



HARRY PERKINS INSTITUTE
OF MEDICAL RESEARCH



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PERKINS Seminar Series

and

Perron Institute/Murdoch University

MONDAY 22 MAY



Dr Brian Kaspar

Principal Investigator in the Centre for Gene Therapy
The Research Institute
Nationwide Children's Hospital, Columbus Ohio

"Gene Delivery Translation in Spinal Muscular Atrophy"

Dr Kaspar's research focuses on basic and translational studies related to neurological and neuromuscular disorders. The laboratory has strengths in animal models of neurodegenerative and neuromuscular disease, gene delivery, and stem cell biology. A main focus of the Kaspar laboratory is centered on the mechanism(s) of neurodegeneration in Amyotrophic Lateral Sclerosis (ALS) and Spinal Muscular Atrophy (SMA). We employ rodent models of this disease to investigate various cell type involvements in disease onset and progression. Furthermore, we are actively developing novel methods to deliver genes and therapies more efficiently to the nervous system and testing novel targets to combat this debilitating, lethal disease. The laboratory also investigates the biological control of embryonic and adult derived stem cells. Our current studies with stem cells are evaluating cell cycle regulation along with developing methods for intricate control of differentiation to defined cellular phenotypes, such as complex motor neurons. Finally, our laboratory works on muscle enhancing strategies in order to combat musculoskeletal disorders. We have identified that follistatin is a potent antagonist of myostatin, and when delivered by a one-time gene delivery to skeletal muscle, enhances muscle size and mass. Interestingly, our work has demonstrated that follistatin reduces the inflammatory environment in dystrophic muscles and induces improvements in functional strength in rodent models of Duchene Muscular Dystrophy. We are currently in a Phase I/II human clinical trial for Becker Muscular Dystrophy and Inclusion Body Myositis using our expertise in Adeno Associated Virus. Furthermore, we have advanced human trials for gene delivery in Spinal Muscular Atrophy. We utilize a variety of viral vectors including Adenovirus, Adeno Associated Virus, Retrovirus and Lentivirus to answer important questions in biology and have the knowledge set for translational studies.

2:00pm till 3:00pm

followed by a light afternoon tea

For more information please contact Jodie Williamson on jwilliamson@ccg.murdoch.edu.au

OR A/Prof Juliana Hamzah on juliana.hamzah@perkins.uwa.edu.au

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