

# KEYSTONE SYMPOSIA

on Molecular and Cellular Biology

## Cerebral Fluid Flow and Function: Lymphatics, Glymphatics and the Choroid Plexus (Q4)

Scientific Organizers: Edwin S. Monuki, Maria Lehtinen and Maiken Nedergaard

Supported by the Directors' Fund

## Brain Therapeutics: Disruptive Technologies and Opportunities (Q3)

Scientific Organizers: Maria-Jesus Blanco, Kalpana M. Merchant and Maria Isabel Loza

February 16-19, 2020 • Eldorado Hotel & Spa • Santa Fe, NM, USA

Supported by the Directors' Fund

Abstract & Scholarship Deadline: October 29, 2019 / Abstract Deadline: November 19, 2019 / Discounted Registration Deadline: December 17, 2019

### SUNDAY, FEBRUARY 16

#### Arrival and Registration

### MONDAY, FEBRUARY 17

#### Welcome and Keynote Address (Q4)

**Kari K. Alitalo**, University of Helsinki, Finland  
*Processing CSF Outflow at the High-End of Lymphatics*

#### Welcome and Keynote Address (Q3)

**Steven M. Paul**, Karuna Pharmaceuticals and Third Rock Ventures, USA  
*Overview of Clinical CNS Therapeutics Landscape*

#### Choroid Plexus and CSF Regulation of Stem Cells (Q4)

**Norman Ruthven Saunders**, University of Melbourne, Australia  
*The Choroid Plexus: Mechanisms that Limit Drug Entry into the Developing Brain*

**Maria Lehtinen**, Harvard Medical School, USA  
*CSF Regulation of the Developing Brain*

**Nanna MacAulay**, University of Copenhagen, Denmark  
*Mechanism of CSF Secretion – Conventional Osmosis versus Active Water Transport*

#### Short Talks Chosen from Abstracts

#### Challenges and Opportunities for Brain Therapeutics: Preclinical to Clinical Translation (Q3)

**Kalpana M. Merchant**, Vincere Biosciences, Inc., USA  
*De-Risking Disease-Modifying Therapies of PD: From Computational Biology Approaches for Disease-Relevant Targets to Patient Enrichment Biomarkers and Digital End-Points*

#### Speaker to be Announced

**Chas Bountra**, University of Oxford, UK  
*New technologies, collaborations and processes, to accelerate the de-risking of pioneer drug targets, for neuro-psychiatric conditions*

#### Short Talks Chosen from Abstracts

#### Workshop 1 (Q4)

#### Short Talks Chosen from Abstracts

#### Roundtable: Collaboration & Knowledge Integration Models for Successful Brain Therapeutics (Q3)

\***Maria Isabel Loza**, University of Santiago De Compostela, Spain

**James E. Audia**, Chicago Biomedical Consortium, USA

*Drug Discovery in the Academic and Not-For-Profit Community*

**Sohini Chowdhury**, Michael J. Fox Foundation, USA

**Suzana Petanceska**, NIA, National Institutes of Health, USA

#### Glymphatics (Q4)

**Maiken Nedergaard**, University of Copenhagen, Denmark  
*Sleep Regulation and Disease Roles of the Glymphatic System*

**Jeffrey J. Iliff**, Oregon Health and Science University, USA  
*Molecular and Cellular Regulation of the Glymphatic System*

**Helene Benveniste**, Yale School of Medicine, USA  
*Glymphatic Flow, Regulation, and Function*

#### Short Talks Chosen from Abstracts

#### Advances in Technologies to Garner Insights into Disease Biology (Q3)

**Edoardo Marcora**, Icahn School of Medicine at Mount Sinai, USA  
*Talk Title to be Announced*

**Lena F. Burbulla**, Northwestern University, Feinberg School of Medicine, USA

*iPS-Derived Cellular Systems: Impact from Latest Research*

#### Short Talks Chosen from Abstracts

#### Poster Session 1

### TUESDAY, FEBRUARY 18

#### Lymphatics and CSF Dynamics (Q4)

**Jonathan Kipnis**, University of Virginia, USA  
*Lymphatic-Glymphatic Roles in Aging and Disease*

**Erik N.T.P. Bakker**, University of Amsterdam, Netherlands  
*Effect of Hypertension on Fluid Management in the Brain*

**Steffi Dreha-Kulaczewski**, University Medical Center, Germany  
*Breath to Drive CSF Flow*

#### Short Talks Chosen from Abstracts

#### Integrating Novel Medicinal Chemistry Approaches Targeting the Brain (Q3)

**Anastasia Khvorova**, University of Massachusetts Medical School, USA

*Novel siRNA and Antisense Oligonucleotide Approaches for Treatment of Neurodegenerative Disorders*

