

2nd Annual Meeting 2ème Assemblée Annuelle

E-Posters

January 28 - 29, 2025
28 - 29 janvier 2025



Explore the ACCESS e-posters

Research Theme Posters

- [Cancer Biology](#)
- [Clinical Trials](#)
- [Access to Innovative Therapies & Optimal Care](#)
- [Regulation, Policy & Economics](#)
- [Education & Training](#)
- [Ethical, Legal and Societal Issues & Implementation Science](#)
- [Psychosocial & Survivorship](#)

Research Group Posters

- [Knowledge Mobilization Group](#)
- [Social Justice, Indigenization & Inclusion Committee](#)

Research Project Posters

- [A Cross-Sectional Study of Childhood Cancers Treated with Proton Radiation Therapy in Canada](#)
- [ACCESS Presents: Education Sessions and Webinars](#)
- [Advancing Inclusive Youth-Led Research in Pediatric Cancer: A Participatory Action Research Project](#)
- [An Assessment of the Knowledge Mobilization Needs of the Childhood Cancer Community in Canada](#)
- [Deciphering 3D Chromatin States Permissive to Driver Alterations in High-Grade Childhood Brain Tumors](#)

- [Developing a Molecular Pathology Strategy](#)
- [Diagnostic and Precision Intervention for Leukemia Arising in Infancy](#)
- [Identifying the Needs of the Canadian Pediatric Cancer Community to Inform the Creation of Educational Resources](#)
- [Investigating the Impacts of Generative AI in Pediatric Oncology: Identifying Ethical-Legal Best Practices and Addressing Patient Community Concerns](#)
- [MIRV: A Phase I/II Study of Mirdametinib and Vinblastine for Newly Diagnosed Patients with Pediatric Low-Grade Glioma and Activation of the MAPK Pathway](#)
- [Pan-Canadian Approaches to Sharing Research Data and Fostering Access by Participants](#)
- [PRrecision Oncology For Young peopLE \(PROFYLE\): A Pan-Canadian Precision Medicine Platform](#)
- [Proton and Photon Consortium Registry \(PPCR\): Enabling Canadian Participation](#)
- [Psychosocial Resources and Standards of Care Across Canadian Pediatric Oncology Centres](#)
- [The Development of a Canadian Pediatric CAR-T Cell Network](#)
- [The National Pediatric Oncology Drug Access Navigator \(DAN-PO\) and the DAN-PO Database](#)
- [The Nationwide Adoption of an Electronic Survivorship Care Plan](#)
- [The Pediatric Cancer Models & Mechanisms \(PCMM\) Network: A National Research Platform](#)
- [Understanding Access to High-Cost Novel Cancer Therapies Across Canada: A Survey of Pediatric Oncology Providers](#)

Sonia Cellot, Sarah Cook, Nada Jabado, Amjad Kayali, Philipp Lange and Nathalie Therrien

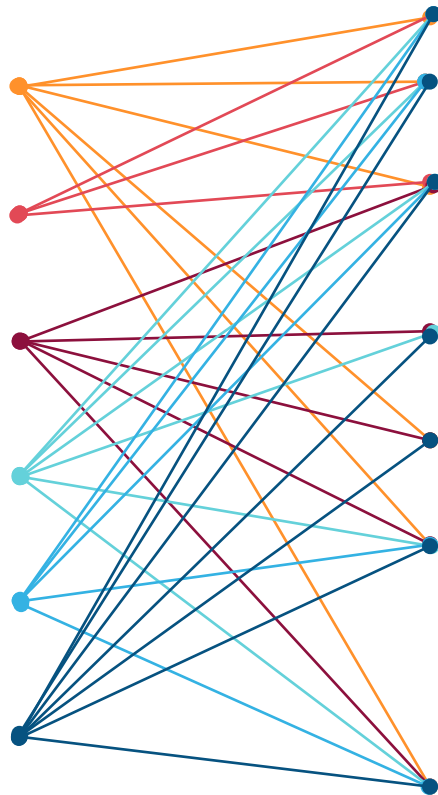


OBJECTIVE

To better understand the biology of pediatric cancers and accelerate research efforts by removing barriers to collaboration, increasing research pathways and building infrastructure to enable knowledge, expertise and data sharing across Canada.

FOCUS

- THERAPEUTIC TARGET DISCOVERY
- NATIONAL RESEARCH PROJECTS
- PLATFORMS
- BIOBANKING
- PERSONALIZED MEDICINE
- EQUITABLE ACCESS & PWLE ENGAGEMENT



OUR PROJECTS

- Brain Tumor
- Sarcoma MetAstasis Research Taskforce (SMART)
- Infant Leukemia
 - Functional Precision Medicine (FPM) Tumor Board
- Biobanking Network
 - Virtual Biobanking Platform
- Pediatric Cancer Models & Mechanisms (PCMM) Network
- Molecular Pathology Strategy
 - Molecular Pathology Board (MPB)
 - National Pediatric Proteomics Centres (NPCCs)
 - Liquid Biopsy Committee
- Modelling Consortium

PROGRESS & MILESTONES

5 of 7 projects approved **B S I P M**
 2 of 7 proposals under review **B M**
 Platforms are actively funding projects and reviewing patient cases **P M**
 Preliminary data generated **B S M**
 PWLE actively engaged **B S I P M B**

ADDITIONAL INFORMATION

View our project posters for 4 of our projects **B I P M**
 Listen to the AGM spotlight presentation on the SMART Project **S**
 Scan the QR code to view the current Cancer Biology Theme newsletter.





Stephanie Badour, Lorena Cook, Norman Cook, Rebecca Deyell, Daniel Morgenstern, Sébastien Perreault and Kirk Schultz

Objectives

To effectively and sustainably increase the short- and long-term national pediatric cancer clinical trial portfolio in order to ensure broader access to state-of-the-art diagnostics and therapies in Canada by: I) supporting Canadian-led academic trials that are ready to initiate within the year; II) developing and implementing the necessary programs and supports to provide investigators with the expertise needed to develop future Canadian-led investigator initiated trials; and III) establishing the framework for a national sponsor model for the conduct of future investigator-initiated trials in order to streamline their initiation and ensure that they widely accessible across Canada.

Progress & Milestones

Funded

- **Canadian-Led Investigator-Initiated Trials:** DECRYPT, [MIRV](#), OPTIMISE and VICTORY trials
- **Other Academic Trials:** Participation of Canadian centres in the Children's Oncology Group's AALL2131 trial
- **Trial Capacity Initiatives:** Development of a [Pediatric CAR-T Cell Therapy Network](#) to establish CAR-T cell manufacturing sites across Canada, [DAN-PO](#)

In Review/Revision

- **Canadian-Led Investigator-Initiated Trials:** Clinical trial incubator initiative to provide centralized expertise and supports for trial proposal or protocol development; Funding program for Canadian-led investigator-initiated pilot trials
- **Trial Capacity Initiatives:** Collaboratively developing national sponsor capacity with C17 (e.g., centralized REB and monitoring support, template agreements, centralized pediatric cancer DSMB, review of national trial infrastructure needs, etc.)

Anticipated Impact

Short-Term

The national trial portfolio will immediately expand as the exemplar trials are activated, and the community will gain greater confidence in and capacity to develop and conduct investigator-initiated trials.

Long-Term

Even greater trial availability across Canada will result upon the implementation of the necessary expertise, infrastructure and resources for further Canadian-led investigator-initiated trials to be developed and conducted, as well as the resources and supports needed to increase site capacity.



Norman Cook, Geoffrey Cuvelier, Paul Gibson, Lisa Goodyear, David Mitchell, Vijay Ramaswamy, Patrick Sullivan and Derek Tsang

Core Priorities



Priority 1: Improve Access to Clinical Trials

- Fund *travel to trials*
- Define *gaps and barriers* to build business cases and trial capacity
- Advocate for proper coverage of routine medical costs associated with participation



Priority 3: Improve Patient Navigation

- Support *patients and families needing access* to care, therapies or trials

Priority 2: Improve Access to Innovative Therapies

- Promote *data collection across the spectrum of diagnosis, treatment and survivorship* to build evidence and promote access and funding for innovative therapies
- Identify and address *regulatory and policy barriers* to access
- Improve *pathways of academic trials/real world evidence* to inform health technology assessments



Supported Projects

Funded

- [A Cross-Sectional Study of Childhood Cancers Treated with Proton Radiation Therapy in Canada](#) (Priority 1)
- [National Drug Access Navigator for Pediatric Oncology](#) (Priorities 1, 2 and 3)
- [Proton and Photon Consortium Registry](#) (Priority 2)
- [Understanding Access to Novel Cancer Therapies Across Canada – A Survey of Pediatric Oncology Providers](#) (Priority 2)
- The National Expansion of the STEP-1 Pilot Initiative – Supporting Travel for Pediatric and Adolescent Patients to Access Academic Phase I/II Trials in Canada (Priority 1) ***Learn more at our spotlight presentation on Tuesday, January 28 at 9:45AM ET!**

In Review

- *Access to Approved Medicines*: Develop a position paper that describes: I) the status in Canada of drugs that have received regulatory approval in the US; and II) the unsuitability of Canadian regulatory and health technology assessment processes for drugs with a pediatric cancer indication (Priority 2)
- *Innovation Sandbox*: Establish a separate ‘test’ environment that mimics the ‘real’ environment for the testing and evaluation of innovative ideas within an existing ecosystem (Priorities 1 and 2)
- *Patient Navigation Initiative*: Conduct an environmental scan and needs assessment of existing patient navigation programs and resources and any remaining needs to guide the development of other patient navigation resources for the pediatric cancer community in Canada (Priority 3)
- *Pediatric Cancer Travel to Treatment Study*: Survey to understand the financial, physical and social impact of travel to treatment on the pediatric cancer community (Priority 2)



Yvonne Bombard, Avram Denburg, Beverley Essue, Valerie McDonald and Keith McIntosh

Objectives

Develop and execute a rigorous program of health policy and systems research to enable evidence-informed policies and programs for equitable, efficient and sustainable child, adolescent and young adult (CAYA) cancer care in Canada

Progress & Milestones

Active Projects

Precision Diagnostics: Analyze State of Health System Implementation for Genome Diagnostics in Childhood Cancer Care

- Interviews and comparative policy document review, mapping of cross-provincial diagnostics landscape ongoing

CATCH Framework: Develop and Implement a Child-Tailored Health Technology Assessment (HTA) Framework

- Framework published, pilot implementation at Canada's Drug Agency (CDA) planned

Proposed Projects

- Precision Oncology Decision-Making: Discrete Choice Experiment on Determinants for Uptake of Sequencing Results into Clinical Care (MTB DCE)
- Equity of Short- and Long-Term Economic and Social Outcomes Amongst Patients/Families Impacted by Childhood Cancer
- C17-Health Canada Bilateral Meeting Program on Clinical Trials Regulation (C17-HC)
- Interprovincial Care of Children with Cancer in Maritime Canada (Maritime Care Case Study)

Anticipated Impact

- Precision Diagnostics: Recommendations to harmonize pathways in access to genome diagnostics for CAYA with cancer in Canada
- CATCH Framework: Implementation of first child-tailored HTA framework in HTA committee reviews at CDA
- MTB DCE: Strategies for effective shared decision-making for youth with hard-to-cure cancers in precision oncology programs
- C17-HC: Optimize regulatory and policy environment for conduct of pediatric cancer trials in Canada
- Maritime Care Case Study: Policy recommendations to support inter-provincial healthcare delivery for children with cancer





Chiquita Hessels, Dawn Pickering, Meera Rayar and Laura Wheaton

Objective

To improve access to education and training for Persons With Lived Experience (PWLE) and Early Career Clinicians (ECC) and Early Career Researchers (ECR) to better pediatric cancer care and research

Anticipated Impact

- Increased advocacy for the needs of PWLE affected by pediatric cancer
- Increased collaboration and sense of community for PWLE, ECC and ECR
- Increased innovation within pediatric cancer training, advocacy and research
- Enhance the next generations participation in leadership, research and advocacy



Proposed

- ACCESS Virtual Summer 2025 Research Program for trainees
- Online Patient Learning Institute and the development of training modules
- ACCESS PWLE & Healthcare Professional Mentoring Program
- Continuation of Children's Oncology Group (COG) KidsCare App for all Canadian pediatric oncology centres
- Pediatric Hematology/Oncology (PHO) Career, Education and Scientific Development Trainee Conference
- Program of Evaluation for Theme 5 Research, Career Development and Advocacy Curricula (proposed)

Goal

Improve access to education and training for PWLE and ECC and ECR to better pediatric cancer care and research

Approved

- ACCESS Presents Educational Webinars
- Funding six Canadians at the Eureka Certificate Course in Childhood and Young Adult Cancers in Toronto Spring 2025

Implemented

- Education and Training Subsidy funding program for PWLE
- Patient and Family Education Community of Practice for nurses across Canadian institutions






Antonia Palmer, Caron Strahlendorf and Ma'n Zawati

Objectives

- Contribute to the study of ethical, legal and societal implications of the work of the consortium
- Explore the opportunity to streamline access to data across Canada
- Study the impact of novel technologies in data-sharing practices

Approved Projects

-  Pan-Canadian approaches to sharing research data and fostering access by participants
-  Leveraging human rights to clarify the risk of genetic discrimination in pediatric oncology
-  Investigating the impacts of generative artificial intelligence in pediatric oncology: identifying ethico-legal best practices and addressing patient community concerns

Anticipated Impact

- Consistency in governance practices, data sharing, etc. across the consortium
- Anticipatory and informed practices on communication of patient data across Canada
- Use of responsible AI in data-sharing practices in pediatric oncology research in Canada



Leandra Desjardins, Vicky Forster, Lindsay Jibb, Paul Nathan, Sapna Oberoi, Fiona Schulte and Chantale Thurston

Objectives

To improve quality of life for pediatric oncology patients and survivors, including the prevention and management of short-and long-term side effects of pediatric and adolescent cancer treatment.

Progress & Milestones

- Implementation of electronic survivorship care plan (Passport for Care) for survivors of pediatric cancers, in collaboration with the Pediatric Oncology Group of Ontario (POGO)
- Evaluation of the psychosocial standards of care in Canadian pediatric oncology centres
- Scoping review and implementation of a digital comprehensive psychosocial screening tool in multiple centers across Canada
- Small-scale funding for trainees to explore new ideas and preliminary data generation for psychosocial and survivorship projects in Canada
- Understand the current landscape of survivorship care for childhood, adolescent, and young adult cancer survivors in Canada and develop a Canadian survivorship care and advocacy network

Anticipated Impact

- A national, systematic approach to psychosocial health screening during cancer trajectory
- A national, systematic approach to monitoring and managing late-effects in childhood cancer survivors
- Improved quality of life and outcomes of children with cancer during treatment and survivorship period



Michel Duval, Karen Haas, Stephanie Reid and Argerie Tsimicalis
 Postdoctoral Fellow: Emily Drake
 Research Staff: Tricia Schneider and Krishihan Sivapragasam

#ChildhoodCancer
 #KnowledgeMobilization
 #Survey

Assessment of Needs and Contexts

- ✓ Knowledge Syntheses, Environmental Scans, and Needs Assessment
- Assess Needs of Children and Other Vulnerable Communities



Maximize Visibility, Reach and Impact

- Connect Community Online, Understand Strengths, Identify Novel Opportunities
- Understand Website Needs
- Increase Social Media Followers
- Provide Social Media Training



Help Us Recruit 300 Voices!
 Send Us Your Hashtag!

Curate and Create Exemplars

- Curate Exemplars
- Create Child-Centric Materials



Create Sustainable Structures

- Build Capacity and Partnerships
- Conduct Network and Bibliographic Analyses of ACCESS
- Create a Sustainable Plan



Address Other Pressing Needs

- Listen and Respond to the Needs of Our Community

Stacey Marjerrison & Caroline Wai

The **Social Justice, Indigenization & Inclusion (SJII)** Committee is a passionate group of individuals who bring their diverse intersectional identities to this work.

