamindra Laverty, M.D.

Chamindra G. Laverty, M.D. is a Professor of Neurosciences at UC San Diego specializing in hereditary and acquired neuromuscular diseases where she directs adult and pediatric clinics. She is the principal investigator for 11 clinical trials in various muscular dystrophies and neuropathies. Her special interest is bringing disease modifying therapy including gene replacement therapy, cell therapy and anti-sense oligonucleotides, to her complex patients. In collaboration with colleagues, Dr. Laverty has described several new muscle diseases. Dr. Laverty launched and directs UCSD's ACGME-accredited Neuromuscular Medicine Fellowship. She completed her training at UCLA after earning her medical degree from Drexel University.

James A. Levine, M.D., PhD, MBA

James Levine M.D., Ph.D., MBA is President of Fondation Ipsen, a global science foundation advancing biotech innovation in rare diseases. With three decades at Mayo Clinic, he has authored 200+ publications, including in Science, Nature, NEJM, Lancet, and JAMA, and written four books published in 37 countries. An innovator with 100+ patents, he co-founded 35 companies and created scalable health solutions for 72 U.S. corporations. Recognized as Innovator of the Year by Minnesota, the World Trade Fair, and NASA, he has consulted for the U.S. President, State Department, and Army. His work focuses on biotech development and global health solutions, particularly in underserved regions worldwide. He earned business training in entrepreneurship at Harvard Business School.

Ana Moreno, Ph.D.

Navega Therapeutics

Ana Moreno, Ph.D. is founder and CEO of Navega Therapeutics, which develops AI-enabled epigenetic therapies for pain and inflammatory disorders. She has authored multiple high-impact research papers, holds six patents and received numerous prestigious awards and fellowships, including the Mitchell Max Award from the NIH Pain Consortium, the 2024 Biocom California Catalyst Award, the 2025 Transatlantic Connections Award, the 2025 Chancellor's Innovation Awards at UC San Diego, and is a 2025 Termeer Fellow. Dr. Moreno earned her M.S. and Ph.D. in bioengineering from UC San Diego and is active in mentoring and promoting diversity in STEM.

Terry Pirovolakis

ELPIDA THERAPEUTICS

Terry Pirovolakis co-founded CureSPG50 when confronted with his child's SPG50 diagnosis in 2019. Through successful fundraising, he led ground-breaking research, treating seven children within three years. Pirovolakis established Elpida Therapeutics, a corporation with a non-profit approach, focusing on gene therapies for ultra-rare conditions. Collaborating with industry leaders, Elpida targets SPG50, CMT4J, and plans to address four more diseases in 2025, utilizing profits to sustain programs. Pirovolakis extends his impact by supporting foundations, providing Gene Therapy 101 classes, and engaging in collaborations, all centered on the goal of saving as many children as possible.

Mark Tuszynski, M.D., Ph.D.

UC San Diego

Mark Tuszynski, M.D., Ph.D. is Distinguished Professor and Director of the University of California San Diego Center for Neural Repair and Founding Director of the Translational Neuroscience Institute. His research explores growth factors, stem cells, and bioengineering for neurodegenerative disease and injury. Dr. Tuszynski led the first human gene therapy trial for Alzheimer's disease and currently directs first-in-human clinical trials of BDNF gene therapy in Alzheimer's disease, and biomimetic nerve regeneration scaffolds in peripheral nerve injury. He has received more than 20 national research awards. Dr. Tuszynski earned his M.D. from the University of Minnesota, trained at Cornell University Medical Center, and received his Ph.D. from the University of California San Diego.

Mitchell Weiss, M.D., Ph.D.

St. Jude Children's Research Hospital

Mitchell Weiss, M.D.,Ph.D. is Chair of the Hematology Department at St. Jude Children's Research Hospital, where he leads the Divisions of Experimental and Clinical Hematology. A physician-scientist, he specializes in pediatric non-malignant blood diseases, with research focused on red blood cell biology and developing therapies for sickle cell disease and β-thalassemia. He has published over 180 papers, maintained continuous NIH funding since 2002, and is a member of the American Society for Clinical Investigation, the Association of American Physicians, and the National Academy of Medicine. Previously, he was Professor of Pediatrics with tenure at UPenn and CHOP. He earned his M.D. and Ph.D. from the University of Pennsylvania and completed training at Boston Children's Hospital, Dana-Farber, and Harvard.

Courtney Young, Ph.D.

MyoGene Bio

Courtney Young, Ph.D. is co-founder and CEO of MyoGene Bio.Inspired by her cousin who was diagnosed with Duchenne muscular dystrophy (DMD), she helped develop a single platform for gene editing to impact disease progression among half of all patients. Dr. Young holds five patents and has received numerous awards and fellowships including the NSF Graduate Research Fellowship, the Charles E. and Sue K. Young Graduate Student Award, the UCLA Jules Brenner Award, MIT Technology Review's 35 under 35 Innovators in Biotechnology, and Biocom's Catalyst Award. She earned her Master of Research at University College London, and her Ph.D. in Molecular Biology from UCLA and has published extensively.

Travis Young, Ph.D.

Calibr-Skaggs Institute for Innovative Medicines | Scripps Research

Travis Young, Ph.D. is Vice President of Biologics at Calibr-Skaggs Institute for Innovative Medicines at Scripps Research. He was a founding member at Calibr and serves as lead investigator on multiple clinical programs and bench-to-bedside antibody & cellular therapy-based programs. Dr. Young has built an experienced cell therapy team at Scripps capable of translating unique cell therapies from concept to first in human clinical. His work has been highly awarded, attracting major support, and resulted in numerous publications and patents with >3000 citations in the past 5 years. Dr. Young earned his Ph.D. in Chemical Biology from Scripps and completed a postdoc at Harvard Medical School.

SATISFACTION SURVEY

Your feedback matters—let us know how we can make future GTI events even better.

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Acknowledgments

Community Partners

The Sanford Advanced Therapy Center (ATC) at UC San Diego's Sanford Stem Cell Institute houses the Advanced Cell Therapy Laboratory (ACTL), a state-licensed GMP facility launched in 2018. The ACTL produces cell therapies for early-phase clinical trials, develops compliant processes, and partners with academia and industry to expand patient access. Services include GMP manufacturing, process development, risk assessment, preclinical consultation, product characterization, cryogenic storage, final formulation, and regulatory support, ensuring FDA standards are met while advancing cell therapy innovation.

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.

The French American Chamber of Commerce is a non-profit institution that empowers companies to reach their full potential. Through our expertise in business needs and regional regulation laws, as well as expansive network of U.S and French industry leaders as well as government officials, we help our members gain tremendous momentum to grow their business. Our mission is to enable and empower businesses by providing them with crucial resources, including but not limited to an extensive network, advice on best business practices, potential client prospection and much more.

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