



Draft Workshop Agenda

Tuesday, June 29, 2021 | 10:00 am - 3:00 pm ET

10:00 am ET

NIH Opening Remarks/Welcome

David McMullen, MD; National Institute of Mental Health
John Ngai, NIH BRAIN Initiative

10:10 am ET

SESSION 1: CIRCUIT TOOLS CASE STUDIES

Session 1 will highlight various existing approaches to manipulating circuits in humans. Clinical case studies of genetic, device, and optogenetic-based tools currently used to treat disorders of vision will be explored. How lessons learned from these approaches can inform circuit manipulation tool development for other indications and circuits will be the focus of the following panel discussion.

10:10 am ET *Gene therapy for restoring high acuity vision*
Deniz Dalkara, PhD; Institut de la Vision

10:30 am ET *From Idea to Bedside – Translation of Concepts to Commercial Product*
Bob Greenberg, MD, PhD; Alfred E. Mann Foundation

10:50 am ET *Restoring Visual Function*
Botond Roska, MD, PhD; Institute of Molecular and Clinical Ophthalmology Basel

11:10 am ET

Panel Discussion

Moderator: Beverly Davidson, PhD; Children’s Hospital of Philadelphia

- Jessica Morgan, PhD; University of Pennsylvania
- Sheila Nirenberg, PhD; Cornell University
- Alberto Auricchio, MD; Telethon Institute of Genetics and Medicine
- Kafui Dzirasa, MD, PhD; Duke University
- Susanne Ahmari, MD, PhD; University of Pittsburgh
- Deniz Dalkara, PhD; Institut de la Vision
- Bob Greenberg, MD, PhD; Alfred E. Mann Foundation
- Botond Roska, MD, PhD; Institute of Molecular and Clinical Ophthalmology Basel

12:00 pm ET

BREAK



12:30 pm ET

SESSION 2: FROM GENE THERAPY TO CIRCUIT THERAPY

Session 2 will explore recent advances in gene therapy and editing approaches and learn how they may inform future CNS circuit therapies. The speakers and subsequent panel will probe how lessons from these fields can be used for circuit manipulation now as well as how it can guide future developments.

12:30 pm ET *Retargeting AAV vectors through chemical modification*
Paul Heppenstall, PhD; Scuola Internazionale Superiore di
Study Avanzati

12:45 pm ET *Engineered gene delivery vectors and microbial opsins for
high-precision minimally-invasive access to diseased nervous
systems*
Viviana Gradinaru, PhD; California Institute of Technology

1:00 pm ET *Drug-Regulated Splicing Switch for Gene Expression Control*
Alex Mas Monteys, PhD; Children's Hospital of Philadelphia

1:15 pm ET *A multipoint injection technology for intraparenchymal
convection enhanced delivery*
Flavia Vitale, PhD; University of Pennsylvania

1:30 pm ET *Approaches to Tackle Emerging Immunological Challenges in
Gene Therapy*
Ying Kai Chan, PhD; Harvard University

1:45 pm ET

Panel Discussion

Moderator: Viviana Gradinaru, PhD; California Institute of Technology

- Holly Tabor, PhD; Stanford University
- Nicole Paulk, PhD; University of California San Francisco
- Mark Budde, PhD; California Institute of Technology
- Timothy Yu, MD, PhD; Boston Children's Hospital
- Tobias Moser, MD; Georg-August-Universität Göttingen
- Paul Heppenstall, PhD; Scuola Internazionale Superiore di Study Avanzati
- Alex Mas Monteys, PhD; Children's Hospital of Philadelphia
- Flavia Vitale, PhD; University of Pennsylvania
- Ying Kai Chan, PhD; Harvard University

2:45 pm ET

Closing Remarks

Doug Meinecke, PhD; National Institute of Mental Health

3:00 pm ET

END OF DAY 1



Wednesday, June 30, 2021 | 10:00 am - 3:00 pm ET

10:00 am ET

Opening Remarks

Doug Meinecke, PhD; National Institute of Mental Health

10:10 am ET

SESSION 3: CNS TRANSLATIONAL HURDLES

Session 3 will identify and discuss major hurdles impeding the translation of circuit therapies from basic science to therapeutic tools. Safely delivering, and validating in humans and animal models, transgene constructs to specific cell-types and circuits poses many difficulties. The speakers for this session will both highlight these difficulties and discuss potential avenues to overcome these hurdles.

10:10 am ET

Delivery: AAV Gene Therapy

Petra Kaufmann, MD; Affinia Therapeutics

10:25 am ET

Immunoengineering: Tissue Interfaces and Regenerative Therapeutics

Kaitlyn Sadtler, PhD; National Institute of Biomedical Imaging and Bioengineering

10:40 am ET

Enhancer AAVs for cell type targeting

Bosiljka Tasic, PhD; Allen Institute

10:55 am ET

AAV immunogenicity, implication for CNS gene therapy

Federico Mingozzi, PhD; Spark Therapeutics

11:10 am ET

Gene Delivery to the CNS in a Primate Model (Marmoset)

Jim Pickel, PhD; National Institutes of Health

11:30 am ET

Panel Discussion

Moderator: David McMullen, MD; National Institute of Mental Health

- Kathy Ferrara, PhD; Stanford University
- Gordon Fishell, PhD; Harvard University
- Adam Hantman, PhD; Janelia Research Campus
- Clive Svendsen, MD; Ceders-Sinai Medical Center
- Sergiu Pasca, MD; Stanford University
- Petra Kaufmann, MD; Affinia Therapeutics
- Kaitlyn Sadtler, PhD; National Institute of Biomedical Imaging and Bioengineering
- Bosiljka Tasic, PhD; Allen Institute
- Federico Mingozzi, PhD; Spark Therapeutics
- Jim Pickel, PhD; National Institutes of Health

12:30 pm ET

BREAK



1:00 pm ET

SESSION 4: CNS TRANSLATIONAL ENVIRONMENT

This final session will bring in speakers from the wider regulatory and commercialization vantages to provide perspective on requisite steps to begin testing in humans (FDA) and successes necessary to demonstrate viability and de-risk for funders (VCs, government partners, and public-private partnerships). We will also hear from the patient perspective to better understand how the translational community and patient groups can work together to advance new therapies.

1:00 pm ET *Preclinical Assessment of Cell and Gene Therapy (CGT) Products to Support an IND*
Feorillo Galivo, MD, PhD; Food and Drug Administration/CBER

1:15 pm ET *From circuits to cures: considering the future*
Scott Sternson, PhD; Janelia Research Campus

1:30 pm ET *Venture & Angel Investing in CNS and other Healthcare*
Bill Podd, JD; Landmark Capital

1:45 pm ET *The Role of Advocacy in Advancing New Therapies*
Rachel Salzman, DVM; Alcyone Therapeutics

2:00 pm ET

Panel Discussion

Moderator: Ryan Richardson, NINDS/OBD

- Allan Basbaum, PhD; University of California San Francisco
- Jose-Alain Sahel, MD; University of Pittsburgh
- Tracy Dixon-Salazar, PhD; Lennox-Gastaut Syndrome Foundation
- Clare Ozawa, PhD; Versant Ventures
- Feorillo Galivo, MD, PhD; Food and Drug Administration/CBER
- Scott Sternson, PhD; Janelia Research Campus
- Bill Podd, JD; Landmark Capital
- Rachel Salzman, DVM; Alcyone Therapeutics

2:50 pm ET

Closing Remarks

David McMullen, MD; National Institute of Mental Health

3:00 pm ET

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