**Jacob M. Hooker**, Harvard Medical School, USA  
*Integrating PET Tracers into CNS Drug Discovery: Impact on the Discovery of Novel HDAC Ligands*

**Maria-Jesus Blanco**, Sage Therapeutics, USA  
*Leading with Human Data: Discovery of Novel Clinical Compounds for CNS Disorders*

**Craig W. Lindsley**, Vanderbilt University, USA  
*Latest Efforts Towards the Discovery of Clinical mGlu4 Allosteric Modulators for Parkinson's Disease*

#### Short Talks Chosen from Abstracts

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### Integrating Brain-Body Signaling (Q4)

**Fiona Doetsch**, University of Basel, Switzerland  
*CSF Regulation of Adult Neural Stem Cells*

**Christer Betsholtz**, Uppsala University, Sweden  
*Fenestrated Blood Vessels in the Brain and Eye*

**Toru Takumi**, RIKEN Center for Brain Science Institute, Japan  
*Choroid Plexus and Circadian Clock*

**Claire Wyart**, Institut du Cerveau et de la Moelle Epinière, France  
*Origin and Role of the Cerebrospinal Fluid Bidirectional Flow in the Central Canal*

Short Talks Chosen from Abstracts

### Diagnostic and/or Disease-State Biomarkers (Q3)

**Gwenn A. Garden**, University of Washington, USA  
*Novel Glial Biomarkers in Human Central Nervous System Disease*

**Steffany A.L. Bennett**, University of Ottawa, Canada  
*Lipidomic-Based Insights into Disease Mechanisms: A Targeted Lipidomics Discovery Pipeline for Network and Flux Analysis*

**David Nguyen†**, Sage Therapeutics, USA  
*Novel Digital Technologies towards CNS Translational Insights*

Short Talks Chosen from Abstracts

### Poster Session 2

#### WEDNESDAY, FEBRUARY 19

### Choroid Plexus and CSF Regulation of Neuroimmune Responses (Q4)

**Adam Chodobski**, Alpert Medical School of Brown University, USA  
*The Choroid Plexus in Brain Injury and Neuroinflammation*

**Jean-François Gherzi-Egea**, Centre de Recherche en Neurosciences de Lyon, France  
*CSF Barriers to Immune Cells and Drugs*

**Michal Schwartz**, Weizmann Institute of Science, Israel  
*Choroid Plexus Regulation of the Neuroimmune Response*

Short Talks Chosen from Abstracts

### Ensuring Target Engagement: Innovative Approaches Towards Neuroimaging and Brain Delivery (Q3)

**Tetsuya Suhara**, National Institutes for Quantum and Radiological Science and Technology, Japan  
*Strategies for Utilizing Neuroimaging Biomarkers in CNS Drug Discovery and Development*

**Mickael Tanter**, Institute Langevin, France  
*Functional Ultrasound Imaging of the Brain Activity: A Fast Growing Neuroimaging Modality with Unique Features for Preclinical and Clinical Studies*

**Maria José Alonso**, Universidade de Santiago de Compostela, Spain  
*Nanotechnology-Based Strategies for the Delivery of Biological Drugs to the Brain*

**Lisa L. Shafer**, NeuroBridge Therapeutics, Inc., USA  
*Emerging Strategies for Brain Drug Delivery: Latest Case Applications*

**Robert G. Thorne**, Denali Therapeutics, USA  
*Characterizing Fusion Protein Transport Across CNS Barriers: Leveraging Collaborations to Develop New Brain Therapeutics*

Short Talks Chosen from Abstracts

### Workshop 2 (Q4)

Short Talks Chosen from Abstracts

### Workshop: Computational Framework for Integrated CNS Drug Discovery and Design (Q3)

Short Talks Chosen from Abstracts

### Diagnostics and Therapies (Q4)

**Kristopher Kahle**, Yale School of Medicine, USA  
*Genetics of Human Hydrocephalus*

**Patricia I. Dickson**, Washington University, USA  
*CSF Delivery of Therapeutics*

**Christopher Moore**, Brown University, USA  
*A Systems Approach to the Choroid Plexus*

**Edwin S. Monuki**, University of California, Irvine, USA  
*Choroid Plexus Regenerative Medicine*

### Innovative Therapeutic Approaches Targeting the Brain (Q3)

**Aletta D. Kraneveld**, University of Utrecht, Netherlands  
*Parkinson's Disease: No Guts No Glory*

Speaker to be Announced

**Giorgia Quadrato**, University of Southern California - USC Stem Cell, USA

*Modeling Human Brain Development and Disease at Single Cell Resolution with Organoids*

Short Talks Chosen from Abstracts

### Meeting Wrap-Up: Outcomes and Future Directions (Organizers) (Q4)

### Meeting Wrap-Up: Outcomes and Future Directions (Organizers) (Q3)

#### THURSDAY, FEBRUARY 20

Departure