

KTRN Tentative Agenda

Subject to Change

Wednesday March 16, 2022

- 7:30 AM** **Breakfast**
- 8:30 AM** **Introduction and Objectives**
- 9:00** **Keynote: Studying Myelinated Axons in Vivo Using Zebrafish**
Professor David Lyons

Newborn Screening: Screening Methodologies and Newborn Screening Outcomes for Krabbe Disease

- 10:00** **Enzyme Assays and Biomarkers to KD and Beyond**
Michael Gelb, Ph.D.
University of Washington
- 10:15** **Benefits of NBS and HSCT in IKD**
Joanne Kurtzberg, M.D.
Duke University

10:30 Break

- 10:50** **A Review of Recent Developments and Applications of BVNLA for Presymptomatic Detection of MPS 1, Pompe disease, and KD**
Kabir Jalal, Ph.D.
University at Buffalo
- 11:05** **A Single Site Experience with KD NBS**
Laura Adang, M.D., Ph.D.
Children's Hospital of Philadelphia
- 11:20** **Question and Answer Period**

11:40 Lunch

- 12:40** **Panel Discussion: State Newborn Screening Laboratory Experiences and Caregiver Insights – Successes and Opportunities for Improvement**

Pathology, Immunology, Natural History, of Krabbe Disease, and Transition to Therapy

- 1:40** **Brain Macrophages in Pathogenesis and Treatment of KD**
Frederick Bennett, M.D.
University of Pennsylvania
- 1:55** **Inhibition of CD8 T-cells Prevents Clinical Disease and CNS Myelin Pathology in a Mouse Model of KD**
Stephen Crocker, Ph.D.
University of Connecticut School of Medicine
- 2:10** **Lymphocyte Infiltration into White Matter is a Common Feature of KD**
Julia Kofler, M.D.
University of Pittsburgh Medical Center
- 2:25** **Question and Answer Period**
- 2:40** **Break**
- 3:00** **Recommendations of Psychosine Interpretation and Clinical Manifestations in KD**
Ecenur Tuc Bengur, M.D.
University of Pittsburgh Medical Center
- 3:15** **Cell Modeling and GALC Protein Assays for Preclinical Drug Development in KD**
Chris Lee, Ph.D.
Biomedical Research Institute of New Jersey
- 3:30** **Biochemical Analysis of KD Patients with p.Y309C GALC Variant**
Amy White, M.S., C.G.C.
Mayo Clinic
- 3:45** **Studying the Role of Autophagy in KD**
Narayan Dhimal
University at Buffalo
- 4:00** **Question and Answer Period**
- 4:20** **Discussion**
- 5:00** **Adjourn | Dinner on your own**

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7:30 AM Breakfast

8:30 Recap of Yesterday

New Therapies for Krabbe Disease

9:00 Panel Discussion: Caregiver Insights on Managing Late Onset Krabbe Disease

10:00 HOLD for FDA /NIH

10:15 Long-Term Efficacy of AAV Gene Therapy for KD Limited by Dilutional Loss of AAV Episomes Over Time

Gregory Heller
University of Illinois at Chicago

10:30 Safety, Biomarker and Preliminary Efficacy Results Following ICM Administration of PBGM01 in Children with Later Onset Infantile GM1 Gangliosidosis: Implications for the Gene Therapy Trial for Krabbe Disease

David Weinstein, M.D., M.M.Sc.
Passage Bio

10:45 Passage Bio Take on KD Clinical Trial PBK-R03

Bruce Goldsmith
Passage Bio

11:00 FBX-101, an IV AAV Gene Therapy After Infusion of HSCT, Extends Efficacious Dose Ranging and Corrects Disease Manifestations in Krabbe Disease

Maria Escolar, M.D., Ph.D.
University of Pittsburgh Medical Center

11:15 Brain Connectome as a Novel Biomarker of KD

TBD

11:30 Predicting Neurodevelopmental Outcomes Using DTI Tractography and Psychosine in IKD

Melissa Greco
University of Pittsburgh Medical Center

11:45 Question and Answer Period & Discussion

12:15 Lunch + Presentation of the Sue Rosenau Award

Non-Profits Helping to Advance Efforts for Their Community

1:15 CureSMA Experience with Gene Therapy

1:45 KrabbeCURES (Community United Research and Engagement Study)

2:15 Hunter's Hope Foundation Update on KD RUSP Nomination

2:30 Break

2:55 Focus Group with Stakeholders and Families Impacted by Krabbe Disease

4:30 Wrap-Up and Adjourn the 2022 KTRN Meeting

Thank you to the following sponsors for their educational support for the 2022 KTRN meeting:

