# 3rd Annual Meeting
# 3ème Assemblée Annuelle

March 9 - 11, 2026
9 - 11 Mars, 2026

# Advancing the Well-being of Childhood Cancer Survivors – Bringing the Passport for Care to Ontario

Advancing Childhood Cancer Experience, Science & Survivorship
Agir contre le cancer des enfants avec succès

CIHR IRSC Canadian Institutes of Health Research Instituts de recherche en santé du Canada

# Bringing the Passport for Care (PFC) to Ontario: Goals

Enhance long-term health outcomes for childhood cancer survivors in Ontario by implementing PFC - a secure, electronic platform that delivers personalized, evidence-informed survivorship care plans for lifelong follow-up

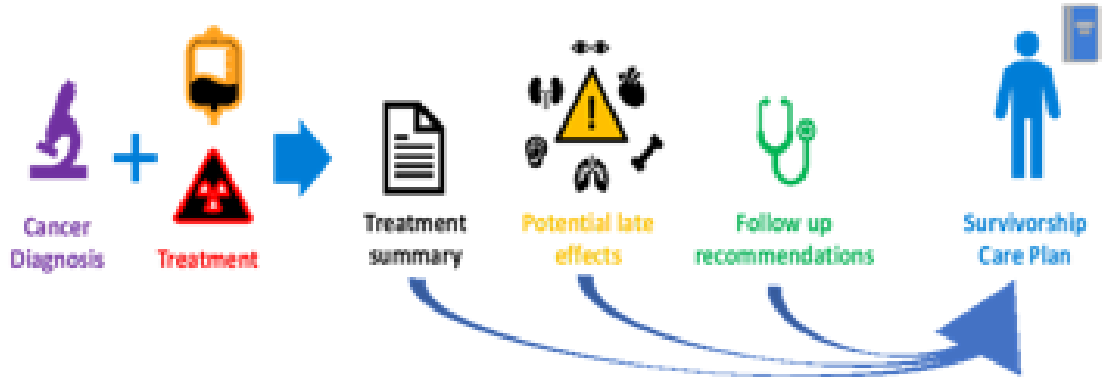
Use the Ontario roll-out as the foundation for Canada-wide implementation, including translation of resources into French (ACCESS #2)

**Lifelong Follow-Up for Childhood Cancer Survivors**

# Bringing the PFC to Ontario: Objectives

- Modernize survivorship care by transitioning from paper-based and manually maintained survivorship care plans to a secure, web-based system.
- Provide survivors and clinicians with personalized, risk-based follow-up guidance aligned with Children's Oncology Group (COG) Guidelines (updated in real time)
- Improve equity, continuity, and consistency of care across providers and regions while reducing administrative burden for clinicians.

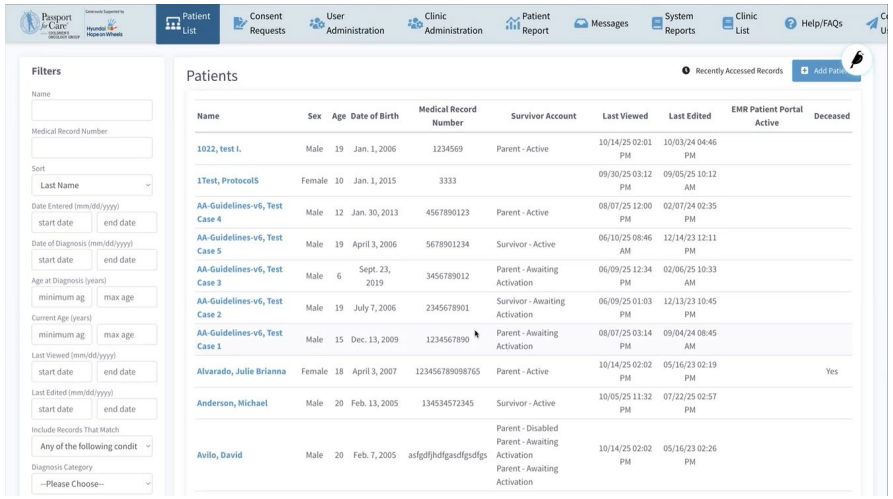
## From Diagnosis to Survivorship: Personalized Care Plan with PFC



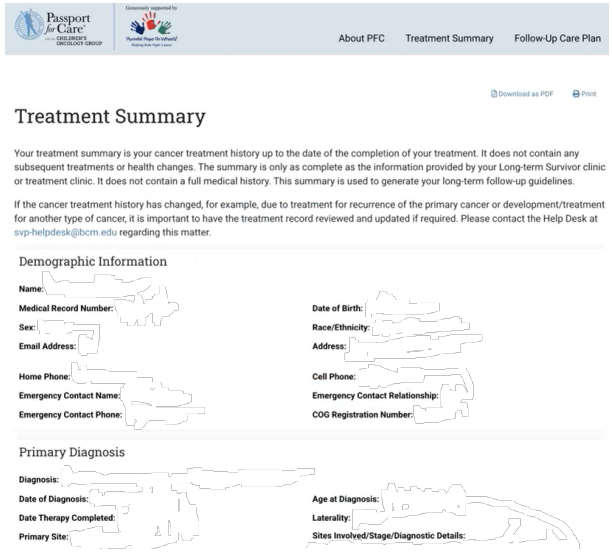
# Bringing the PFC to Ontario – Overview

- PFC is a secure clinical decision-support platform developed by Baylor College of Medicine and Texas Children's Cancer Center, USA.
- Currently used in 165+ national and international survivorship programs, including clinics in Manitoba and Nova Scotia.
- Provides survivors and clinicians with lifelong access to treatment summaries, late-effect risks, and evidence-based follow-up guidance.
- Integrated with the Children's Oncology Group Long-Term Follow-up Guidelines (www.survivorshipguidelines.org)
- Learn more: cancersurvivor.passportforcare.org/

## PFC: Clinician Web Portal




## PFC: Survivor Web Portal




## PFC: Survivor Mobile Health App

# Bringing PFC to Ontario: Approach

- Partnership with ACCESS and Baylor College of Medicine to implement PFC in Ontario.
- Data strategy that enables CYP-C data and provincial cancer registry information integration into PFC, offering an easy-to-use model that could support national adoption.
- Project Manager hired in July 2025
- Privacy, legal, and technical readiness activities underway.
- Legal compliance documents shared with hospitals
- Pilot rollout anticipated in the new fiscal year, followed by phased provincial expansion.

**All-in-One Digital Survivorship Care**

# Bringing the PFC to Ontario: Results

- Secure, electronic access to personalized treatment summaries and survivorship care plans for Ontario childhood cancer survivors.
- Automated generation of risk-based follow-up recommendations aligned with COG Guidelines.
- Reduced manual effort for clinicians through streamlined data integration.
- Designed to support future pan-Canadian implementation through interoperable, automated data integration.

## Supporting Survivors Across Ontario and Beyond


*Source: Pediatric Oncology Group of Ontario (POGO)*

# Bringing the PFC to Ontario: Impact

- Empowers survivors to proactively manage long-term health risks.
- Supports earlier identification of late effects across the survivorship continuum.
- Improves continuity and consistency of care across providers and regions.
- Reduces inequities by enabling access to evidence-based survivorship information.
- Lays the foundation for a nationally scalable survivorship care model.

