

# PAEDIATRIC GRAND ROUNDS

Alberta Children's Hospital

October 9, 2024  
8:30 - 9:30 AM

**\*\*\*Registration Required for Zoom\*\*\***

**\*\*\*Kinsmen Learning Centre Auditorium (4<sup>th</sup> Floor - ACH) for In-Person\*\*\***

## Gene Therapy for Rare Disorders: Example using Sanfilippo Syndrome

**Kim L McBride**

Medical Genetics

### Objectives:

1. Identify benefits and challenges of gene transfer trials for rare diseases.
2. Examples from the mucopolysaccharidoses (MPS).
3. Learn about the science behind gene transfer including the different viral vectors.
4. Describe potential adverse effects and problems in using gene therapy in real world setting.