Symposium Program

September 19, 2024

La Jolla, CA

PROGRAM

07:30AM — **Breakfast & Registration**

08:30 AM — **Welcome & Introduction - GTI Video**
Stephanie Cherqui, PhD, Department of Pediatrics, UC San Diego
Nancy and Geoffrey Stack Family Foundation
Theodore Friedmann, MD, PhD, Department of Pediatrics, UC San Diego

SESSION 1: EX VIVO HEMATOPOIETIC STEM CELL GENE THERAPY: BROAD APPLICATIONS AND THERAPEUTIC POTENTIAL

CHAIR: CHRIS MASON, MD, PHD

08:55 AM — **Hematopoietic stem cell-based cell and gene therapy beyond monogenic diseases - KEYNOTE**
Masayuki Kai, PhD, VP, Head of Research, Kyowa Kirin, Inc.

09:40 AM — **From cystinosis to Friedreich's ataxia and Alzheimer's disease: lessons learned treating a rare metabolic disease using ex vivo gene therapy**
Stephanie Cherqui, PhD, Department of Pediatrics, UC San Diego

10:10 AM — **BREAK**

SESSION 2: AAV-MEDIATED GENE THERAPY: CHALLENGES, INNOVATIONS AND CLINICAL APPLICATIONS

CHAIR: CHAMINDRA LAVERTY, MD

10:25 AM — **Redefining the landscape of arrhythmogenic cardiomyopathy: from mechanistic understanding to lifesaving interventions**
Farah Sheikh, PhD, Department of Medicine, UC San Diego

10:50 AM — **Neuron-targeted caveolin gene therapy to mitigate neurodegeneration**
Brian Head, PhD, Professor, Department of Anesthesiology, UC San Diego

11:05 AM — **Design and evolution of new AAV serotypes with expanded functionality for gene therapy**
Loren Looger, PhD,Department of Neurosciences, UC San Diego

11:20 AM — **Onasemnogene Abeparvovec-xioi for spinal muscular atrophy: bench to bedside**
Nayla Mumneh, MD, Executive Medical Director, NeuroMuscular, Novartis Pharmaceuticals Corporation

11:45 AM — **Q&A Panel**

12:00 PM — **LUNCH/SCIENTIFIC POSTER SESSION**

01:00 PM — **Afternoon Session Introduction**
Gaby Haddad, MD, Chair of Pediatric, UC San Diego
Alysson Muotri, PhD, Department of Pediatrics, UC San Diego

SESSION 3: TESTING GENE THERAPIES IN INDUCED PLURIPOTENT STEM CELL MODELS

CHAIR: MARTIN MARSALA, MD

01:15 PM — **Brain organoids as a pre-clinical model for neurological conditions**
Alysson Muotri, PhD, Department of Pediatrics, UC San Diego

PROGRAM

01:40 PM **CRISPR introduction of an amyloid plaque-protecting microglial gene**
Angels Almenar-Queralt, PhD,Pediatrics, UC San Diego

01:55 PM **Q&A Panel**

SESSION 4: RESOURCES & SUPPORT FOR THE DEVELOPMENT OF GENE THERAPY STRATEGIES FOR RARE DISEASES
CHAIR: BETTY CABRERA, MPH

02:05 PM **The power of patient advocacy: passion, determination and collaboration**
Nancy Stack, President, Cystinosis Research Foundation, Patient Advocate

02:15 PM **The Platform Vector Gene Therapy (PaVe-GT) program at NIH, a large collaboration among NCATS, National Human Genome Research Institute (NHGRI), and the National Institute for Neurological Disorders and Stroke (NINDS)**
Rodica Stan, PhD, Senior Scientific Project Manager, Therapeutic Development Branch, Division of Preclinical Innovation, National Center for Advancing Translational Sciences (NCATS), NIH

02:25 PM **Advancing cell and gene therapies in California and beyond**
Lisa Kadyk, PhD, Associate Director - Therapeutics Development
California Institute for Regenerative Medicine (CIRM)

02:35 PM **Moderated Panel**

03:05 PM **BREAK**

SESSION 5: THERAPEUTIC ADVANCES IN RNA TECHNOLOGIES FOR THE TREATMENT OF RARE AND ULTRA-RARE DISEASES
CHAIR: SHYAMANGA BOROOAH, MD, PHD

03:20 PM **Advancing novel precision medicine therapies: AI enabled antisense oligonucleotide design for treatment of an ultra-rare disorder**
Nicole Coufal, MD, PhD,Pediatrics, UC San Diego

03:45 PM **Vectorized RNA-targeting therapeutics**
Gene Yeo, PhD, MBA, Cell and Molecular Medicine, UC San Diego

04:10 PM **Developing RNA-therapeutics for neurological diseases**
Holly Kordasiewicz, PhD, Vice President Neurology Research, Ionis Pharmaceuticals, Inc.

04:35 PM **Q & A Panel**

04:50 PM **Summary and Concluding Remarks**
Alysson Muotri & Stephanie Cherqui, Co-Directors of GTI

05:00 PM **Networking Hour/ Scientific Poster session**

Speakers

Masayuki Kai is Vice President and Head of Research at Kyowa Kirin, Inc. The company's portfolio spans treatments for nephrology, oncology, neurology, and immunology indications. Earlier this year, it acquired Orchard Therapeutics, a gene therapy pioneer in treatments involving hematopoietic stem cells. Masayuki is a leader in the enterprise that seeks to expand applications of this approach to meet the unmet needs of those affected by devastating genetic and other severe diseases.