## Why PFC Matters?

*"Passport for Care provides survivors—regardless of where they live or receive care—with a single, invaluable tool to understand their treatment, long-term risks, and recommended follow-up, empowering them to optimize their long-term health and quality of life."*

*- Medical Director, AfterCare Clinics*

# Thank you and Acknowledgements


- This research has been funded by the Canadian Institutes of Health Research (184352)
- POGO's Provincial Pediatric Oncology After Care Program and related initiatives are funded by the Ontario Ministry of Health.


access acces

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# Understanding Access to High-Cost Novel Cancer Therapies: A Survey of Pediatric Oncology Providers

Kriti Kumar

Pediatric Oncologist

Solid Tumor Section, Division of Haematology/Oncology

The Hospital for Sick Children

Advancing Childhood Cancer Experience, Science & Survivorship
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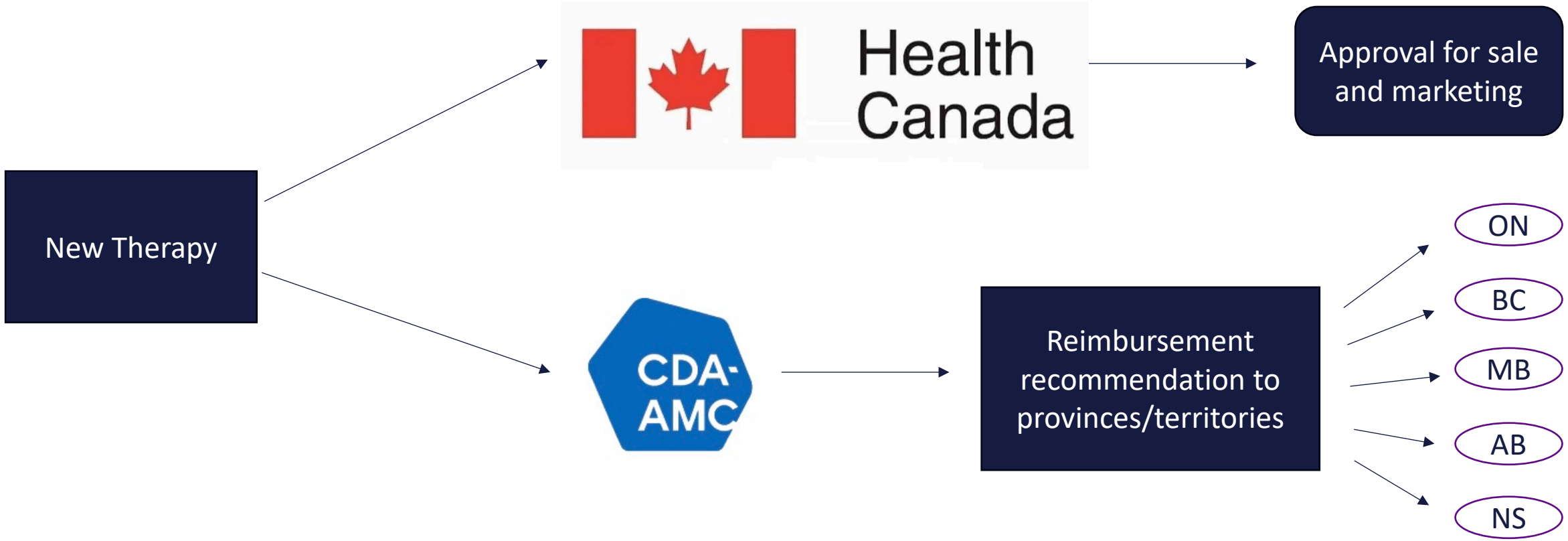
# Conflict of Interest

- None to disclose

# Background

- Innovative therapies continue to expand their use in pediatric oncology
- Many are high-cost and not currently publicly funded



# Global Disparities in Access to High-Cost Therapies

- Underserved populations not offered blinatumomab and cellular therapy due to prohibitive consumer costs and logistical complications
- Exposure to poverty and lower socioeconomic status have also been shown to impact selection of certain therapies and ultimately relapse rates
- Children from households with higher incomes and with private insurance being more likely to receive proton therapy in a retrospective study

Shen 2017 *Cancer*
Ning 2019 *Int J Radiat Oncol*
Tarnasky 2021 *J Pediatr Hematol Oncol*
Sehdev 2024 *Curr Oncol*

# Canadian Context

- To understand disparities in access to these high-cost innovative therapies across Canada's geographic expanse, we devised an online national survey

- Therapies for the survey were selected on the basis of being evidence-informed, but not universally funded:
  - Blinatumomab for patients with low risk first relapse of B-cell acute lymphoblastic leukemia (B-ALL)
  - Larotrectinib for pediatric TRK-fusion positive tumours
  - Proton beam therapy for head and neck sarcomas in children
  - Tisagenlecleucel for patients with Down syndrome and first relapse of B-ALL

# Methods

- Vignette-based survey distributed to pediatric oncology clinicians across 16 pediatric oncology centres
- Potential participants were contacted directly by email and/or through email distribution lists of: the ACCESS network, C17 Council, and the Canadian Association of Radiation Oncologists (CARO)

**Blinatumomab** for low-risk relapse of acute lymphoblastic leukemia (ALL)

**Larotrectinib** for a TRK-fused soft tissue sarcoma

**Proton beam therapy** for an unresectable head and neck sarcoma

**Cellular therapy** for first relapse of ALL in a patient with Down Syndrome

# Methods

- Primary outcome was access to each therapy, defined as the patient's ability to receive the specified therapy
- Secondary outcomes included time to access, funding sources, and perceived barriers.

