



Webinar

Course Title: A New Era of Medicine, Now & the Future: Gene Therapy for Paediatric Neuromuscular Diseases

Course Date and time: December 2nd, 2021 at 7:00 PM

Chairs and Speakers:

- Dr. Jim (James) Dowling, MD, PhD; SickKids Hospital
- Dr. Hugh McMillan, MD, MSc, FRCPC, FAAN; Montreal Children’s Hospital
- Dr. Pranesh Chakraborty MD, FRCPC, FCCMG, Childrens Hospital of Eastern Ontario
- Stacey Lintern; CEO, Muscular Dystrophy Canada
- Dr. Homira Osman, PhD; VP, Research and Public Policy, Muscular Dystrophy Canada

Course Description:

Through expert presentations, participants will learn about exciting advances in gene therapy for the treatment of neuromuscular diseases, including SMA. Speakers will also explore the critical role newborn screening plays in facilitating pre-symptomatic diagnosis and early treatment of disease, and the implementation of newborn screening in Canada. The session will then be followed by an interactive live Question & Answer session with all speakers.

By the end of this course participants will be able to:

- Discuss the current clinical research evidence and application of gene therapy in paediatric neurological and neuromuscular diseases (NMD)
- Appraise the role of current treatments in helping SMA patients reach their milestones
- Summarize the key considerations, learnings and outcomes related to public and private sectors working on newborn screening (NBS)
- Determine the role of the clinical and research community in facilitating the inclusion of additional NMDs’ into NBS

Audience:

Child Neurologist | Neuro Physiologist | Resident | Fellow | Nurses with interest in topic

Learning Level:

Basic (Resident, New Information) | Intermediate (Practicing Physician) | Advanced (SIG, Higher Level Discussion)

Learning Format:

Virtual meeting | Live Q&A and panel discussion

CanMED Roles:

Medical Expert | Scholar | Communicator | Collaborator | Leader | Health Advocate | Professional

Time	Description	Name of Presenter
7:00 – 7:05 PM	Introduction	Dr. Dowling
7:05 – 7:20 PM	Gene therapy in neuromuscular diseases	Dr. Dowling
7:20 – 7:35 PM	Implementation of newborn screening in Ontario	Dr. Chakraborty
7:35 – 7:50 PM	MDC and Canada-wide implementation of newborn screening	Stacey and Homira
7:50 – 8:05 PM	Current SMA treatment and milestones	Dr. McMillan
8:05 – 8:30 PM	Q&A and panel discussion	All

This Webinar is an unaccredited group learning approved by the Canadian Neurological Sciences Federation. The program was developed by the Canadian Neurological Sciences Federation and Faculty and was planned to achieve scientific integrity, objectivity and balance. This Webinar was supported by Novartis Pharmaceuticals Canada Inc. who provided an unrestricted grant and in-kind support.