

## Program Schedule

Friday, April 14, 2023

8:00 – 8:10 *Welcome* (10)  
**Gabriel Haddad, MD**  
Distinguished Professor of Pediatrics and Neurosciences  
Chairman of Pediatrics & Vice Dean, Children's Academic Programs  
University of California, San Diego  
Physician-In-Chief & Chief Scientific Officer  
Rady Children's Hospital – San Diego

8:10 – 8:40 *Keynote Address: "Vision and Potential Collaborations with the Skaggs School of Pharmacy and Pharmaceutical Sciences"* (30)  
(Introduction by Gabriel Haddad, MD)  
**Brookie Best, PharmD, MAS**  
Dean  
UC San Diego | Skaggs School of Pharmacy and Pharmaceutical Sciences

### NOVEL THERAPIES, ASOs & GENE THERAPY

*Moderator Welcome: Tariq Rana*

(8:40-8:45 – Clinical Relevance)

8:45 – 9:15 *"Unraveling the Biological Properties of Adeno-Associated Viruses for Enhanced Gene-Based Therapeutics"* (30)  
**Mark Kay, MD, PhD**  
Dennis Farrey Family Professor  
Departments of Pediatrics and Genetics  
Head, Division of Human Gene Therapy  
Stanford University

9:15 – 9:45 *"Development of Gene Therapy for Artemis Deficient Severe Combined Immunodeficiency"* (30)  
**Jennifer Puck, MD**  
Professor of Pediatrics  
Division of Allergy/Immunology and Blood and Marrow Transplantation  
University of California San Francisco School of Medicine  
and UCSF Benioff Children's Hospital San Francisco, CA

- 9:45 – 10:15    ***“Novel Therapies, ASOs & Gene Therapy: Hemoglobinopathies”***    (30)  
**Mark Walters, MD**  
Professor & Chief, Hematology Division  
Dept. of Pediatrics, UCSF School of Medicine  
Jordan Family Director, Blood & Marrow Transplant Program  
UCSF Benioff Children’s Hospital, Oakland
- 10:15 – 10:45    ***“Scaling CRISPR to the Challenge of N=1 Genetic Disease  
in an Academic/Nonprofit Setting”***    (30)  
**Fyodor Urnov, PhD**  
Professor of Molecular Therapeutics, MCB Department, UC Berkeley  
Scientific Director, Innovative Genomics Institute
- 10:45 – 11:00    ***B R E A K***    (15)
- ENVIRONMENTAL SCIENCE, CLIMATE CHANGE & HEALTHCARE**  
**Moderator Welcome: Tina Chambers**  
(11:00-11:05 – Clinical Relevance)
- 11:05 – 11:35    ***“Health Outcomes Across the Lifespan Following Extreme  
Weather Events: Mitigating and Adapting to Climate Change”***    (30)  
**Julia Gohlke, PhD**  
Associate Professor, Department of Population Health Sciences  
Virginia Tech
- 11:35 – 12:05    ***“Measuring the Timeline of Infant/Childhood Environmental  
Exposures through Deciduous (Baby) Teeth”***    (30)  
**Manish Arora, BDS, MPH, PhD, FICD**  
Professor and Vice Chair  
Division Director, Environmental Health  
Department of Environmental Medicine and Public Health  
Icahn School of Medicine at Mount Sinai
- 12:05 – 12:35    ***“Oceans and Human Health: A New Frontier”***    (30)  
**Jack Gilbert, PhD**  
Associate Vice Chancellor for Marine Science  
Professor, Department of Pediatrics and Scripps Institute of Oceanography  
University of California, San Diego
- 12:35 – 1:50    ***L U N C H & Poster Session***    (75)  
**Poster Session – Room 1900**  
***Please see booklet for abstracts***

1:50 – 2:30 ***Closing Keynote: “Translating a Trillion Points of Data into Therapies, Diagnostics, and New Insights into Disease”*** (40)  
(Introduction by Gabriel Haddad, MD)

**Atul Butte, MD, PhD**

Priscilla Chan and Mark Zuckerberg Distinguished Professor  
Director, Bakar Computational Health Sciences Institute  
University of California, San Francisco  
Chief Data Scientist, University of California Health

## **ORGANOIDS & DEVELOPMENTAL DISEASES**

***Moderator Welcome: Alysson Muotri***

(2:30-2:35 – Clinical Relevance)

2:35 – 3:05 ***“Brain Organoid Models of Genetic Developmental and Epileptic Encephalopathies”*** (30)

**Jack Parent, MD**

William J Herdman Professor of Neurology and Research Professor  
Michigan Neuroscience Institute, University of Michigan

3:05 – 3:35 ***“Generation and Utility of Regionalized Human Brain Organoids”*** (30)

**In-Hyun Park, PhD**

Associate Professor of Genetics  
Yale Stem Cell Center, Yale University

3:35 – 4:05 ***“Distinct Neural Oscillation Activities and Deficits in Cortex and Hippocampus Assembloid Models of Genetic Epilepsy”*** (30)

**Bennett Novitch, PhD**

Professor, Department of Neurobiology  
David Geffen School of Medicine at University of California, Los Angeles

4:05 – 4:20 ***Closing Remarks and Award Winners Announced*** (15)

4:20 – 6:00 ***Poster Session – Room 1900***

***Please see booklet for abstracts***