UC San Diego
GENE THERAPY INITIATIVE

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**



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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***