

Workshop on CNS Immunogenicity Considerations for AAV-mediated Gene Therapy

Natcher Conference Center | Balcony B | NIH Campus | 9000 Rockville Pike | Bethesda, MD

June 11, 2019

Agenda

- 8:00 a.m. Registration**
- 8:30 a.m. Welcome**
Christopher P. Austin, M.D., Director National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)
- NCATS NIH Opening Remarks**
Anne Pariser, M.D., Director, Office of Rare Diseases Research (ORDR), NCATS, NIH
PJ Brooks, Ph.D., Program Director, ORDR, NCATS, NIH
- 8:40 a.m. Cures Acceleration Network Review Board Remarks**
Ronald Bartek, M.A., NCATS Council Member, President, Friedreich's Ataxia Research Alliance
- 8:45 a.m. Organizing Committee Remarks**
R. Jude Samulski, Ph.D., Professor, Department of Pharmacology, University of North Carolina School of Medicine
Samantha Parker, M.B.A., Chief Patient Access Officer, Lysogene, Paris
- 8:50 a.m. CBER/FDA Remarks**
Mercedes Serabian, M.S., Branch Chief, Pharmacology/Toxicology Branch, Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration (FDA)
- 8:55 a.m. American Society of Gene and Cell Therapy (ASGCT) Remarks**
Guangping Gao, Ph.D., Professor, Microbiology and Physiological Systems, Penelope Booth Rockwell Professor in Biomedical Research, Co-Director, Li Weibo Institute for Rare Diseases; Research Director, Horae Gene Therapy Center and Vector Core; Scientific Director, UMass Medical School (UMMS)-China Program Office
- 9:00 a.m. Introduction**
Olaf Stuve, M.D., Ph.D., Department of Neurology and Neurotherapeutics, University of Texas (UT) Southwestern Medical Center
- 9:30 a.m. Session 1: Clinical Data**
Session participants will review clinical trial experience, including immunosuppressive protocols, immune response, issues around pre-existing antibodies, re-dosing and differentiation between CRIM-negative and CRIM-positive patients.



Moderators: **Ronald G. Crystal**, M.D., Professor and Chairman, Department of Genetic Medicine, Weill Cornell Medical College
Nathalie Cartier, Ph.D., Cell and Gene Therapy for Neurodegenerative Diseases, INSERM ICM (Institute for Brain and Spine)

Panelists: **Krystof Bankiewicz**, M.D., Ph.D., Department of Neurological Surgery, University of California, San Francisco (UCSF)
Diana X. Bharucha-Goebel, M.D., Neurogenetics Branch, National Institute of Neurological Disorders and Stroke (NINDS), NIH
Barry Byrne, M.D., Ph.D., Director, University of Florida (UF) Powell Gene Therapy Center (PGTC); Professor, Pediatrics and Molecular Genetics and Microbiology, UFHealth
Martin K. (Casey) Childers, D.O., Ph.D., Chief Medical Officer, AskBio
Manuela Corti, P.T., Ph.D., Assistant Professor, Child Health Research Institute, UF
Olivier Danos, Ph.D., Chief Scientific Officer, Regenxbio
Paola Leone, Ph.D., Professor, Director of the Cell and Gene Therapy Center, Department of Cell Biology and Neuroscience, RowanSOM
Sophie Olivier, M.D., Chief Medical Officer, Lysogene
Caroline Sevin, M.D., Kremlin Bicetre Hospital, Paris

1:00 p.m. **Lunch** (on your own)

2:00 p.m. **Session 2: Pre-Clinical Development**

Session participants will discuss animal models and assays and their role in clarifying current gaps and issues related to immunogenicity.

Moderators: **Federico Mingozzi**, Ph.D., Genethon; Chief Scientific Officer, Spark Therapeutics
Steven Gray, Ph.D., Associate Professor, Department of Pediatrics, UT Southwestern Medical Center

Panelists: **Brian R. Long**, Ph.D., Senior Scientist, BioMarin Pharmaceuticals
Ying Kai Chan, Ph.D., Harvard Medical School
Klaudia Kuranda, Ph.D., Immunology Leader, Spark Therapeutics
Miguel Sena Esteves, Ph.D., Associate Professor, Department of Neurology, Horae Gene Therapy Center, University of Massachusetts Medical School
Juliette Hordeaux, D.V.M., Ph.D, Dipl. ECVP, Research Director, Lysosomal Storage Disease, Gene Therapy Program, University of Pennsylvania

4:00 p.m. **Open floor discussion with Q&A for panelists**

5:00 p.m. **Adjournment**