The SJII Committee is dedicated to upholding the values of **reciprocity, equity** and **human rights** into our work. We share the responsibility to create a diverse and inclusive community and reduce inequities in pediatric cancer care. We advise the ACCESS network on applying an equity lens through patient and family centredness, collaboration, anti-oppression, innovation and excellence.

**WHO ARE WE?
WHAT DO WE DO?
WHY DO WE DO IT?**

HOW DO WE DO OUR WORK?

Community Engagement:

- Collaborating with the PWLE Network
- Building stakeholder networks:
 - **Indigenous Research Circle:** a group of expert collaborators to lead principles of Indigenous data governance, inclusion and project development

Community Education: Ongoing development of resources & education sessions for the ACCESS and Canadian pediatric cancer community

Project Review: Reviewing and providing feedback on all ACCESS projects



WHAT HAVE WE DONE SO FAR?

WHAT DO WE HAVE PLANNED NEXT?

- Created an equity statement for ACCESS
- Crafted a best practice guidance document for collecting sensitive information within ACCESS
- Created community diversity goals and assessed the diversity of our community
- Surveyed our research personnel for training needs
- Developed **education sessions**, including most recently at the annual Children's Oncology Group Meeting in September 2024

- Gather a list of **research engagement policies** for working with Indigenous communities, and principles with other equity-deserving populations
- Develop a list of **standardized questions** for assessment of sensitive sociodemographic information for use by the entire Canadian pediatric cancer community
- Develop **projects** in conjunction with PWLE and equity-deserving community members



Sylvia Cheng, Megan Sim, Sarah Hooseman, Andrea Lo, Samir Patel, Robert Nordal, Craig Erker, Paul D'Alessandro, Magimairajan Vanan, Chantel Cacciotti, Sébastien Perreault, Anne-Marie Charpentier, Sonia Skamene, Liana Nobre, Mary-Pat Schlosser, Lucie Lafay-Cousin, Kendrew Wong, Sharon Bulger, Aine Mooney, Emily Jewels and Derek S. Tsang



Introduction

Pediatric cancer patients in Canada are currently being referred to the United States for proton beam therapy (PBT) as part of their treatment due to its advantages, including a reduction of long-term toxicities. This out-of-country referral results in many incidental, out-of-pocket costs for patients and their families, including airfare/transportation, accommodations and meals. The patient and family experience and perspective, including the economic and financial impacts of an out-of-country referral and treatment, as well as barriers to the process, are largely unknown. The objective of this study is to: I) determine the potential barriers and factors that may affect patients receiving PBT; II) describe the barriers for healthcare providers to the referral and treatment with PBT; and III) evaluate the economic, financial and social impacts on patients and families who received PBT.



Methods & Design

- Patients and families, as well as healthcare providers (HCP), will be voluntarily surveyed
- Eligibility criteria: A) patient is 20 years old or younger at time of cancer diagnosis; B) treated at any of the 16 Canadian pediatric center; and C) received PBT with curative intent



Study Progress

- Received **48** completed HCP surveys (Oncologist [20]; Nursing [13]; Pediatrician [1]; Radiation Oncologist [4]; Neurologist [2]; Allied Health [3]; Medical Trainee [4]; Other [1])
- Patient and family surveys to launch in BC in **January 2025**, with the remainder of Canada to launch in Q2 or Q3 of 2025

Conclusions

This study will help us describe and better understand the barriers of PBT at both a patient/family and HCP level in order to better align current resources to support patients, families and HCPs before, during and after PBT. The information collected will assist in informing areas of improvement in the healthcare delivery among Canadian pediatric oncology programs as they continue advocacy work to acquire and distribute PBT care across the country for all pediatric cancer patients in Canada.

Future Directions

We hope to design future studies to further explore quality of life in patients and families. We plan to continue to engage and build networks among all provinces/territories and health communities to join in these future projects that would provide a deeper perspective on the impacts of PBT on patients and families.





Lead: Laura Wheaton

Collaborators: Chiquita Hessels, Dawn Pickering and Meera Rayar

Introduction

- ACCESS Presents education sessions support ACCESS' mandate by hosting free educational sessions for the pediatric oncology community throughout the year
- These sessions include research updates and insights from a variety of experts in paediatric cancer research, innovation, care and more

Progress

- Two successful webinars to date:
 - 'The Pediatric Cancer Models and Mechanisms (PCMM) Network' by Dr. Chris Maxwell
 - 'Application of Precision Oncology: From Clinical Trials, to Liquid Biopsy, and Beyond' by Dr. Marion Mateos
- To view previous webinars, please visit: bit.ly/ACCESSPresents

Suggest Future Speakers Here!





Antoine Boivin, Michel Duval, Ghislaine Rouly, Gwenvaël Ballu, Vera Granikov, Geneviève Castonguay, Élodie Bergeron, Jacob Randell, Adela, Helena Kirk, Sarah Calderwood and Vicky Forster

Overall Objectives

1 FOR ACTION
Support research projects led by youth living with cancer based on objectives they set themselves

2 FOR LEARNING
Support capacity-building for youth engagement in pediatric research

3 FOR SCIENCE
Advance knowledge on inclusive youth engagement in pediatric research

What We Have Achieved

What are the **HOPES** and **DREAMS** of youth living with cancer?

116 youth mobilized
3 workshops
1 young researcher



How to build **CULTURALLY SAFE** partnerships with Indigenous communities?

Partnership with the Atikamekw Nation from the Manawan nity



How to **COMMUNICATE SCIENCE** with youth?

2 young researchers
2 youth-led publications
1 keynote presentation at a national conference

How to **BUILD CAPACITY** for participatory research with youth?

Work in progress:
2 peer-reviewed manuscripts
1 toolbox for research teams
Stay tuned!



Argerie Tsimicalis, Michel Duval, Emily Drake, Sonia Angela Castiglione, Karen Haas and Stephanie Reid



Interested in Getting Involved in this National Project?



n = 300

- Join our Advisory Council
- Complete the Survey
- Send us Your Hashtag(s)
- Share the Social Media Posts
- Participate in a Focus Group or Interview
- Join Sub-Group Analyses (e.g., by ACCESS theme, cancer group or equity-deserving group)

Research Opportunity!

Are you a Canadian who ...

- has cared for a child who was diagnosed with cancer?
- has had a family member diagnosed with childhood cancer?
- is caring for a child who was diagnosed with cancer?



Do you work in pediatric oncology in Canada? If so, we would like to hear about your [#KnowledgeMobilization](#) needs! Please consider filling out our [#ChildhoodCancer](#) [#KnowledgeMobilization](#) survey!

Post Needs Assessment



- Assess Needs of Children and Other Vulnerable Communities
- Create Materials
- Maximize Online Visibility, Reach and Impact
- Create Sustainable Structures

Nada Jabado and Vijay Ramaswamy

High-grade brain tumors are devastating with a tremendous impact on patients, families and health care. Many survivors suffer from serious health issues that affect their quality of life and ability to lead fulfilling lives in society. Unfortunately, for some patients, the current diagnosis is often grim, with limited effective treatment options available.

Brain tumors have distinct genetic alterations and changes in the way DNA is organized (chromatin architecture) at the core of the processes that sustain tumor formation, but we have not fully understood what these changes represent and how to target them effectively. Research suggests that gaining a deeper understanding of how these 3D/4D chromatin changes function, as well as how tumor cells communicate with each other and with their surrounding environment, is crucial for developing better treatments.



AIMS

- Investigate how cancerous brain cells manipulate their chromatin architecture and influence their immediate surroundings
- Identify communication pathways within the tumor that can be disrupted with therapy to effectively kill tumor cells

RESULTS

In H3K27M-mutant glioma tumors, Polycomb Repressive Complex 1 (PRC1) member CBX2 is at the core of 3D genome architecture that maintains stemness. We are exploring CBX allosteric modulators that can be translated to the bedside.