# Results

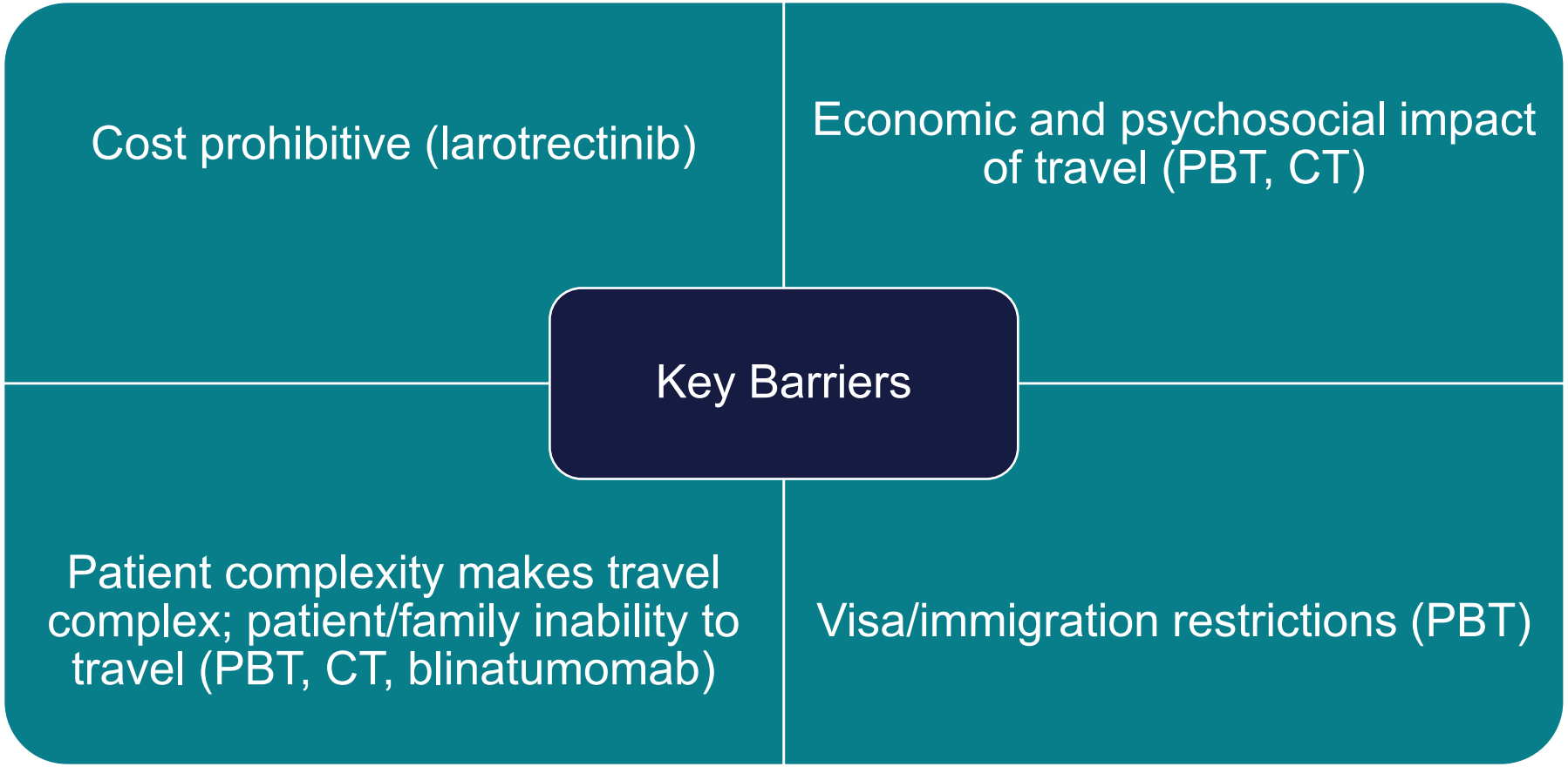
# Participant Demographics

| Variable | Number (%) |
|---|---|
| | n=68 |
| **Primary Role** | |
| Pediatric medical oncologist | 31 (45.6%) |
| Radiation oncologist | 7 (10.3%) |
| Oncology pharmacist | 10 (14.7%) |
| Hematopoietic stem cell transplant/cellular therapy physician | 3 (4.4%) |
| Nurse Practitioner/Advanced Practice Nurse | 7 (10.3%) |
| Other | 3 (4.4%) |
| | |
| **Region** | |
| Western Canada | 14 (20.6%) |
| Ontario | 29 (42.6%) |
| Quebec | 14 (20.6%) |
| Atlantic Provinces | 4 (5.9%) |
| | |

# Provider-Reported Access

| Province | Blinatumomab Mean (SD) % N=35 | Larotrectinib Mean (SD) % N=27 | Proton Therapy Mean (SD) % N=30 | Tisagenlecleucel Mean (SD) % N=30 |
|---|---|---|---|---|
| Total | 89.0 (23.3) | 79.0 (29.5) | 59.2 (30.0) | 94 (11.7) |
| Province | | | | |
| Alberta | 87.3 (24.8) | 99 (1.7) | 82 (27.7) | 96.7 (5.7) |
| British Columbia | 80.3 (26.7) | 76.7 (25.2) | 63.5 (19.1) | 70 (28.3) |
| Manitoba | 100 (NA) | 76 (NA) | 100 (NA) | 74 (NA) |
| Nova Scotia | 75 (35.4) | 58 (46.7) | 33.3 (15.3) | 100 (0) |
| Ontario | 96.4 (8.7) | 82.5 (23.6) | 42.4 (22.2) | 98.5 (3.4) |
| Quebec | 70 (44.7) | 64.7 (52.8) | 68.5 (41) | 95 (10) |
| Saskatchewan | 100 (0) | 100 (NA) | 72.3 (25.4) | 91.5 (12) |

# Barriers


Cost prohibitive (larotrectinib)
Economic and psychosocial impact of travel (PBT, CT)
Key Barriers
Patient complexity makes travel complex; patient/family inability to travel (PBT, CT, blinatumomab)
Visa/immigration restrictions (PBT)

# Conclusion

- Uneven access to key high-cost evidence-informed pediatric cancer therapies in Canada
- Notable barriers, including patient and family inability to travel, and the economic and psychosocial impact of travel
- Funding sources alleviated costs of direct therapies and supportive care
  - Costs of travel, accommodations, and food not consistently supported
  - Widens economic disparities in populations but also potentially impacts selection of therapies for patients, impacting overall oncologic outcome and exposure to long-term toxicity
- These variations in access challenge the notion of truly “universal” healthcare for children with cancer in Canada

# Knowledge Translation

Meeting Abstract: 2025 ASCO Annual Meeting I

FREE ACCESS | Care Delivery/Models of Care | May 28, 2025

## Understanding access to novel high-cost therapies across Canada: A survey of pediatric oncology providers.

**Authors**: Kriti Kumar, Avram Denburg, Celine Cressman, Derek S. Tsang, Marcel Romanick, and Paul James Gibson | AUTHORS INFO & AFFILIATIONS

*J Clin Oncol* **43**, e13500(2025) • Volume 43, Number 16_suppl • DOI: 10.1200/JCO.2025.43.16_suppl.e13500

- Manuscript submitted for publication, pending decision

# Making Family Distress Visible in Pediatric Oncology: Implementing Psychosocial Screening into Routine Practice

Lindsay Jibb, Fiona Schulte, and Leandra Desjardins

(on behalf of Thematic Matrix 7: Psychosocial and Survivorship Care: Paul Nathan, Sapna Oberoi, Vicky Forster, Megan Easton, Renu Jeyapala)



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# The hidden burden of caregiver distress

- Family distress in pediatric oncology is:
  - Common
  - Clinically significant
  - Associated with negative child and family outcomes
- Identification → Support

RESEARCH ARTICLE OPEN ACCESS

**Delivering Care Consistent With the Psychosocial Standards—Provider Report: Implementing the Standards Together—Engaging Parents and Providers in Psychosocial Care (iSTEPPP) Study**

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Kamyar Arasteh[1] | Emily Pariseau[1] | Eric Sandler

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**Correspondence:** Anne E. Kazak (anne.kazak@nemours.org)

**Received:** 19 May 2025 | **Revised:** 15 July 2025 | **Accepted:** 19 J

**Funding:** This research was supported by a grant from the Andre
Healthcare Delivery Science and the Intramural Research Progran

**Keywords:** pediatric oncology | psychosocial | standards of care


**ABSTRACT**

**Background:** Evidence-based Standards for Psycho
2015. Determining how often care delivery practices
reach of the Standards.

**Procedure:** Medical ($n = 73$) and psychosocial ($n = 95$
in the United States completed an online survey. E
approaches for each Standard. Program size was con

**Results:** Using a 5-point scale, services consistent w
4.61 (5-point scale). Standards related to screening a
to intervention and support ($M = 4.25$), supportive c
$< 0.001$). Ratings of quality of care are near the mid
related to family and community support was less fre
quality or frequency.

**Conclusions:** Providers report that psychosocial car
positively. Variability in types of care, frequency, an
highlight the importance of improving the implemer


# Systematic Psychosocial Screening in Canada

- Across participants (n=51), 87% ‘usually/always’ assess psychosocial needs
  - Most commonly by informal discussion or interview

Barriers include:

- Choosing appropriate tools
- Workflow integration challenges
- Uncertainty around implementation

