Expanding the use of patient-derived organoids to guide personalized treatment in pediatric cancers: a pilot study

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PROJECT CONTEXT / INTRODUCTION

- Pediatric cancers with high incidence of relapse or with limited therapeutic options such as rare cancers remain a major clinical challenge. The survival rate remains low and clinical teams rapidly need data to tailor the treatment based on the patient specific tumor.
- Precision oncology approaches based on genomic profiling helped personalizing the treatments of pediatric cancers but do not always translate into effective treatment strategies. In some cases, no specific druggable targets are identified or no evidences support the choice of a treatment between many options.
- Patient-derived tumor organoids provide a functional ex vivo model to complement molecular data and explore therapeutic vulnerabilities. This model consist of "mini-tumors" grown in vitro and offers many advantages such as:
  - High similarity to the patient cancer cells with retention of the mutation profile.
  - Can be grown and amplified to allow many drug testing. Easily adaptable to medium and high-throughtput drug screening.
  - Relatively low cost and does not need complex infrastructure.
  - Can be easily used and shared with research laboratories to advance knowledge on pediatric cancer.

This project aims to structure, standardize, and expand the use of pediatric organoids in Canada within a clinically relevant and ethically robust framework, with the potential to inform personalized therapeutic strategies.

GOAL

- To expand patient-derived tumor organoids (PDTOs) as a feasible and scalable platform to support personalized treatment strategies for hard-to-treat pediatric cancers.
- Establish a robust culture protocol design that will allow the growth of organoid models with high efficiency for almost all type of pediatric tumors.

OBJECTIVES

- Establish pediatric PDTO lines from diagnosis or relapse samples
- Validate model fidelity using morphological and molecular comparisons
- Perform exploratory drug sensitivity testing (DST)
- Enable knowledge sharing through a structured organoid biobank

STUDY METHODS

IMPACT / OUTCOMES

- Demonstrates feasibility of pediatric organoid establishment
- Captures intra-patient tumor heterogeneity
- Integrates developmental controls to strengthen interpretation of exploratory and guided drug sensitivity testing
- Opens avenues for integrating organoid-based functional testing into personalized treatment strategies in pediatric oncology.

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TIMELINE

Phase 1 – Platform consolidation & pilot cases (Summer 2025 – Winter 2025)

- Finalization of BOP governance, SOPs, and ethical framework
- Establishment of pediatric organoid workflows across different tumor types
- Pilot cases to validate feasibility and robustness

Phase 2 – Comparative characterization & functional assays (Spring 2026 – Fall 2026)

- Morphological and molecular validation of organoids versus primary tumors
- Implementation of drug sensitivity testing (DST) workflows
- Refinement of interpretation framework for functional results

Phase 3 – Expansion & network integration (Fall 2026 – Early 2027)

- Continue expansion with additional pediatric tumor types and cases
- Integration within the Pediatric Preclinical Modelling program
- Standardization of data reporting and sample sharing procedures
- Generation of pilot data to support future prospective studies

End of project milestone (March 2027)

- Consolidation of results and platform performance metrics
- Dissemination of findings within PCMM / ACCESS networks
- Positioning of the project for sustained funding and long-term integration into pediatric precision oncology programs