PROJECT TEAM

Guillaume Bourque, Craig Erker, Jennifer Chan, Anne-Claude Gingras, Karen Haas, Claudia Kleinman, Philipp Lange, Simon-Luc Laporte, Sébastien Perreault, Magimairajan Issai Vanan and others.

ACHIEVEMENTS

- Diverse project team from across Canada (and internationally), including two PWLE;
- Biospecimens collected;
- Promising preliminary data

DELIVERABLES

- Consolidate the pediatric brain tumor network and provide expertise in various areas (i.e., spatial transcriptomics and proteomics, tumor microenvironment, and 3D/4D chromatin architecture);
- Acquire/integrate invaluable complex datasets;
- Identify novel targets in brain tumors and extend approaches to other pediatric and young adult cancers

ADDITIONAL INFORMATION

Scan the QR code to view the current Cancer Biology Theme newsletter



Developing a Molecular Pathology Strategy

Philipp Lange and Liana Nobre



Access to the next generation of high throughput sequencing platforms has improved how we diagnose, classify, and treat pediatric cancers. Thanks to sequencing research studies like PROFYLE, KiCS, SIGNATURE, and POG, we can now better identify specific genetic alterations in tumors. While many patients respond remarkably well to targeted therapies we don't always find actionable alterations and the response does not often last long term. There is significant untapped potential in advance molecular diagnostics to refine disease classification, treatment, and monitoring using complementary and less invasive strategies. The **AIM** is to identify and advance the next tests and platforms with clinical use and accelerate their move from research labs into everyday clinical practice to make them accessible for all children with cancer in Canada.

STEP ASSAYS

01 Identify promising assays and tools to advance



ASSAY VALIDATION & FEASIBILITY

Via research studies and health economics data



STEP 02

STEP 03

STANDARD OF CARE

Establish pipelines & engage policy makers to advance assays into clinical practice



OUR PROJECTS

NATIONAL PEDIATRIC PROTEOME CENTRES (NPPCs)

Proteome profiling is the large-scale study of proteins expressed by a biospecimen at a given time. It has been identified as a promising tool to find new treatment targets in cancer. NPPCs are being established in Canada to streamline the identification of targets and data supporting treatment decisions. A congruence study is underway.

LIQUID BIOPSY TESTING

Liquid biopsy samples like blood and urine are minimally invasive to obtain and promising to detect tumor-specific alterations released by cancer cells. They can be used to refine diagnoses, inform treatment decisions, monitor treatment responses and recurrence. Protocols are being harmonized and leaders are currently strategizing the best path forward.

MOLECULAR PATHOLOGY BOARD (MPB)

The MPB (i) supports the development of promising assays. It also (ii) discusses, recommends, and funds appropriate clinical assays for patient cases that would not otherwise be accessible, which may help diagnose, monitor, and treat patients. First patient cases were discussed by the MPB in January 2025.

Scan the QR code to view the current Cancer Biology Theme newsletter



Sonia Cellot



Recent advancements in genetics have revealed that pediatric leukemia are much more diverse than we previously thought, but this diversity is just starting to be considered in how we diagnose, treat, and monitor the disease. One major challenge is that we still do not have enough treatments tailored to each child's specific condition, which is why cure rates for the most fatal forms of leukemia remain low, and standard treatments can cause harmful side effects for survivors. "Infant leukemia", which arise in babies before 12 months of age, is a rare and highly varied form of leukemia with poor cure rates.

AIM

Forge a national multidisciplinary **functional precision medicine (FPM)** consortium, a team of expert from different fields, to generate and integrate chemo-genomic datasets to uncover new treatment biomarkers and guide biology informed treatment decisions. This team will produce patient management recommendations, establish research priorities, and engage multiple stakeholders, including PWLE, to significantly and positively impact international efforts in the field.

KEY DELIVERABLES

- Establish the FPM tumour board
- **Develop personalized treatment and diagnostic methods tailored to the unique genetic and functional traits of each disease**
- Ensure equitable access to baseline molecular profiling for all pediatric patients with leukemia
- **Discover new targets for treatment**



CURRENT FOCUS

- Harmonize protocols to collect and process biospecimens
- **Identify and collect infant leukemia patient samples for the project (target of 50)**
- Coordinate activities with other project teams (including the Biobanking Network and Modelling Consortium) in relation to biospecimen collection, drug sensitivity profiling, and the FPM tumor board

ADDITIONAL INFORMATION

Scan the QR code to view the Cancer Biology Theme newsletter

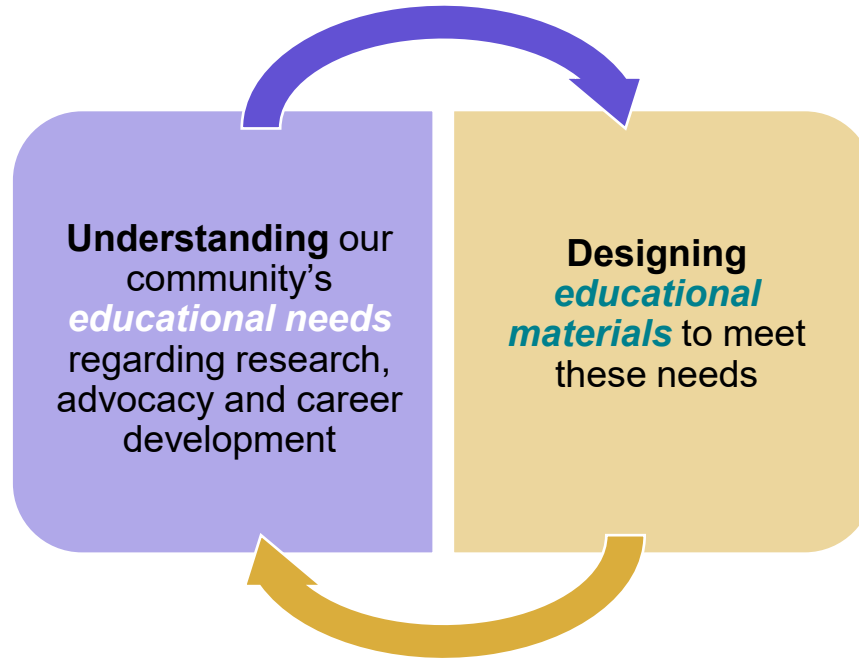




Chiquita Hessels, Dawn Pickering, Meera Rayar and Laura Wheaton

Background

- Education and training is essential to help patients and families navigate the pediatric cancer system and advocate for continuous improvement and change
- Similarly, career development and research training are pivotal for early career researchers and healthcare providers to help guide their way into the clinical and academic world



Goal

The aim of the project is to identify the needs of people with lived experience, as well as early career researchers and healthcare providers, regarding their involvement in pediatric cancer research and career development

Healthcare Providers



We invite you to share experiences and provide input to help to create educational content

Use the QR code to complete an Expression of Interest Form to join an upcoming focus group!



**Recruitment for Persons With Lived Experience Needs Assessment and Focus Groups will be shared soon!*



Co-Lead PI: Ma'n Zawati
Study Coordinator: Yuan Stevens
Collaborators: Antonia Palmer, Caron Strahlendorf

Introduction

This project explores the use of generative artificial intelligence (AI) in pediatric oncology. It will examine the ethical and legal issues associated with the use of generative AI in pediatric oncology and care.



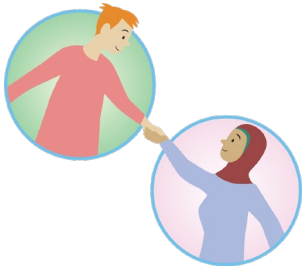
Methods & Design

This study employs the following methods:

- Scoping review of literature to better understand the landscape of generative AI and its use in pediatric oncology
- Focus groups with young people (14+) and parents from the pediatric oncology community to explore their trust, perceptions, concerns and recommendations about generative AI in pediatric oncology

Anticipated Impacts

We will deliver a comprehensive information guide on generative AI in pediatric oncology tailored for patients, researchers, and clinicians in the ACCESS community. Partnering with PWLE, we will create an ethico-legal guidance document to benefit ACCESS stakeholders and the broader pediatric oncology field in Canada and beyond. Our research will also culminate in an academic article and a presentation at a leading scientific conference.





