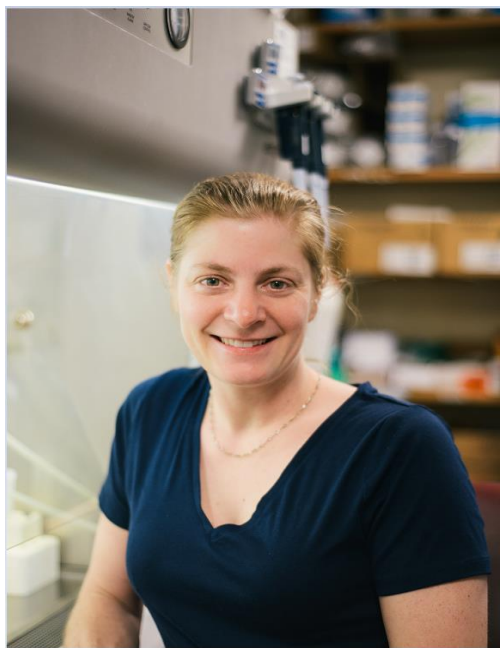
manitoba neuroscience network



Bench-to-Bedside Stories: In Vitro Modelling and Therapeutic Development for Neurological Disorders

SEMINAR & VISITING SPEAKER SERIES

DATE
Monday, January 30th, 2023
10:45 AM

LOCATION
Apotex Centre 050

SPEAKER

Kathrin Meyer, PhD
Assistant Professor, The Ohio State University, Department of Pediatrics; Principle Investigator, Nationwide Children's Hospital, Center for Gene Therapy, Columbus Ohio, USA

BIO

Dr. Meyer received her PhD at the University of Berne, Switzerland working with Prof. Daniel Schuemperli on RNA biology. As a post-doctoral fellow in Dr. Brian Kaspar's laboratory at NCH she gained extensive experience in translational gene therapy programs, including generation of new vectors, and the design and execution of IND-enabling efficacy and safety studies in mice and non-human primates. Dr. Meyer was involved in 5 successfully translated AAV programs for SMA, forms of Batten Disease and SMARD1/CMT2S. Dr. Meyer serves in several scientific advisory boards to patient foundations who are actively pursuing gene therapy programs. In addition to the gene therapy efforts, Dr. Meyer developed a new reprogramming method for in vitro modelling of nervous system diseases and its use for therapeutic testing that has been adapted by several international collaborators. The Meyer lab uses these models to study disease mechanisms and develop new therapeutic strategies with particular focus on seizure, neurodevelopmental and autism spectrum disorders.

ABSTRACT

The seminar will touch base on key aspects of translating adeno-associated viral vector (AAV) gene therapy from bench to clinical trials for neurological and neurodegenerative disorders based on multiple clinical-stage programs (SMA, Batten Diseases and SMARD1/CMT2S). In addition, a novel AAV gene therapy approach aimed towards X chromosome reactivation that could serve as a platform therapy for multiple disorders will be presented. In the context of developing novel therapeutics, the importance of the use of patient-derived cell lines for the study of disease mechanisms as well as drug testing (including gene therapy) will be discussed. Amongst many diseases, the Meyer lab studies pathological mechanisms for a newly discovered neurological disease called NEDAMSS, which is caused by mutations in the IRF2BPL protein. The data indicates that the mutated protein sequesters the wild-type form to the cytoplasm causing reduced abundance in the nucleus. This data challenges the classic view of the disease being caused by haploinsufficiency and proposes a gain of function for the mutated truncated protein isoforms. In addition, novel data on phenotypic variability as well as disease mechanisms in Pitt Hopkins Disease will be shared. Common underlying disease pathways could lead to the identification of novel therapeutics with applications in multiple disorders.

OBJECTIVES

1. Further understanding of translational aspects of AAV gene therapy
2. Present novel X-reactivation AAV gene therapy approach for Rett Syndrome
3. Emphasize the importance of disease mechanism research using patient-derived cell lines for therapeutic development including but not limited to gene therapy

For more information:

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