

CELL GENE

M E E T I N G O N T H E M E S A

DAY 2
PF

PUBLIC FORUM

THURSDAY | OCTOBER 4, 2018
SANFORD CONSORTIUM FOR REGENERATIVE MEDICINE

5:45pm | PUBLIC FORUM OPENS

6:00pm – 6:45pm

FEATURED PRESENTATION: REJUVENATING STEM CELL FUNCTION AND MUSCLE STRENGTH: NO PAIN, NO GAIN!

Featured Speaker:

Helen M. Blau, Ph.D., Donald E. and Delia B. Baxter Foundation Professor; Director, Baxter Laboratory for Stem Cell Biology, Stanford University

6:45pm – 7:30pm | RECEPTION AND NETWORKING

Sponsored by Mesoblast

7:30pm | PUBLIC FORUM CLOSES

DAY
3

SCIENTIFIC SYMPOSIUM

FRIDAY | OCTOBER 5, 2018
SALK INSTITUTE FOR BIOLOGICAL STUDIES

7:00am – 8:00am | REGISTRATION AND BREAKFAST

Sponsored by Polyplus-transfection

8:00am | GENERAL SESSION

8:00am – 8:15am

WELCOME REMARKS

Speaker:

Alysson Muotri, Ph.D., Chair, Scientific Symposium Steering Committee; Co-Director, Stem Cell Program; Professor, Department of Pediatrics and Cellular and Molecular Medicine, UC San Diego

8:15am – 8:55am

SELF-ORGANIZING SYNTHETIC HUMAN EMBRYOS AND ORGANOIDS TOWARDS CURING HUNTINGTON'S DISEASE

Sponsored by Homology Medicines

Keynote Speaker:

Ali Brivanlou, Ph.D., Robert and Harriet Heilbrunn Professor; Head, Laboratory of Stem Cell Biology and Molecular Embryology, The Rockefeller University

8:55am – 10:15am

PANEL: EX VIVO GENE THERAPY: USING BLOOD STEM CELLS TO TREAT GENETIC DISORDERS

Gene therapy is now a therapeutic reality for some terminal or severely disabling disorders. Ex vivo hematopoietic stem cell gene therapy has the critical advantage to turn the cells into widespread delivery vehicles to obtain stable and sustained expression of a defective protein in all appropriate tissues after a single systemic infusion. This session will highlight the potential of this approach in different disorders

illustrating the technologies for hematopoietic stem cell gene-correction and the engagement of stem cells to prevent tissue degeneration.

Chair / Introduction By:

Stephanie Cherqui, Ph.D., Associate Professor, Department of Pediatrics, Division of Genetics, UC San Diego

Clinical Translation of Hematopoietic Stem Cell Gene Therapy for Cystinosis, Mechanism of Action and Other Applications

Stephanie Cherqui, Ph.D., Associate Professor, Department of Pediatrics, Division of Genetics, UC San Diego

Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy

Florian Eichler, M.D., Director, Center for Rare Neurological Diseases; Associate Professor, Neurology, Harvard Medical School; Assistant in Neurology, Massachusetts General Hospital

Genome Editing of Hematopoietic Stem Cells to Treat Human Genetic Diseases

Matthew Porteus, M.D., Ph.D., Associate Professor, Pediatrics – Stem Cell Transplantation and Regenerative Medicine, Stanford University

10:15am – 10:40am | MORNING BREAK

Sponsored by PeperoTech and WiCell

10:15am – 10:40am | POSTER VIEWING

Sponsored by Brammer Bio

10:40am – 12:00pm

PANEL: ADVANCED THERAPIES FOR SKELETAL MUSCLES

This panel will focus on the central role of muscle stem cells (MuSC) in skeletal muscle homeostasis and repair. Speakers will discuss how the MuSC compartment changes in the context of aging and muscle degenerative diseases and how they interact with the tissue microenvironment, as these findings reveal novel potential tools/targets to promote MuSC function and tissue repair. In addition, the panel will also discuss novel translational approaches to generate myogenic progenitors for cell-based therapies for muscle diseases.