Principal Investigator: Sébastien Perreault

Co-Investigators: Sylvia Cheng, Craig Erker, Lucie Lafay-Cousin, Geneviève Legault, Liana Nobre, Samuele Renzi, Uri Tabori, Stéphanie Vairy, Magimairajan Issai Vanan

Biological/Biomarkers: Benjamin Ellezam, Nada Jabado, Cynthia Hawkins, Uri Tabori

Radiology: An Lessage



Sponsor and Coordinating Centre: CHU Sainte-Justine

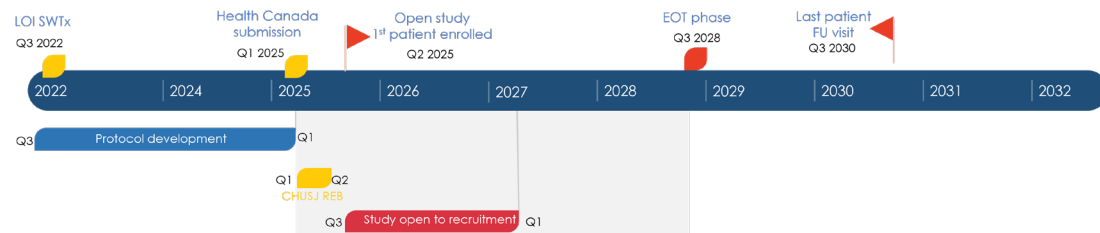
Central Data Operations: CSGAD at CHU Sainte-Justine

Study Coordinators: Bianka Courcelle, Sabrina Cerro

Implementation of ACCESS Core Values:

- **Equitable access** to MIRV trial
 - **Satellite sites** are directly included in the protocol
 - **CRAFT tools** will be used
- Commitment to **EDI** principles
 - To follow ACCESS **Guidance Document on the Collection and Use of Potentially Sensitive**
- **PWLE involvement**, including on trial's DSMB

Study Timeline:



Study Overview

Study Design: Multi-Center, Phase II, Open-Label, Non-Randomized Trial

Sample Size: 50 Patients

Clinical Sites: ± 10 Canadian Centers

Main Eligibility Criteria: Patients ages 2 years to 25 years at time of study enrollment who require upfront treatment of pediatric low-grade glioma

Feasibility Phase: The maximum tolerated/recommended phase II dose (MTD/RP2D) of the mirdametinib plus vinblastine combination will be assessed using a modified Rolling-6 design

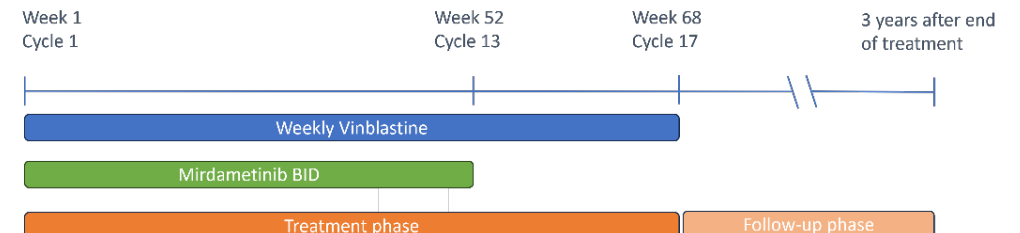
Treatment Phase: Oral mirdametinib twice daily at a fixed dose (2mg/m²) + weekly IV vinblastine at MTD will be given for a total of 17 cycles

Study Follow-up: Every 6 months for 36 months

Primary Objectives: To determine the: I) maximum tolerated dose; and II) objective response rate

Secondary Objectives: To evaluate the: I) efficacy outcome measures (progression-free survival, time to progression and overall survival); II) safety and tolerability; III) effect on neurological evolution; and IV) quality of life during treatment

Exploratory Objectives: To evaluate the response rate based on tumor volume, as well as to investigate and correlate the following biological features to tumor response: I) gene expression; II) DNA methylation; III) RNA expression; and IV) cerebrospinal fluid circulating tumor DNA





Co-Lead: Ma'n Zawati
Study Coordinator: Terese Knoppers
Collaborators: Antonia Palmer, Caron Strahlendorf

Introduction

Fragmented and siloed data, coupled with the inability to share it across platforms, institutions, and provinces, hinders pediatric cancer research and its clinical implementation. This qualitative project will address important data sharing bottlenecks pertinent to the mission of ACCESS.

Methods & Design

This project has two branches:

- Survey of institutional legal representatives on better facilitating data-sharing across ACCESS member sites/institutions
- Survey of adult survivors of childhood cancer and parents from the pediatric oncology community on improving access to patient clinical/research healthcare data



Anticipated Impacts

We will produce both academic and practical deliverables towards data sharing and access for patients, caregivers, researchers, healthcare professionals across Canada. This includes two articles as well as informational tools and template data request and sharing forms for PWLE and researcher stakeholders.



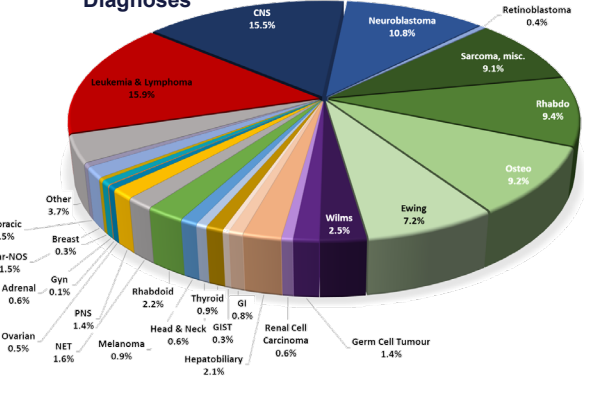
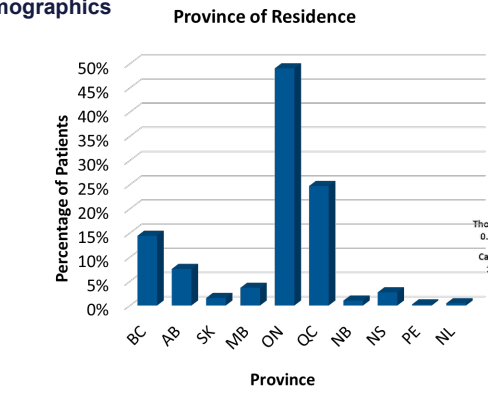
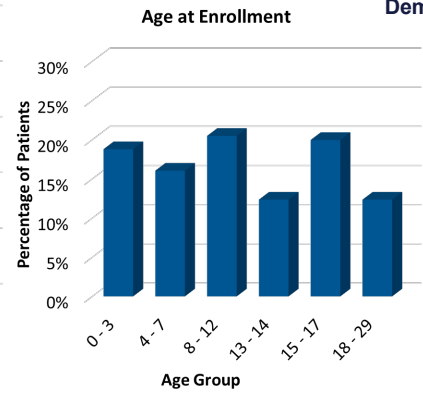
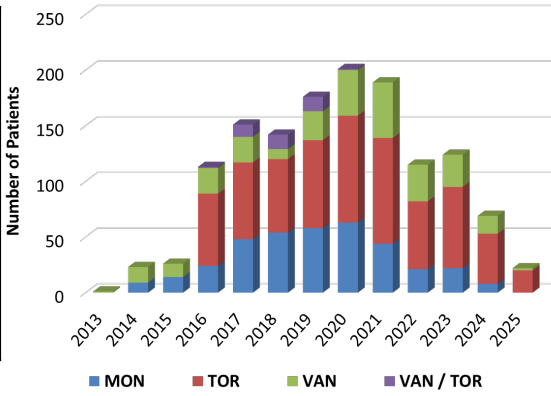