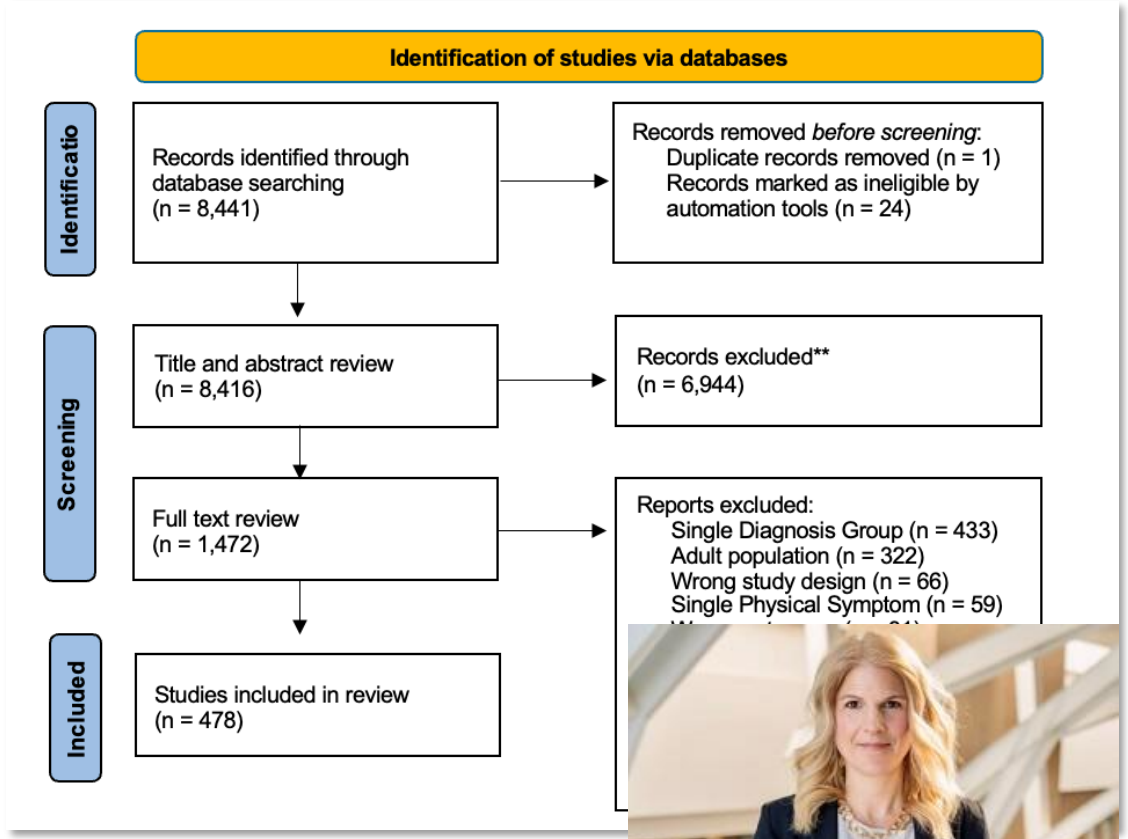

# Thematic Matrix 7 Aim

- National priority-setting work with children, families, and clinicians identified family psychosocial health as a key research priority.
- Community engagement through two ACCESS Town Hall meetings further highlighted:
  - impact of cancer-related distress on families
  - the need for systematic assessment of family psychosocial needs

Implement context-sensitive family psychosocial screening into routine practice in select settings to improve clinical capacity to detect family distress.

# Identifying the Right Tools

- Scoping review to identify psychosocial screening tools suitable for parent- & child-report in pediatric oncology
- Assessment of:
  - Strength of evidence in literature
  - Validity and reliability
  - Clinical utility, including cost, multilingual availability, and actionable cut-points for follow-up




# Preparing for Implementation

**Study Questions:**
- How can Epic-integrated psychosocial screening be best implemented into practice?

**Study Design:**
- Pre-implementation pilot using qualitative description

**Participants:**
- 10 parents
- 10 multidisciplinary clinicians

**Recruitment:**
- Leukemia/Lymphoma Section, SickKids

**Data collection:**
- Semi-structured interviews

**Data analysis:**
- Thematic analysis with reference to KTA and RE-AIM

## Parents

**Simplicity & integration** with existing

**Emotic**
**timing** of scre

Scree
**meaningful**

**Human relationships** remain central to implementation

## Clinicians

**Clear response pathways** engaging
oviders needed

pe
s **languages**
**xts**

xisting

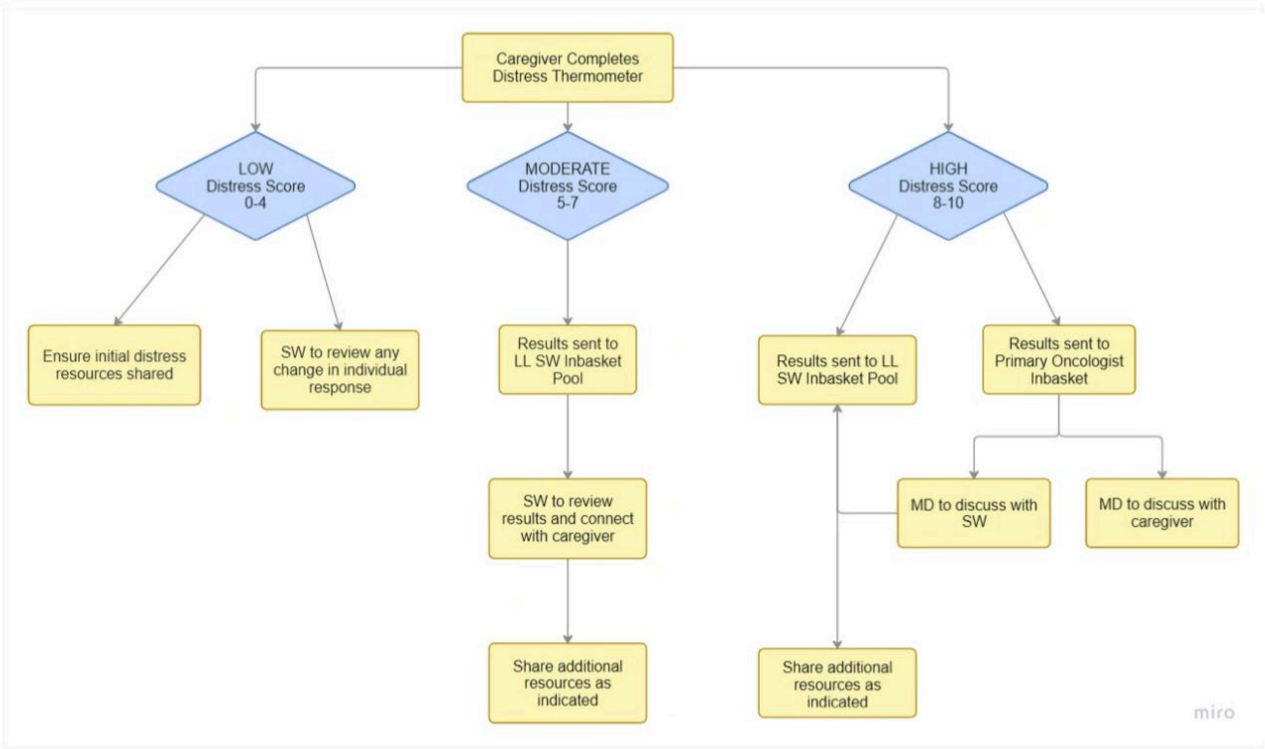
Psychosocial needs are recognized, yet **not systematically**

"I think that the people that will benefit the most are probably people that wouldn't speak to us as often as they truly need to… a lot of people don't want to ask for help for themselves, they're here for their child."

# What Will Make Screening Work?

Successful caregiver e-screening will require:

- Easy completion for parents
- Seamless workflow integration
- Real-time identification of distress
- Clear follow-up and triage pathways



# From Insight To Action

- Pilot implementation at four sites
- Evaluation guided by RE-AIM
  - Reach, Effectiveness, Adoption, Implementation, Maintenance


# Thank you and Acknowledgements

This research has been funded by the Canadian Institutes of Health Research (184352)

ACCESS

Thematic Matrix 7

Dr. Renee Potashner

Rachel Hamilton

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David Brownstone

Sonia Lucchetta

Nicole Withers

Parent and clinician participants

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# A National Pediatric Preclinical Modelling Program to Advance Precision Oncology for Childhood Cancers in Canada