Chair / Introduction By:

Alessandra Sacco, Ph.D., Associate Professor, Development, Aging and Regeneration Program; Associate Dean of Curriculum, Graduate School of Biomedical Sciences, Sanford Burnham Prebys Medical Discovery Institute

Focus on Muscle Stem Cell Aging

Andrew Brack, Ph.D., Associate Professor, Orthopaedic Surgery Research, Department of Orthopaedic Surgery, UC San Francisco

From Skin to Skeletal Muscle: A Potential for Autologous Transplantation in Muscular Dystrophies

Rita Perlingeiro, Ph.D., Lillehei Professor in Stem Cell and Regenerative Cardiovascular Medicine, Lillehei Heart Institute; Professor of Medicine, Cardiovascular Division, University of Minnesota

Cellular and Molecular Responses of Skeletal Muscle to Homeostatic Perturbations in Health and Disease

Pier Lorenzo Puri, M.D., Ph.D., Professor, Development, Aging and Regeneration Program, Sanford Burnham Prebys Medical Discovery Institute

12:00pm – 1:15pm | LUNCH

Sponsored by the Sanford Stem Cell Clinical Center at UC San Diego Health

12:00pm – 1:15pm | POSTER VIEWING

Sponsored by Brammer Bio

1:15pm – 2:35pm

PANEL: GENE THERAPY FOR NEURODEGENERATIVE DISEASES

This session will provide an in-depth update on clinical and preclinical programs of AAV9 gene therapy for spinal muscular atrophy, antisense oligonucleotide therapy for ALS and growth factor gene therapy for Alzheimer's disease.

Chair / Introduction By:

Mark Tuszynski, M.D., Ph.D., Director, Center for Neural Repair; Professor, Department of Neurosciences, UC San Diego

Translation of Gene Therapeutics in Neurological and Neuromuscular Diseases

Brian Kaspar, Ph.D., Principal Investigator, Center for Gene Therapy; Associate Professor, Department of Pediatrics and Department of Neuroscience, The Ohio State University; Chief Scientific Officer, AveXis

Making Sense of Antisense: ASO Therapy Development for C9orf72 Amyotrophic Lateral Sclerosis and Frontotemporal Dementia

John Ravits, Ph.D., Professor of Clinical Neuroscience, UC San Diego

Growth Factor Gene Therapy for Alzheimer's Disease

Mark Tuszynski, M.D., Ph.D., Director, Center for Neural Repair; Professor, Department of Neurosciences, UC San Diego

2:35pm – 3:00pm | **AFTERNOON BREAK**

Sponsored by PeproTech and WiCell

2:35pm – 3:00pm | **POSTER VIEWING**

Sponsored by Brammer Bio

3:00pm – 4:20pm

PANEL: USING STEM CELLS TO STUDY NEUROPSYCHIATRIC DISORDERS

The ability to generate neural derivatives from accessible somatic cells from patients with mental disorders (and appropriate controls) is beginning to make these heretofore “mechanistically-unapproachable” complex conditions amenable to rigorous molecular and cellular interrogation. Links are emerging between psychopathology and dysregulation of synaptogenesis, dendritogenesis, cytoskeleton, channels, glial support and inflammation, to name some examples. This session will provide an update on progress in this emerging area. A key take-away will be an appreciation that stem cell modeling has allowed us to begin to gain previously elusive insights into the potential cellular and molecular underpinnings of pathologies that manifest principally by abnormalities in behavior.

Chair / Introduction By:

Evan Snyder, M.D., Ph.D., Director, Center for Stem Cells and Regenerative Medicine; Professor, Human Genetics Program, Sanford Burnham Prebys Medical Discovery Institute

Modeling the Impact of Common and Rare Variants in Schizophrenia Using Stem Cells

Kristen Brennand, Ph.D., Associate Professor, Departments of Genetics and Genomics, Neuroscience and Psychiatry, Icahn School of Medicine, Mount Sinai; New York Stem Cell Foundation – Robertson Investigator

Engineering Brain Organoids for Understanding Human Brain Development and Diseases

Guo-Li Ming, M.D., Ph.D., Professor of Neuroscience, Perelman School of Medicine, University of Pennsylvania

Using Stem Cell Models to Study Bipolar Disorders and Neuroinflammation

Carol Marchetto, Ph.D., Senior Staff Scientist, Laboratory of Genetics, Salk Institute for Biological Studies

4:20pm – 5:00pm

GENE EDITING IN HUMAN EMBRYOS

Sponsored by Homology Medicines

Keynote Speaker:

Kathy Niakan, Ph.D., Group Leader, The Francis Crick Institute

5:00pm – 6:30pm | **NETWORKING RECEPTION**

5:00pm – 6:30pm | **POSTER VIEWING**

Sponsored by Brammer Bio

6:30pm | **SCIENTIFIC SYMPOSIUM CLOSES**