

**11<sup>th</sup> collaborative gathering of the Krabbe Translational Research Network (KTRN)**

**The Westin Tampa Waterside**

**Wednesday April 19 – Friday 21, 2023**

**Wed AM: Krabbe disease Neuroinflammation and Pathology:**

**1. 9 AM-10 AM**

1. **Marius Wernig** (Keynote Speaker): The role of neuroinflammation in neurodegeneration

**2. 10 AM-10:30 AM**

1. **Chris Lee**: GALC deficiency and pathologic abnormalities in white matter of KD
2. **Stephen Crocker**: Role of CD8 T cells in Krabbe Disease

**3. 10:30-10:45 AM Break**

**4. 10:45-11:15 AM**

1. **Diego Zelada**: Dysfunction of hippocampal synaptic function: impact on memory in Krabbe disease.
2. **Chris Bennet- *Prerecorded*** -Engineering an inhibitor-resistant human CSF1R variant for microglia replacement.

**5. 11:15 AM-11:45 AM: Panel Discussion and Q&A**

**11:45 AM-1:30 PM:** Posters & Lunch

**Wed PM: Emerging Therapies in Krabbe disease:**

**1. 1:30-2:30 PM**

1. **Tony Filiano** (Keynote speaker): Novel (cell) therapeutic innovations for neuroinflammatory disease

**2. 2:30-3:00 PM**

1. **Noemie Hamilton**: Using zebrafish leukodystrophy models to develop novel therapies.
2. **Steven LeVine: *Pre-recorded***: Repurposing FDA Approved Drug as a Substrate Reduction Therapy for Krabbe Disease

**3. 3:00-3:15PM Break**

**4. 3:15-5:00 PM**

1. **Mark Sands**: Preliminary data on a modified form of GALC for the treatment of Krabbe disease
2. **Maria I. Givogri**: Extracellular vesicle assisted ERT for temporary treatment of infants with Krabbe disease Emerging Therapies

3. **Natalia Saldivia-Soto:** Resurgence of localized demyelination in the adult Krabbe brain after neonatal AAV-gene therapy
4. **Panel Discussion and Q&A**
5. **5:00-6:00 PM Sponsor Presentations:**
  1. **Li Ou:** rAAV immunogenicity, toxicity, and durability in 255 clinical trials: A meta-analysis
  2. **Others TBD**

**Wednesday Evening: Cocktails, Dinner, Presentation of the Sue Rosenau Legacy Award**

**Thurs AM & PM: NBS after the RUSP**

- **Thurs AM 8:30AM-10:30 AM-** NBS strategy and laboratory diagnosis of Krabbe disease
  - **Dietrich Matern** (Keynote speaker): Improved screening and diagnosis with psychosine
  - **Joseph Orsini:** The evolution of NBS for KD in NY over 16 years – laboratory perspective
  - **Panel Session 1:** NBS test interpretation and management
- **10:30 AM-10:45 Break**
- **10:45-12:30 PM:** Plenary Session
- **12:30 PM-2:30 PM:** Lunch & Posters
- **Thurs PM 2:30-5:30PM** Clinical consideration for Krabbe Disease NBS
  - **Joanne Kurtzberg** (Keynote Speaker): Treatment outcomes in Infantile Onset Krabbe Disease
  - **Robert Stone *Prerecorded*:** Classification and long-term follow up of infants who screen positive for Krabbe Disease
  - **Paul Levy:** The evolution of NBS for KD in NY over 16 years – clinical perspective
  - **Paul Orchard:** Getting ready to support Krabbe disease screening – perspective from a regional transplant center.
  - **Panel Session 2:** The clinical follow up and management following a positive NBS result: one size does not fit all.

**Fri 8:30AM-Noon: KTRN/Krabbe Connect Joint Session: Ethical, Cultural, and Accessibility Considerations**

- **Suhag Parikh:** Case Presentation
- **Panel Discussion** (led and moderated by Jeremy Sugarman, Joanne Kurtzberg, Stacy Pike-Langenfeld:
  - Bioethics
  - Communication and processing of complex/ambiguous information:

- HCP and patient perspectives
- Cultural considerations
- Access to care, IE
- Identifying and addressing barriers to accessing care (incl. discussion of whether Krabbe is different from other RUSP conditions)