Co-leads: Drs. Jason Berman, Donna Senger and James Lim

Advancing Childhood Cancer Experience, Science & Survivorship
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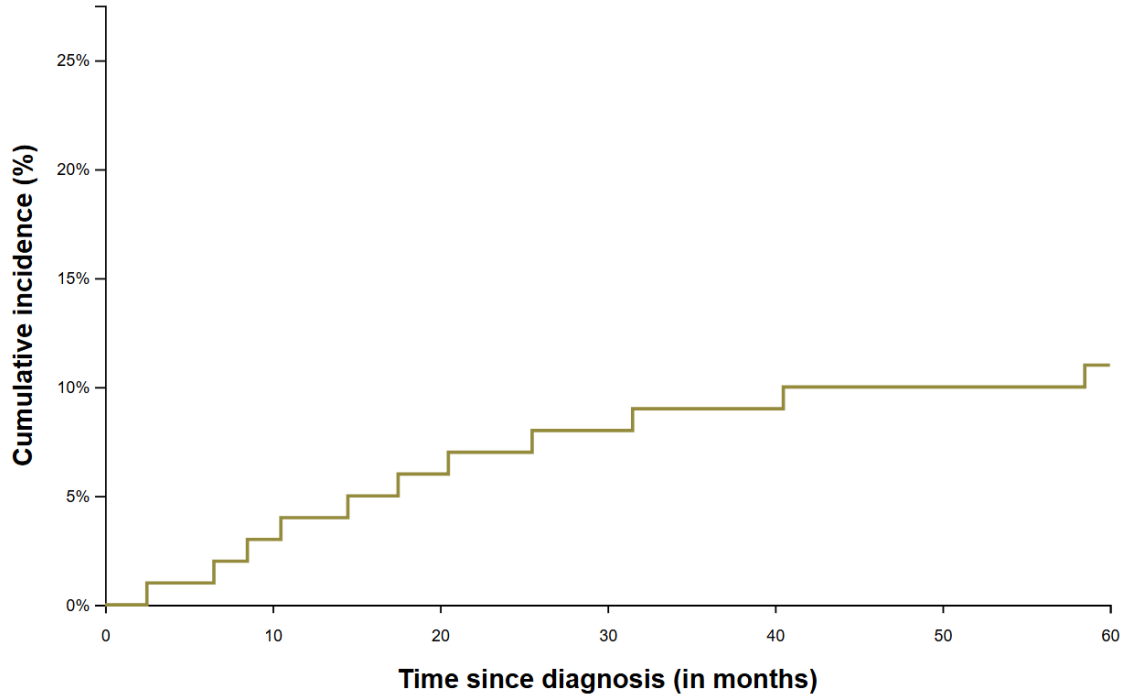
# Pediatric Cancer – Survival Rates

- For children with refractory, relapsed, or metastatic disease, survival rates are <15%
- More than 80% of survivors will experience long-term health impacts from the disease itself or treatment toxicity – a heavy price for high cure rates.

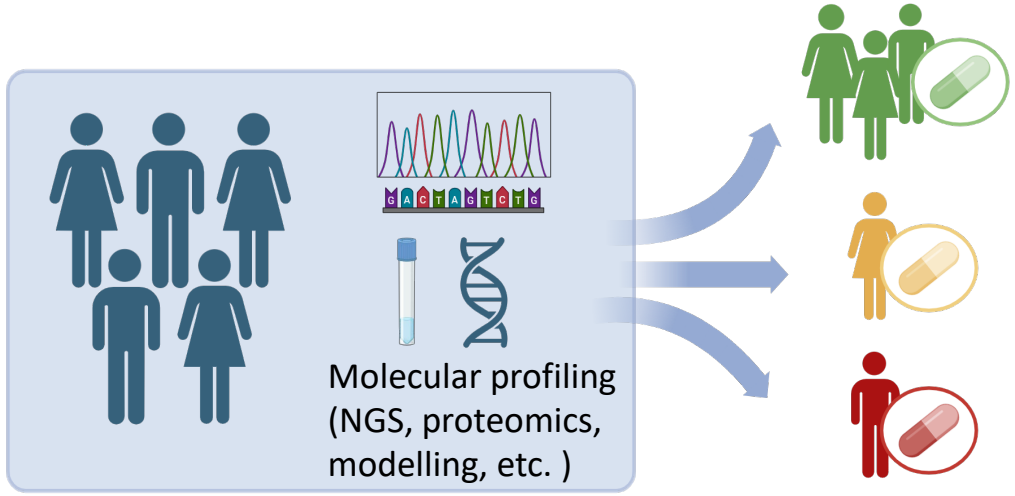
**Cumulative incidence of relapse**




*(The Cancer in Young People in Canada (CYP-C) program)*

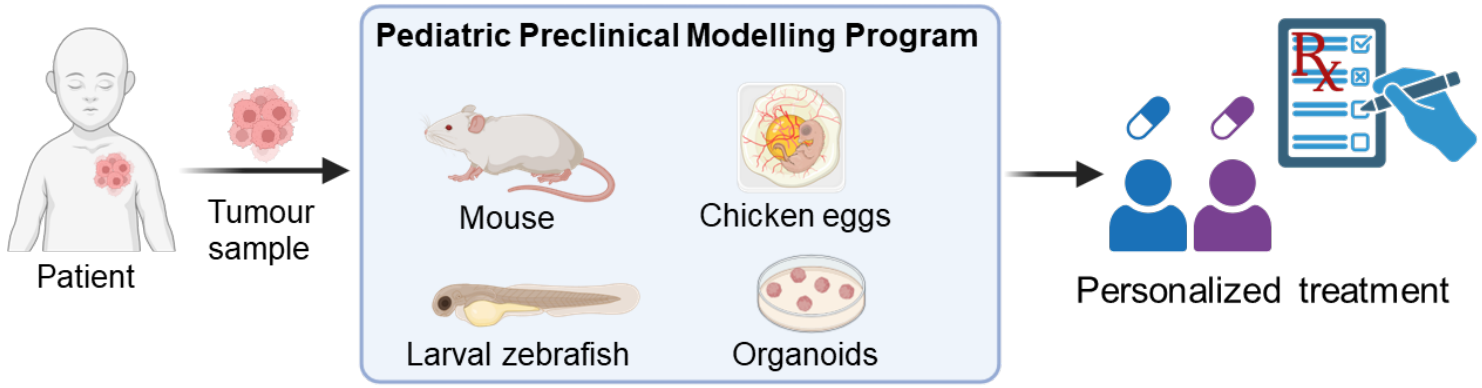
# Pediatric Cancer – Rationale for Personalized Treatment

- Pediatric cancers are rare diseases and different from adult cancers. Children are not simply small adults.
- Biology and genetics are complex and often poorly misunderstood
- Understanding each tumour's unique biology enables identification of targeted therapies
- Personalized approached are essential to improve outcomes and reduce toxicity



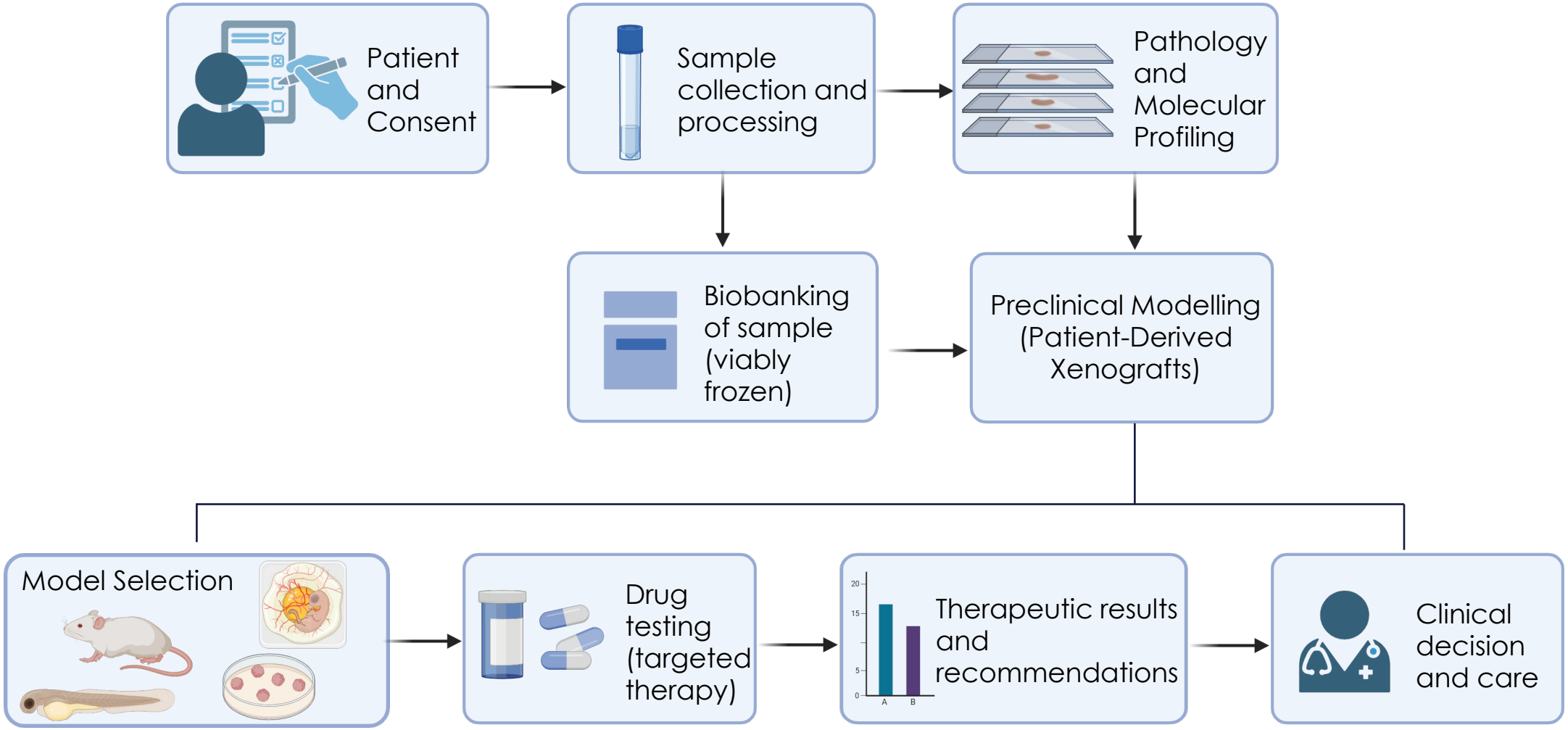
# Pediatric Preclinical Modelling Program (PPMP)