The PROFYLE Leadership Committee

PROFYLE BACKGROUND

- The national collaborative program was created and launched in 2017, with the goal to develop and implement a pipeline providing equitable access to next-generation molecular tools and cancer model systems to identify disease- and patient-specific biomarkers that are tractable/novel targets for therapy in a clinically relevant timeframe for children, adolescents and young adults (CAYA) with refractory, relapsed and metastatic ('hard-to-cure') cancers
- Prior to PROFYLE, the Personalized Oncogenomics Project (POG) (Vancouver), SickKids Cancer Sequencing Program (KiCS) (Toronto), and Personalized Targeted Therapy in Refractory or Relapsed Cancer in Childhood (TRICEPS) (Montreal) constituted the bulk of childhood precision oncology efforts in Canada. PROFYLE was designed to unite and build upon them
- The PROFYLE consortium, which includes >20 institutions including all 16 Canadian pediatric cancer centres, has united an interdisciplinary team of experts, leaders, clinicians, researchers, patient and family end-users and advocates from across Canada

| | Winter 2024 |
|--|--------------|
| PedsPOG/PROFYLE (Vancouver) | 279 |
| TRICEPS/Signature/PROFYLE (Montreal) | 365 |
| KiCS/PROFYLE (Toronto) | 669 |
| KiCS/PedsPOG/PROFYLE (Vancouver/Toronto) | 39 |
| Total | 1,352 |



ACCESS' PRECISION MEDICINE PLATFORM DRIVER PROJECT

- In early 2024, PROFYLE became a signature driver project within ACCESS
- Including PROFYLE as a driver project:
 - Helps address a gap in activities in ACCESS related to genomic medicine and molecular profiling
 - Allows for data, infrastructure, platforms and processes generated from PROFYLE to be utilized within ACCESS
 - Reduces duplication of efforts and redundancies and facilitates collaboration and connection between stakeholders

For more information, please contact: profyle.program@sickkids.ca



Alejandro Shiri Moreno*, Hitesh Dama*, Andrea Lo, Yina Shan*, Samir Patel, Natalie Logie, Marwan Tolba, Carol Oliveira, Sara Dennehy, Torunn I. Yock and Derek S. Tsang*

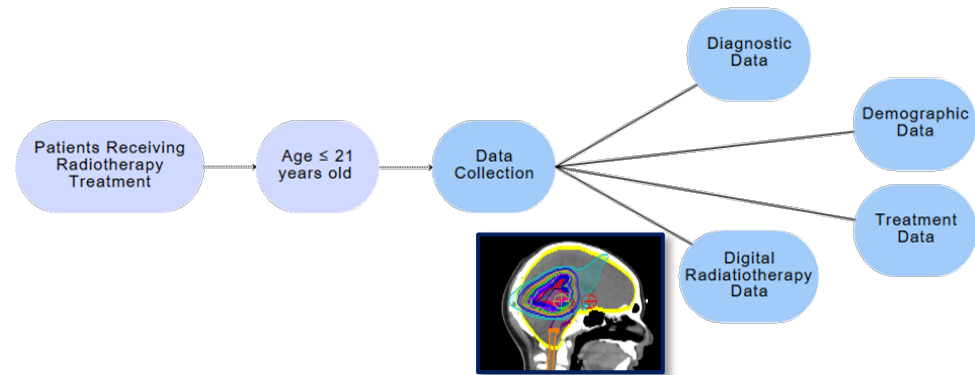
*Princess Margaret Cancer Centre and The Hospital for Sick Children, Toronto, Ontario, Canada

Introduction

Results – Current Canadian Enrollment

Canada currently lacks a national registry for tracking children receiving proton or photon radiotherapy. The Proton/Photon Consortium Registry (PPCR) is an international effort to standardize data collection and analyze treatment outcomes in pediatric patients.

Methods & Design



n = 114

 n = 59

 n = 55

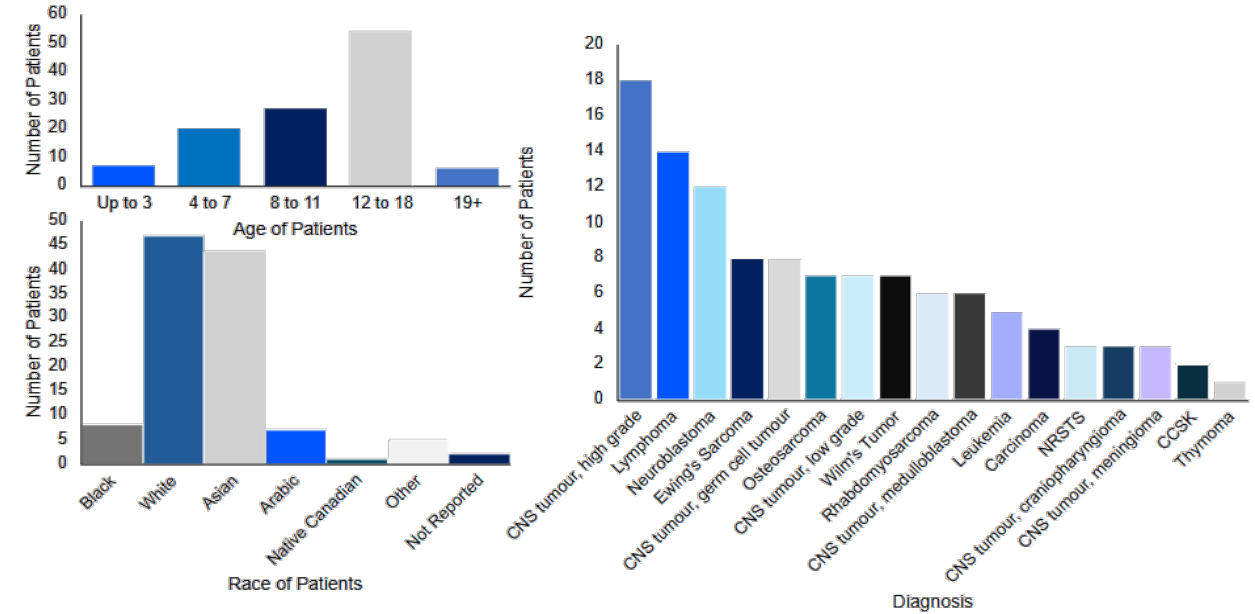


Figure 1. Patient Demographics from the PPCR at Princess Margaret Cancer Centre. Data includes the number of patients, sex, age, race and diagnosis.

Ongoing Activities

Toronto leads Canadian registry participation with 114 patients (as of 30 November 2024).

The study is currently being opened in **Calgary, Edmonton, Montreal and Halifax**.



The study opened in **Vancouver** this year, with 7 participants registered (as of 30 November 2024).



Toronto (Yina Shan) is using Canadian PPCR data to investigate patterns of proton beam therapy utilization and follow-up care in pediatric patients. Data analysis is ongoing.





Leandra Desjardins, Nadège Gendron, Lindsay Jibb, Fiona Schulte, Chantale Thurston, Victoria Forster, Paul Nathan and Sapna Oberoi

Introduction

The Standards for Psychosocial Care for Children with Cancer were initially published in 2015 and outlined 15 domains of evidence-based psychosocial care that should be provided to children diagnosed with cancer and their families. However, many years later, it remains unknown the extent to which of the pediatric cancer programs in Canada are delivering care consistent with the Standards. The Psychosocial Standards of Care study, therefore, aimed to: 1) map the availability of pediatric cancer centre psychosocial resources across Canada; and 2) assess the provision of care aligned with the Psychosocial Standards across pediatric cancer programs in Canada.

Methods & Design

- Online REDCap survey, in which the content was based on previous work on adherence to Psychosocial Standards in the United States, with modifications to reflect the Canadian context (e.g., questions about availability of resources in French).
- Participation from all 16 centres in Canada a goal; at least one medical (e.g., oncologist, nurse) and one psychosocial (e.g., social worker, psychologist) healthcare provider from each centre.
- Recruitment window from July 2024 to end of January 2025.