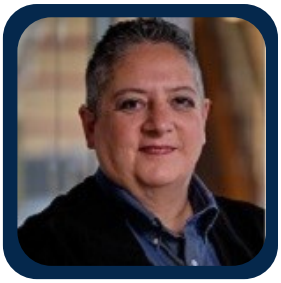
Stephanie Cherqui is Professor in the Department of Pediatrics at UC San Diego recognized for her gene therapy work, specifically in genetic disorders using stem cells. Named as a top academic entrepreneur by BIOS, she is renowned for her bench-to-bedside contributions to cystinosis research. Her recent work expands applications of ex vivo gene modified hematopoietic stem cells to other indications such as Alzheimer's disease.

Farah Sheikh's gene therapy research includes pioneering human-relevant mouse models, uncovering new molecular targets, and designing a gene therapy treatment for arrhythmogenic cardiomyopathy, currently in a Phase 1/2 clinical trial with LEXEO Therapeutics Inc. She has launched successful gene therapy start-ups and maintains industry partnerships.

Brian Head is dual appointment Professor of Anesthesiology at UC San Diego and a Research Career Scientist awardee at the VA San Diego Healthcare System. He has 18 years of experience testing AAV-mediated gene therapy technologies and interventions that afford neuroprotection against trauma and neurodegenerative disease models of Alzheimer's disease (AD) and amyotrophic lateral sclerosis (ALS).

Loren Looger is a Professor of Neurosciences at UC San Diego and a Howard Hughes Medical Institute Investigator. His lab combines protein engineering, molecular modeling, and directed evolution to develop biology tools that advance basic science and medicine. He engineers new viral serotypes with improved properties for use in neuroscience and gene therapy.

Speakers



Nayla Mumneh is board certified in Allergy and Immunologyand has held several positions at Novartis Pharmaceuticals,including US medical affairs medical director in the respiratory unit, global medical affairs senior medical director working on Zolgensma in spinomuscular atrophy (SMA), and is now executive medical director in US Medical Affairs at Novartis, Neuroscience leading the medical team working on neuromuscular assets.

Alysson Muotri is a professor at the Departments of Pediatrics and Cellular & Molecular Medicine at UC San Diego. He is also the Director of the Sanford Stem Cell Education and Integrated Space Stem Cell Orbital Research (ISSCOR) and Co-Director of the Gene Therapy Initiative. His research focuses on brain evolution and modeling neurological diseases using human induced pluripotent stem cells and brain organoids.

Angels Almenar-Queralt is a Project Scientist in the Department of Pediatrics at UC San Diego with over a decade of experience as a stem cell neurobiologist. Her research focuses on investigating the underlying mechanisms of neurological disorders, including Alzheimer's disease and rare neurodevelopmental conditions, and guiding the development of effective therapies using advanced and reliable hiPSC-derived neural systems.

Nancy Stack is founder and president of the Cystinosis Research Foundation, which supports medical research including stem cell research seeking a cure for the rare disease cystinosis. The Foundation has funded over 238 research studies and fellowships, one of which led to the to the first stem cell and gene therapy clinical trial for cystinosis in the United States.

Rodica Stan is a Senior Scientific Project Manager within NCATS' Division of Preclinical Innovation where she manages translational projects focused on gene therapy and small-molecule projects for rare and ultra-rare diseases. She helps steward Investigational New Drug–enabling studies by supporting teams of NIH internal and external collaborators, contract research organizations, and NCATS scientists and administrative personnel.

Speakers

Lisa Kadyk is Associate Director of Therapeutics Development at CIRM, where she is responsible for managing a diverse portfolio of translational, IND-enabling, and clinical trial stage awards, as well as contributing to strategic planning within CIRM. Previously, Lisa worked over twelve years in biotech, at Exelixis, Inc. and Axys Pharmaceuticals, primarily in target discovery and validation and alliance management. Lisa earned a Ph.D. in Genetics from the University of Washington in the lab of Dr. Lee Hartwell and did postdoctoral training at the University of Wisconsin with Dr. Judith Kimble.

Nicole Coufal is Associate Professor in the Department of Pediatrics at UC San Diego. She is a physician scientist who cares for critically ill children in the pediatric ICU and her research aims to understand the role of microglia in neurodevelopment and their contribution to common and rare neurodevelopmental disorders.

Gene Yeo is an Endowed Chair and Professor of Cellular and Molecular Medicine at UC San Diego, Director of the Center for RNA Technologies and Therapeutics, and Director for the Sanford Stem Cell Institute's Innovation Center. He is a computational and experimental scientist who studies how RNA processing is regulated by RNA binding proteins in development and disease. Gene has founded and advised several biotech companies.

Holly Kordasiewicz is Senior Vice President of Neurology Research at Ionis Pharmaceuticals, a company that specializes in RNA therapeutics. Dr. Kordasiewicz leads a team focused on identifying antisense oligonucleotide therapeutics for currently untreatable neurologicaldiseases, including drugs now in clinical trials for ALS, Alzheimer's disease, and Parkinson's disease.

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