- Develop and use preclinical models to identify specific treatments that target the unique makeup of each patient's tumour before administering it.
- Motivation and Mission: Provide equitable access to this state-of-the-art approach for all high-risk childhood cancer patients in Canada and aid in the delivery of target-based therapeutic options in a clinically relevant timeframe.

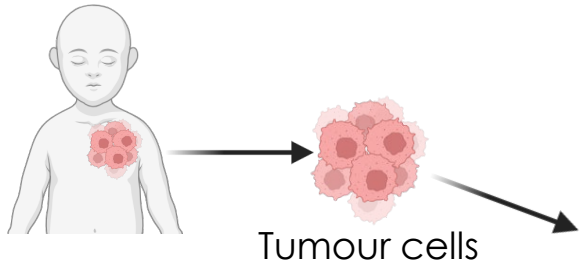


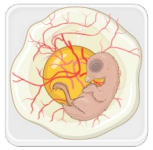
# PPMP –Leadership and Team

# PPMP - Pipeline



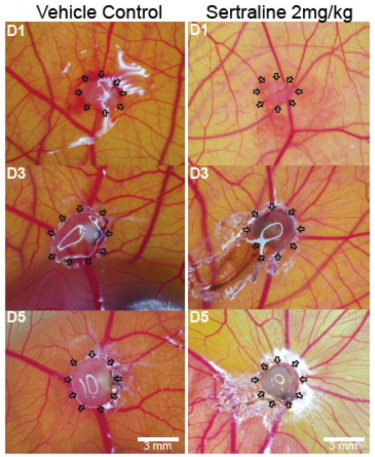
# PPMP – Current Preclinical Models

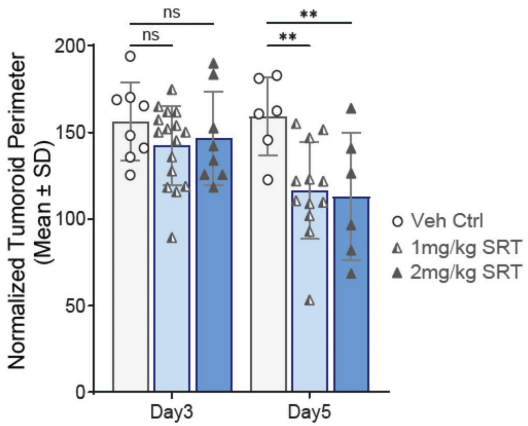


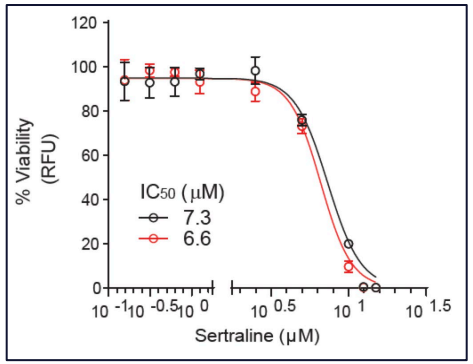

| | Models | | |
|---|---|---|---|
| | **Mouse** | **Chicken eggs (CAM)** | **Larval zebrafish** |
| |  |  |  |
| **Time** | Months | 2 -3 weeks | 7-10 days |
| **Cells per animal** | 0.5 - 5x$10^6$ cells | 1 - 5x$10^5$ cells | 100 - 200 cells |
| **Animal per group** | 2 - 5 | 5 - 10 | 35 - 40 |
| **Renewable resources (tumour expansion)** | ✓ | ✓ (limited) | ✗ |
| **Throughput** | | | |
| **Cost** | $$$ | $$ | $ |

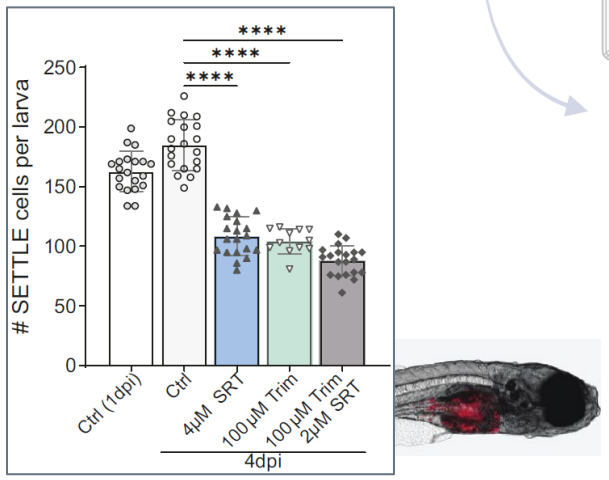
# From bedside to bench to beside: Patient with progressive SETTLE tumour

