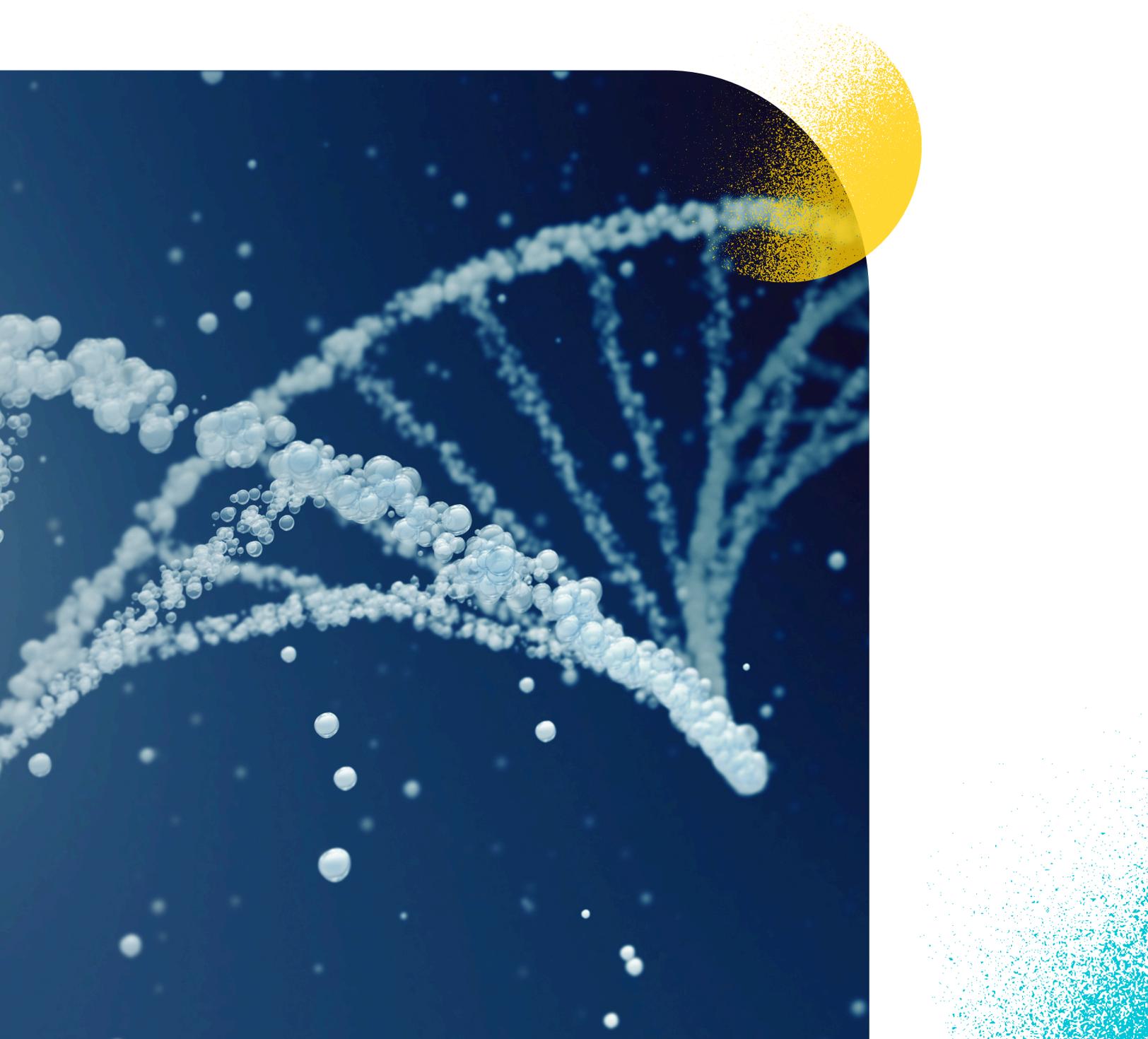


The UC San Diego Gene Therapy Initiative

COLLABORATING TOWARD BREAKTHROUGHS



THANK YOU

One year ago, thanks to a transformational gift from the Nancy and Geoffrey Stack Foundation, UC San Diego cemented its status as a leading hub for gene therapy research. The establishment of the UC San Diego Gene Therapy Initiative is a powerful reflection of the commitment and vision of our supporters. You understand the incredible potential of gene therapy to change the course of the lives of those with diseases once considered incurable – it already has. We are so grateful for this opportunity to commit our expertise and resources toward this effort.

