Joint NDI – ND FAWG workshop: June 3

Time: 1:30 to 5:30 pm

Location: Conference room 3101, Child Health Institute, 89 French Street, New Brunswick

Program:

1:30 – 1:45 Welcome (Detlev Boison & Chiara Manzini)

1:45 – 2:45 Lectures #1-4: 15 min each incl. Q&A

2:45 – 3:00 Coffee Break

3:00 – 4:00 Lectures #5-8: 15 min each incl. Q&A

4:00 – 5:30 Networking Mixer with Pizza and Drinks

Lectures:

1. Li Cai:

Title: GSX1 Gene Therapy for Spinal Cord Injury: A Regenerative Approach

Impact Statement:

Spinal cord injury (SCI) causes lifelong disability with no approved restorative therapies. To bridge this gap, we developed AAV6-GSX1, a gene therapy that re-engages endogenous repair. GSX1 activates adult neural stem cells to generate functional interneurons, reduces glial scarring, and restores locomotion. Validated in clinically relevant rodent contusion models, this platform aligns with NIH regenerative medicine priorities and is poised for IND-enabling studies. AAV6-GSX1 represents a scientifically validated opportunity to restore neural function, improve patient outcomes, and reduce the national socioeconomic burden of CNS injuries.

2. Detlev Boison:

Title: An adenosine-driven epigenetic basis for autism spectrum disorder?

Impact Statement:

Adenosine, a product of DNA methylation reactions in the nucleus, plays an important role as epigenetic disease modifying factor in epilepsy. Using a maternal immune activation model in pregnant mice we found that a poly(I:C) induced immune challenge led to a maternal adenosine surge and disruption of the placental adenosine barrier. We also found

that the maternal immune challenge caused hypomethylation of DNA in the fetal brain. We hypothesize that a maternal adenosine surge, reaching the fetal brain, causes epigenetic changes, which might contribute to the development of autism.

3. Vadim Ten:

Title: Can resuscitation be modified to enhance neuroprotection?

Impact Statement:

1. The pace of bioenergetic recovery during resuscitation contributes to cerebral cellular fate.
2. Initiation of resuscitation with 100% oxygen significantly faster revives cerebral bioenergetics.
3. Mode of resuscitation modifies genetic response to ischemia and reperfusion.

4. KiBum Lee:

Title: Multimodal Neuroengineering Platforms for Enhanced CNS Repair

Impact statement:

By combining biomaterials, bioactive vesicle therapeutics, and next-generation diagnostics, multimodal neuroengineering platforms offer a new paradigm for CNS repair. These integrated systems may overcome longstanding barriers to regeneration by enabling coordinated intervention, monitoring, and personalization of therapy."

5. Yoon-seong Kim:

Title: Age-Dependent Propagation of Colonic Lumen α-Synuclein PFF to the Brain

Impact statement:

This study establishes the colon as an active origin and driver of α-synuclein pathology by defining how luminal α-synuclein is induced, processed, and propagated to the brain through epithelial, immune, and ICC-mediated pathways, particularly under aging conditions, thereby uncovering novel targets for early intervention in Parkinson’s disease.

6. Steven Levison:

Title: Giving low birthweight premature infant brains a therapeutic LIFt

Impact statement:

Very low birth weight premature infants experience repeated, brief episodes of intermittent hypoxemia during the initial weeks of life with attendant diffuse white matter injury that often leads to intellectual disability and sensorimotor impairments. Much needed is a better understanding for the mechanisms responsible for the dysgenesis of white matter myelination. Using in vivo and in vitro mouse models of repeated intermittent hypoxia we are trying to identify those key intracellular signaling changes underlying the failure of oligodendrocyte progenitor differentiation and we are testing the therapeutic efficacy of leukemia inhibitory factor (LIF) as a therapeutic to restore white matter maturation and function.

7. Chiara Manzini:

Title: Photoreceptor and motor neuron loss in congenital muscle disease

Impact Statement:

Studies in zebrafish models of autosomal recessive congenital muscular dystrophies affecting the brain and eye revealed a combination of unappreciated developmental and degenerative features with hallmarks of retinitis pigmentosa and motor neuron disease. These studies redefine the pathophysiology of these disorders, often in a gene-specific manner.

8. Jennifer Mulle:

Title: 3q29 deletion syndrome: insights driving a next wave of discovery linking genes, brain, and behavior in rare neuropsychiatric conditions

Impact Statement:

The 3q29 deletion has a high effect size for a range of neurodevelopmental and psychiatric conditions; understanding how this deletion impacts molecular, cellular and circuitry-based neurodevelopmental processes will lend insights to mechanisms underlying idiopathic conditions such as autism and schizophrenia.