## Sertraline inhibits growth of SETTLE PDX

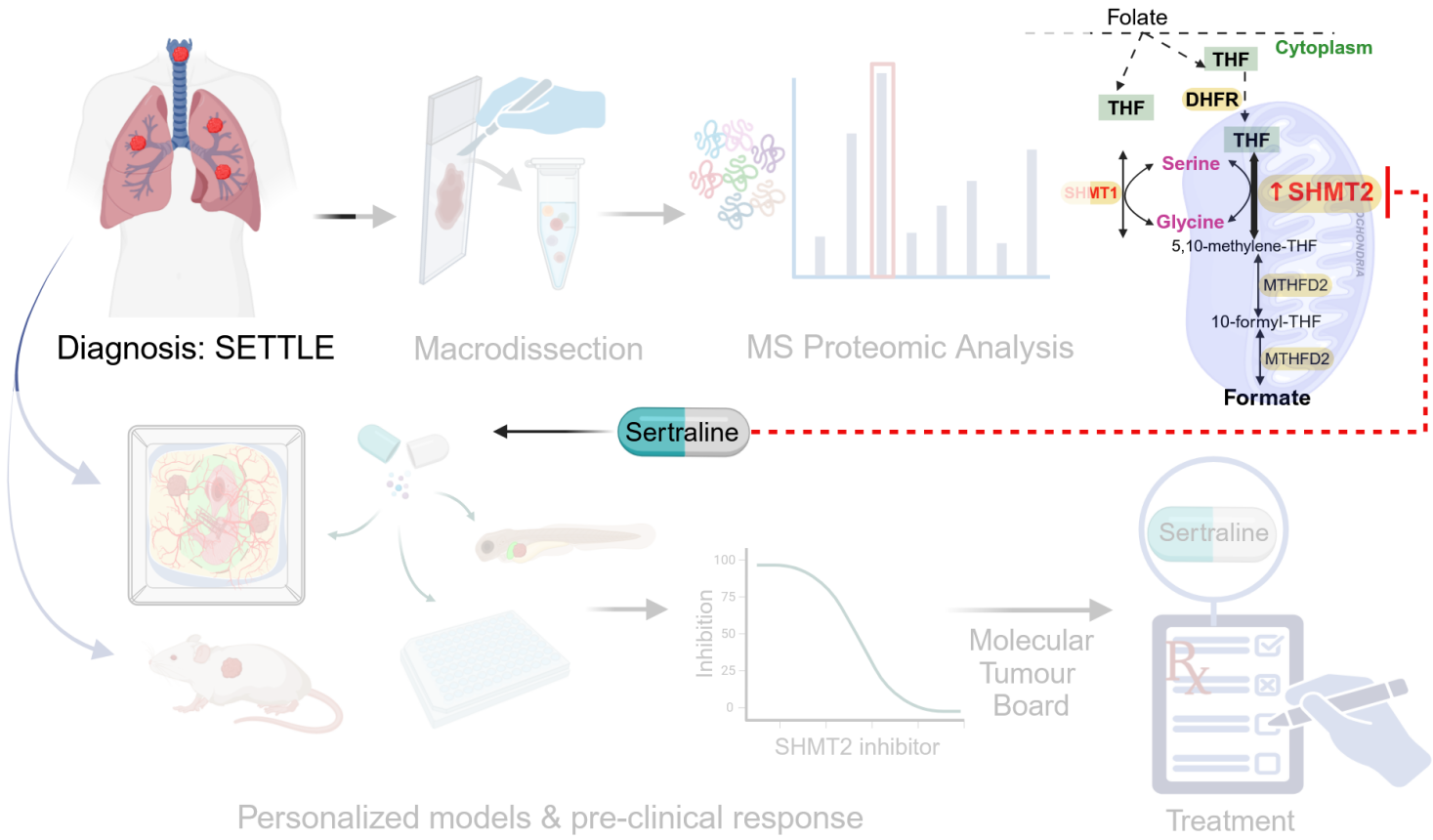





**EMBO Mol Med (2025) 17:625-644** PMID:**40204966**

# Case Study: PRO-00828 – Patient information

| | |
|---|---|
| **Age, Gender** | |
| **Primary Cancer** | Osteosarcoma of the Rib |
| **Date of Diagnosis** | |
| **Date of Metastatic disease** | Localized disease but hemothorax from chest tube placement |
| **Treatment** | Received standard first line chemotherapy (Cisplatin, Doxorubicin, Methotrexate)<br>Tumor resected at week 12 with unfavorable necrosis (45%) but margins negative<br>Completed therapy May 2023 |
| **Post therapy Surveillance** | Remained in a complete response until late May 2025 |
| **Relapse** | Presented with back pain radiating down his leg<br>Imaging showed an isolated relapse in L5 vertebrae with large mass causing cauda equina syndrome and abdominal extension<br>Biopsy confirms Osteosarcoma<br>Treated with High Dose Ifosfamide x 6 cycles<br>Unresectable so treated with high dose radiation |

# Case Study: PRO-00828 - Background

**Treatments of interest from PROFYLE report**

**High priority:**

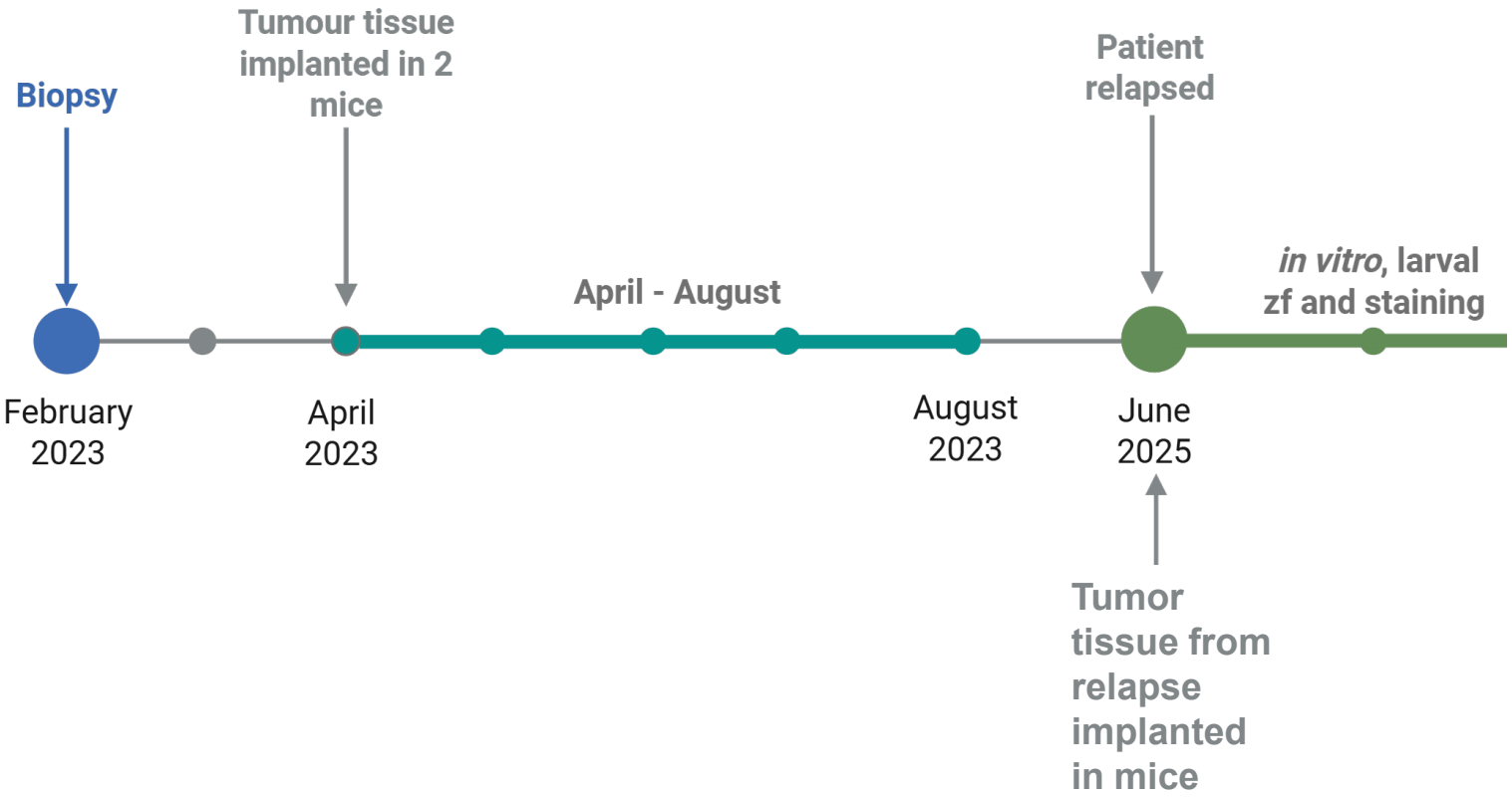
- **Regorafenib** | ↑ **VEGFA, VEGFR inhibitor**
- **5-Fluorouracil** | **DPYD variant**

**Medium priority:**

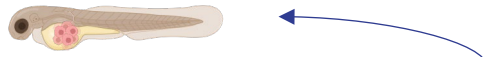
- **Gemcitabine** | ↑ **SLC29A1 (Gem transporter)**
- **Alisertib** | ↑ **AURKA/B inhibitor**

