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.

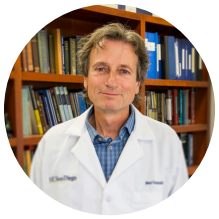
Navega Therapeutics

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.