This year we formed an exceptional advisory committee to help guide our efforts as we pursue our mission to drive strategic innovations and collaborations that will advance gene therapy. We hosted our inaugural symposium which attracted top gene therapy experts from across the region and the country. We also awarded our first round of seed grants to promising projects that aim to push the boundaries of our understanding of gene therapy and explore how it can be applied to a variety of conditions. Our leadership has been raising national awareness of the GTI mission via the 2024 Global Genes RARE Drug Development Symposium, the 27th Annual Meeting of the American Society for Gene and Cell Therapy, and the 2024 Thermo Fisher Cell Biology North America Meeting.

We are proud to share with you what this initiative has accomplished in its first year. Gene therapy is a sophisticated, highly personalized form of treatment that technology has only recently allowed humanity to harness, and we are leading the effort in this important field. Thank you for your philanthropic partnership in this important work.

Sincerely,

Stephanie Cherqui, PhD
Professor, Department of Pediatrics
UC San Diego School of Medicine
Chair, Cystinosis Stem Cell and Gene Therapy Consortium

Alysson Muotri, PhD
Professor, Departments of Pediatrics and
Cellular and Molecular Medicine
UC San Diego School of Medicine



Meet the Gene Therapy Initiative Advisory Board

Our newly formed board includes researchers, physician scientists, adult and pediatric clinicians, entrepreneurs and pharmaceutical executives, each of whom is focused on developing or delivering cell and gene therapies and committed to finding cures and treating those with unmet medical needs.

Eric D. Adler, MD
Clinical Professor, Dpt. of Medicine
Dir., Strauss Center for Cardiomyopathy

Shyamanga Borooah, MBBS, PhD
Physician-Scientist, Shiley Eye Institute

Stephanie Cherqui, PhD
Professor, Department of Pediatrics
Co-Director, Gene Therapy Initiative

Don Cleveland, PhD
Professor, Department of Medicine
Chair, Cellular & Molecular Medicine

Chamindra G. Lavery, MD
Professor, Dpt. of Neurosciences
Director, Multidisciplinary
Neuromuscular Clinics

Marsala Martin, MD
Professor, Anesthesiology

Chris Mason, MD, PhD
Clinical Professor of Cell and Gene
Therapy, University College London

Alysson Muotri, PhD
Professor, Department of Pediatrics
Co-Director, Gene Therapy Initiative
and Stanford Stem Cell Institute

Farah Sheikh, PhD
Professor, Department of Medicine

Mark H. Tuszynski, MD, PhD
Professor, Department of Medicine
Director, Center for Neural Repair

INAUGURAL SYMPOSIUM SPARKS CONNECTIONS

The UC San Diego Gene Therapy Initiative (GTI) held its inaugural symposium on September 19, attracting over 200 gene therapy researchers, clinicians, industry representatives and patient advocates. Attendees shared discoveries in gene therapy and discussed potential partnerships and future opportunities to advance gene therapy products from the lab to the clinical setting.

Our very first symposium represents a step forward in advancing gene therapy breakthroughs through resource sharing, collaboration and partnerships. Special thanks to the California Institute for Regenerative Medicine for providing platinum-level support for this event. Plans are in the works for the 2025 symposium; for more information, please contact GTI_admin@health.ucsd.edu.

GRANTS SPUR RESEARCH ACROSS DISCIPLINES

The UC San Diego Gene Therapy Initiative provided its first round of seed funding to twelve grant recipients at UC San Diego. There was an enthusiastic response to the call for proposals across the campus, with 10 different departments represented among 35 applicants. The awarded projects were selected among a competitive pool of very innovative and high-caliber research proposals that investigated a variety of different gene therapy technologies with applications to over 20 different diseases. Recipients discussed their work at the GTI Symposium. We look forward to sharing their progress and findings along with the announcement of our next round of funding recipients.