Preliminary Results

Impact

The study's results will :

- ✓ Inform implementation efforts;
- ✓ Support advocacy for psychosocial resources;
- ✓ Facilitate knowledge sharing and equitable access to care; and
- ✓ Provide a baseline evaluation that will then allow assessment of progress as ACCESS focuses on implementing various psychosocial projects related to the Standards.





Joerg Krueger, Henrike Bittencourt, Michel Duval, David Mitchell, Ashley Chopek, Divya Subburaj, Greg Guilcher, Victor Lewis, Ravi Shah, Geoff Cuvelier, Sunil Desai, Nicole Prokopishyn, Audi Setiadi, Mohamed Abdelhaleem, Karin Hermans, Erilda Kapplani, Kirk Schultz and Amanda Li
on behalf of the *Cell Therapy Transplant Canada Pediatric Committee*

Introduction

Chimeric Antigen Receptor (CAR) T-cell Therapy has improved outcomes for patients with B-cell malignancies, **however:**

- Significant barriers exist in delivery of this complex and costly therapy to children across Canada
- Further innovation is needed to improve the long-term success of current CAR T-cell therapies and expand this powerful technology to treat other pediatric diseases
- Pediatric cancers are comparatively rare and industry R&D increasingly focusing on adult malignancies, increasing reticence to commit to pediatric trials (Rossig *et al.* JCO 2024)

Aim No. 1: Develop Point-of-Care CAR T-Cell Manufacturing

- Develop experience and expertise to manufacture CAR T-cell products using a commercial closed-circuit cell processing system at five (5) Canadian pediatric institutions
- Validation of CAR T-cell manufacturing, product release and quality assurance testing

Aim No. 3: Advance Post CAR T-Cell Disease Monitoring

- Bring Next-Generation Sequencing Minimal Residual Disease (NGS MRD) testing to Canada by validating post- CAR T-cell therapy samples

Aim No. 2: Improve Equitable Access and National Standards of CAR T-Cell Therapy Delivery

The Canadian Pediatric CAR T-cell Network (CPCN) will:

- Bring together local-regional pediatric oncologists, cellular therapy/bone marrow therapy care teams, Persons With Lived Experience and advocates
- Develop consensus recommendations and practice standards regarding pediatric CAR T-cell care, provider training resources, and create infrastructure to facilitate patient access to standard CAR T-cell therapy, CAR T-cell clinical trials and innovation



Sandra Judd, Patricia Schneider, Stephanie Grover, Valerie McDonald, Vijay Ramaswamy, Stephanie Villeneuve and Rebecca Deyell

Introduction

Most children accessing innovative therapies for cancer in Canada do so **outside conventional clinical trials**. Pediatric oncologists were poorly supported, especially at smaller sites, to navigate the complex pathways required to advocate for their patients to access therapies. The DAN-PO role was created with a **national mandate** to address this growing need.

Progress & Milestones

Prior Deliverables Achieved

- Assessed and published retrospective innovative therapies landscape¹
- Creation of innovative drug access database creation (internal to SickKids)
- Established a Drug Access Community of Practice

Current Ongoing Support

- Investigator-initiated trials (IITs) and single-patient studies (SPSs) protocol development
- Individualized support for compassionate access or alternate pathways for commercial drugs

Main Deliverable for Q3/4 2024

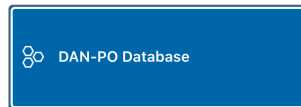
- In December 2024, launched the DAN-PO External Database
- Site champions were identified and used to disseminate information at the local level

Future Directions for DAN-PO Role

The upcoming year's work will include: I) collecting DAN-PO Database feedback, as well as data on metrics and user integration; II) supporting the development of IITs and SPSs; III) leading Drug Access CoP develop advocacy efforts for drug access in pediatric oncology; and IV) developing a plan for the targeted prospective collection of real-world data for innovative therapy use to support evidence base.

¹Judd S, et al. *Cancer Med.* 2024 Feb;13(3):e7033. PMID: 38400668.

The DAN-PO Database



Due to the proprietary nature of the information stored in the DAN-PO database, only given access.

Click here to access the [DAN-PO Database User Guide](#).

Any questions, comments, or drug information updates, please contact National Pediatric Oncology Drug Access Navigator (DAN-PO) at sandra.judd@sickkids.ca.

Other available drug access resources:

- [Advancing Childhood Cancer Experience, Science & Survivorship \(ACCESS\)](#)
- [U-Link Canada](#)
- [Health Canada Special Access Program Drugs](#)
- [Health Canada Drug Database](#)
- [Health Canada Drug Submissions Under Review](#)
- [Canada's Drug Agency CDA-AMC \(formerly CADTH\)](#)
- [FDA Approved Drugs](#)

Any other useful links or resources? Please email, [Sandra Judd](mailto:Sandra.Judd).

Generic Name: **dabrafenib** Brand Name: Tafinlar Drug Class: RAF/RAS/MEK/ERK inhibitors Company: **Novartis** [Click for more info and contacts](#)

All formulations are marketed in Canada, no longer SAP. Peds pts > 1yr with LGG on BOTH dabrafenib and trametinib - enroll on Sentia Patient Support Program. All other patients -compassionate access requests through Novartis MAP.

Compassionate drug access requests can be made via GEMS, the online system for Novartis Managed Access Program. Please see link below. If approved, Novartis legal agreement is required. SickKids staff should submit to Legal for review and institutional signatures. A Legal Intake form for Compassionate Use (you). See template questions below.

Process steps (SK):

1. Novartis approval (on GEMS, MD will receive an email). Until dispersible tablet is marketed.
2. Novartis will send documents to MD to review + send legal agreement.
3. MD to complete and send SK Legal Checklist; Legal will get signatures once review done on agreement.
4. Novartis will send email with MD attestation to be done on GEMS once legal agreement done - MD to log on to do.
5. Novartis will authorize shipment of drug.

[Novartis GEMS system Login](https://www.cybergrants.com/novartis/maps)

[Novartis GEMS user guide](https://www.novartis.com/sites/www.novartis.com/files/novartis_gems_user_guide.pdf)

Sentia_PSP_Enrollment Form- ENG Interactive (1)

SickKids Legal Compassionate Checklist Nov 2020

Available Dosage Forms/Strengths (4) [Click the arrow to expand](#)

| Dosage form | Availability in Canada | DIN | Open DDI |
|--|------------------------|----------|--------------------------|
| > tablet for oral dispersion - 10 mg, package size: 35 tablets/bottle | Marketed/Available | 02540053 | |
| > dispersible tablet for oral suspension (marketted) - 10mg, package size: 210 dispersible tablets | Marketed/Available | 02540053 | |
| > capsule - 50 mg, package size: 120 capsules/bottle | Marketed/Available | 02409607 | |
| > capsule - 75 mg, package size: 120 capsules/bottle | Marketed/Available | 02409615 | |

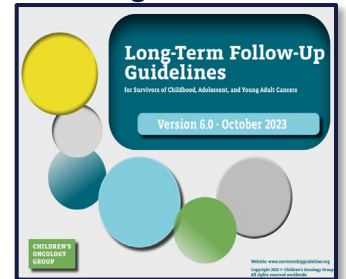


Paul Nathan in Collaboration with the Pediatric Oncology Group of Ontario

Introduction

There is broad consensus that all childhood cancer survivors require a treatment summary and **survivorship care plan (SCP)**. In North America, survivorship care is guided by the **Children's Oncology Group Long-Term Follow-Up (LFTU) Guidelines**. However, challenges to their implementation include:

- Lack of a national electronic platform for dissemination
- Paper-based SCPs cannot be updated, are often lost, and become outdated
- Current approaches to SCP building mostly require manual entry of treatment exposures
- Privacy laws have hampered the adoption of electronic platforms not housed in Canada

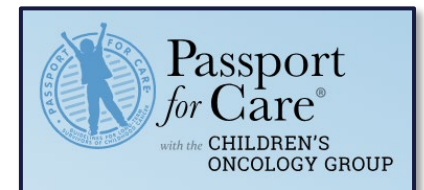


Opportunity No. 1: Cancer in Young People in Canada (CYP-C) Data Tool

- Collects diagnosis and treatment data from most Canadian pediatric cancer centers
- Captures essential exposure information needed to populate a survivorship care plan

Opportunity No. 2: Passport for Care (PFC)

- **Web-based** clinical support tool that generates **personalized SCP based on the COG LTFU Guidelines** maintained by Texas Children's Hospital
 - Eliminates the need for future medical record abstraction and referral to the guidelines during subsequent visits
 - Survivors and their families can view their SCP and share it with their primary or subspecialty medical providers
- Two Canadian provinces already using it (Manitoba and Nova Scotia)
 - Privacy concerns blocked launch in Alberta
- **ACCESS has supported a pilot project at the Pediatric Oncology Group of Ontario (POGO; 5 cancer centres) to work with PFC to overcome privacy/data storage barriers, create a platform for electronic transfer of data from CYP-C to PFC and offer enrollment to Ontario survivors**
- Will work to integrate CYP-C data transfer in Manitoba and Nova Scotia, **and plan for nation-wide adoption of the tool, including addressing provincial privacy concerns**



The Pediatric Cancer Models & Mechanisms (PCMM) Network: A National Research Platform

Project Lead: Chris Maxwell



OVERALL AIM: PROMOTE & ENABLE PRECLINICAL STUDIES IN PEDIATRIC CANCER ACROSS CANADA

OUTPUT 1: national **EXPERT REGISTRY** (50+ registered) linked to ~1,300 international experts

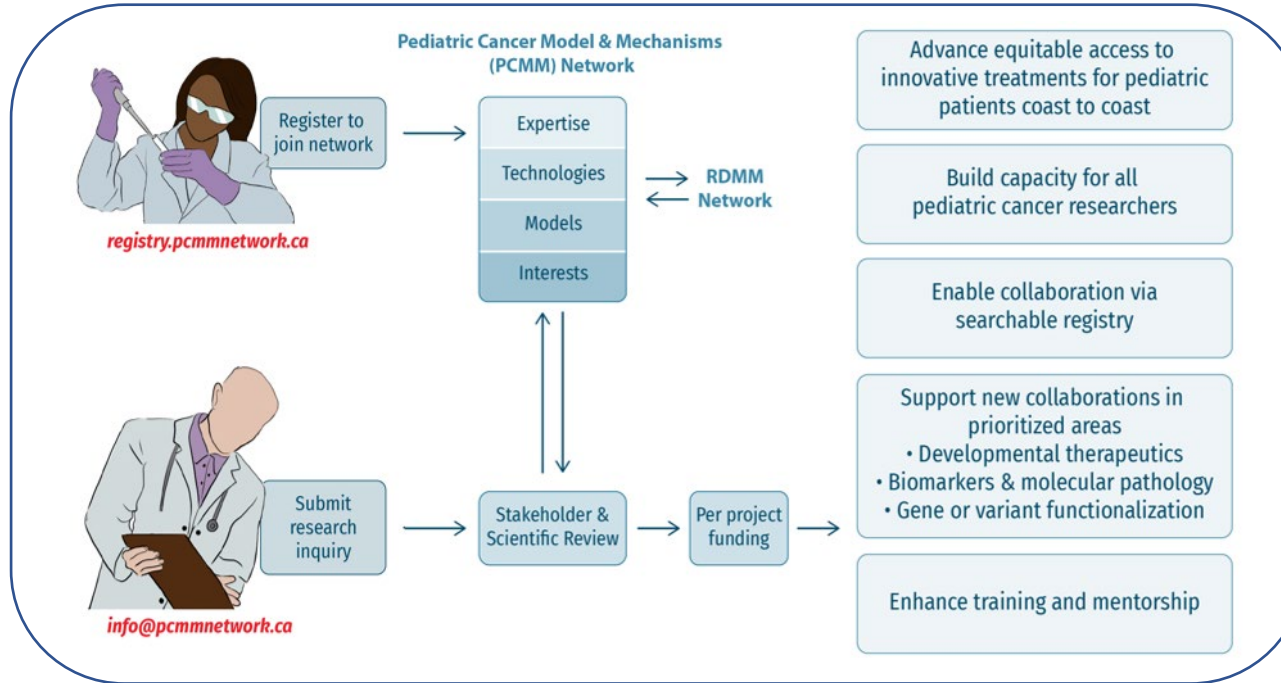
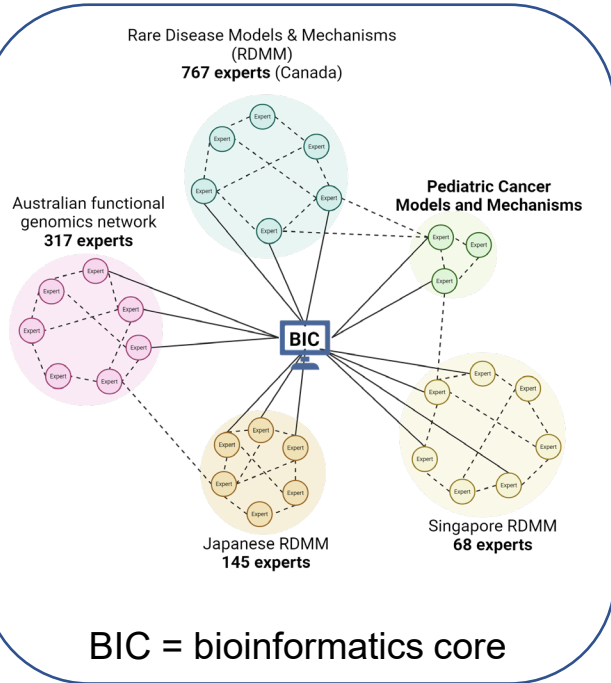
OUTPUT 2: pan-Canadian Governance and Scientific Oversight Committee + **MATCHING PROGRAM**

ROUND 1 (July 2024) supported 3 teams in (i) Developmental therapeutics, (ii) Biomarkers, & (iii) Gene function

1. EXPERT REGISTRY

2. MATCHING PROGRAM FOR TRANSLATIONAL RESEARCHERS

**COMING SOON:
PARTNERING ROUND 2**



Partnering Support: **up to \$70k**

Deadline: **March 14, 2025**

Visit www.pcmnetwork.ca for application details

Visit registry.pcmnetwork.ca to register your expertise





Kriti Kumar, Avram Denburg, Celine Cressman, Derek Tsang, Marcel Romanick and Paul Gibson

Introduction

- Targeted therapies, proton beam therapy, cellular therapy are high-cost therapies in Canada and not always publicly funded
- Provinces and territories differ in their funding review processes, leading to differing degrees of reimbursement for novel therapies
- Developed survey to explore disparities in access to four select high-cost novel therapies: blinatumomab, larotrectinib, proton therapy, cellular therapy

Methods & Design

- Online survey distributed to Canadian pediatric medical oncologists, radiation oncologists, pharmacists, hematopoietic stem cell transplant/cellular therapy physicians, nurse practitioners
- Four vignettes: blinatumomab for low-risk relapse of acute lymphoblastic leukemia (ALL), larotrectinib for a TRK-fused soft tissue sarcoma, proton therapy for an unresectable head and neck sarcoma, and cellular therapy for first relapse of ALL in a patient with Down Syndrome

Current Status

- Survey distributed through various networks and directly to pediatric oncology providers
- 70 respondents completed the survey
- Data analysis is ongoing

Future Directions & Acknowledgments

- Plan for the dissemination of results in the spring and summer of 2025
- Barriers identified in study will be used to identify specific institutional and health policy changes

Preliminary Results

| Novel Therapy | % of Time Therapy is Accessible (Mean, SD) |
|------------------|--|
| Blinatumomab | |
| Larotrectinib | |
| Proton Therapy | |
| Cellular Therapy | |