SEED GRANT RECIPIENTS AND PROJECTS

Angels Almenar-Queralt, Pediatrics

Assessing the functional impact of an Alzheimer's disease protective SNP in the CD33 microglial gene

Steven Dowdy, Cellular and Molecular Medicine

Tackling the siRNA and ASO therapeutics delivery problem with new chemistry

Brian Head, Anesthesiology

Proof-of-concept hominid-suitable AAV.CAP-Mac to deliver neuron-targeted caveolin gene therapy in MS

Vivian Hook, Neuroscience and Pharmacology

Capthepsin B siRNA gene silencing for development of a new therapeutic approach for Alzheimer's disease (AD), traumatic brain injury (TBI) and related neurological diseases

Wonkyu Ju, Ophthalmology

A-kinase anchoring protein 1-mediated neuroprotection in glaucoma

Dan Kaufman, Regenerative Medicine

Targeted virus-like particles for in vivo engineering of lymphocytes for improved anti-tumor activity

Ester Kwon, Bioengineering

Lipid nanoparticle engineering for in vivo immune cell cancer therapy

Loren Looger, Neuroscience

De novo design of functional AAV capsids and AAV serotypes with increased payload limit using computational protein design and machine learning

Prashant Mali, Bioengineering

Engineering and validation of novel AAV variants with enhanced tissue tropism in non-human primates

Alysson Muotri, Pediatrics and Cellular and Molecular Medicine

Zika virus as a novel gene therapy delivery system

Thomas Rogers, Medicine

(bnAb)-Engineered B cell response rescue (BEBRR) in immunocompromised systems

Gene Yeo, Cellular and Molecular Medicine

Novel RNA-targeting therapeutic strategy for ALS



The 2024 UC San Diego Gene Therapy Initiative Symposium was held in September at the Sanford Consortium in La Jolla, California.

WELCOME

BETTY CABRERA, DIRECTOR OF ENGAGEMENT AND OPERATIONS

The Gene Therapy Initiative (GTI) is excited to welcome Betty Cabrera to the team. A proud UC San Diego alumna, Betty brings over 20 years of experience in research development at her alma mater and more than a decade of expertise in gene therapy. As the GTI Director of Engagement and Operations, she will leverage her extensive knowledge and network in regenerative medicine, clinical research and patient interaction to advance our leading gene therapy program.



In her previous position as Director of Clinical Research for the Alpha Clinic at UC San Diego, Betty helped create and manage one of the first clinical research units in the state dedicated to building infrastructure for regenerative medicine clinical trials. She has held roles as a trial coordinator and patient registry curator, which gave her a profound appreciation for the needs and hopes of patients with rare genetic diseases and their families.

CONGRATULATIONS

NANCY AND GEOFFREY STACK RECEIVE UC SAN DIEGO CHANCELLOR'S MEDAL

On March 23, UC San Diego Chancellor Pradeep K. Khosla presented the 2023 Chancellor's Medal to Nancy and Geoffrey Stack in recognition of their 20 years of transformational support for research into rare diseases. Beyond supporting the launch of the UC San Diego Gene Therapy Initiative, the Nancy and Geoffrey Stack Family Foundation created the Cystinosis Research Foundation in 2003 as both a catalyst for cystinosis research and an inspiration for other families dealing with rare diseases.



Spurred by their daughter's cystinosis diagnosis when she was just a baby, the couple is committed to empowering researchers to pursue novel ideas that can lead to a cure. The foundation's support to UC San Diego helped propel research into improved treatment options for cystinosis, including the development of a slow-release medication to manage the disease.

CONTACT US

To learn more about the UC San Diego Gene Therapy Initiative, visit genetherapy.ucsd.edu or contact us at GTI_admin@health.ucsd.edu or 858-822-4735. To make a gift or discuss supporting our work, visit giving.ucsd.edu or contact Rachel Stroud Hunsinger at 503-477-2769 or rstroud@ucsd.edu.