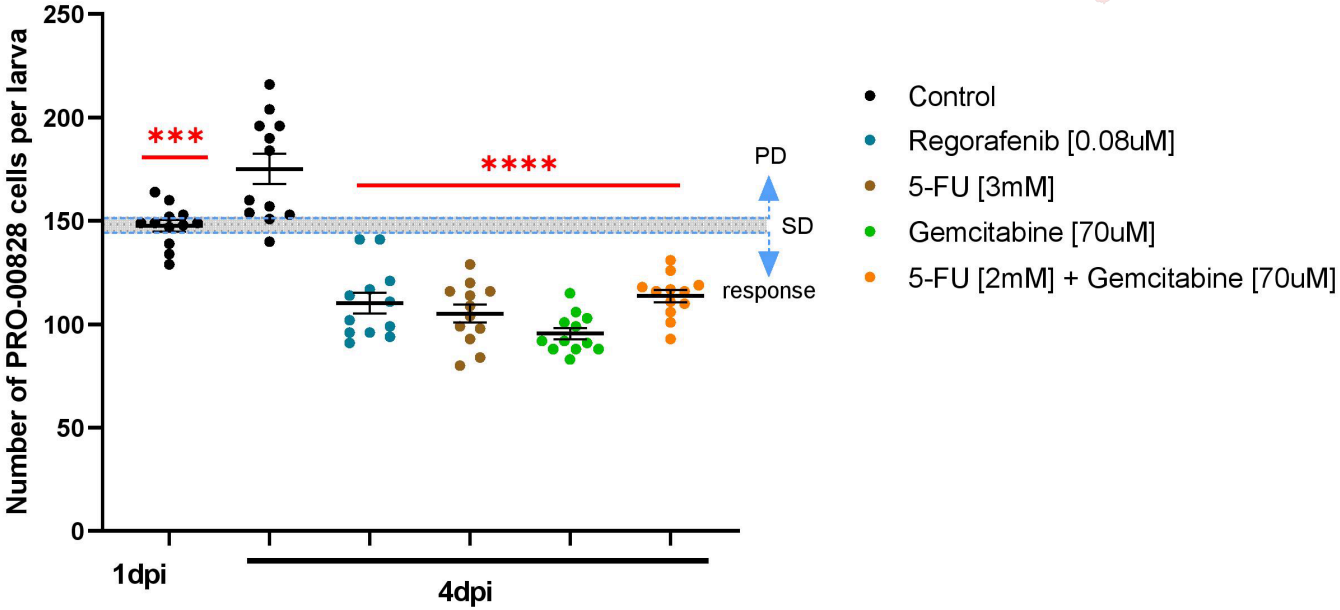
2023 biopsy tumour tissue sent to Dr. Donna Senger's lab for expansion in mouse

- Tumour cells/tissue expanded in mouse were cryopreserved for further drug testing



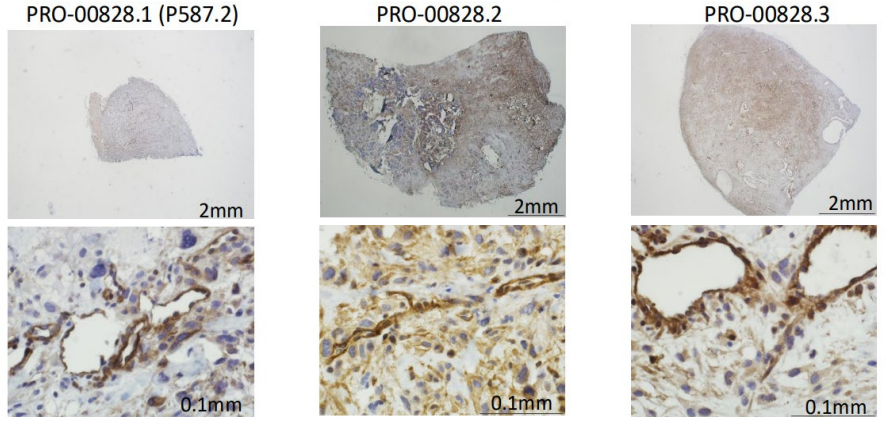
# Case Study: PRO-00828 – Modelling and staining



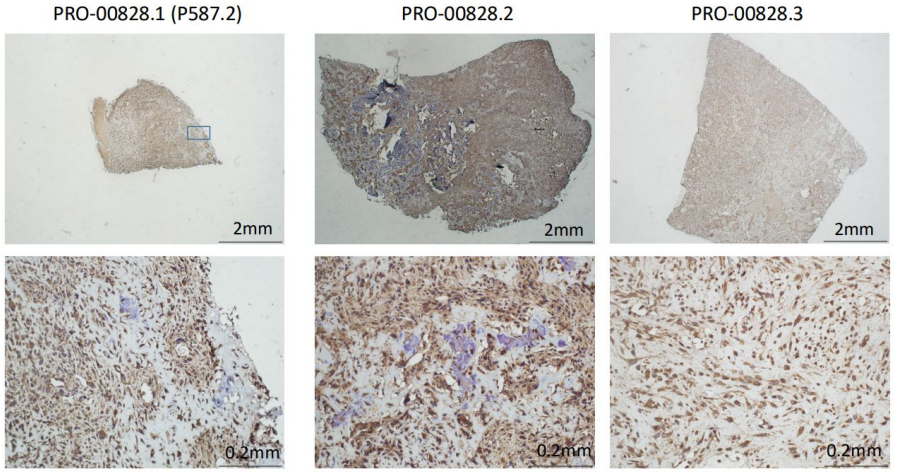



1dpi – initial number of cells (baseline)
4dpi – number of cancer cells after treatments
PD – progressive disease
SD – stable disease

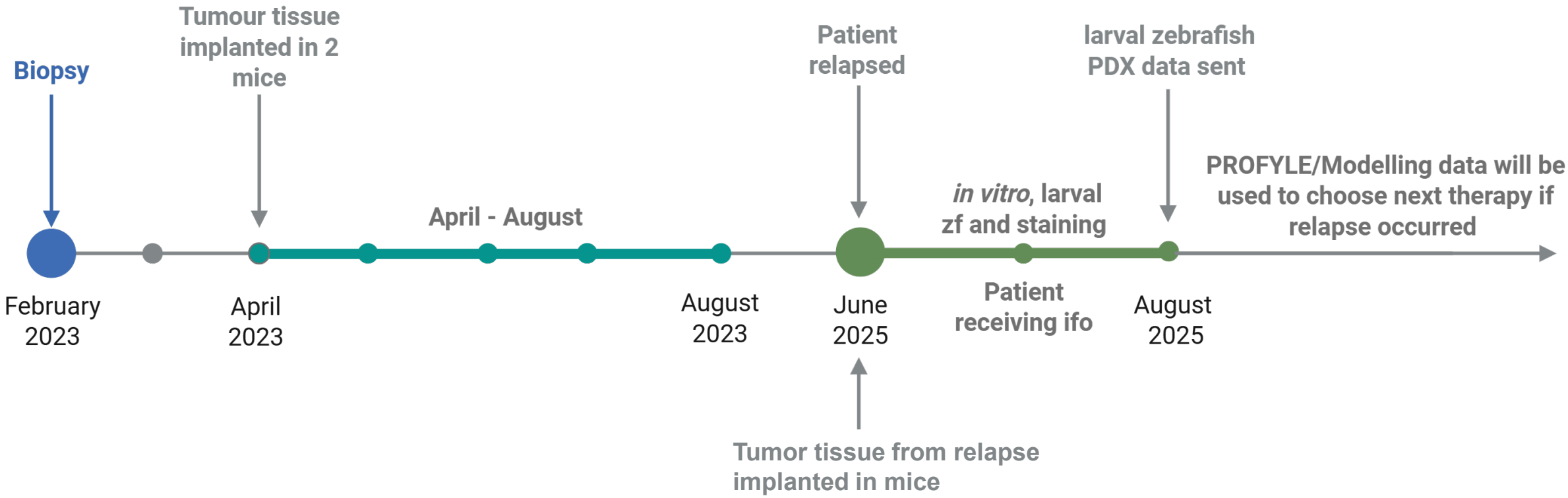
Immunohistochemistry –VEGFR (Cell Signaling Cat#: 2479)




Immunohistochemisty – VEGF (Santa Cruz Cat#: sc-7269)

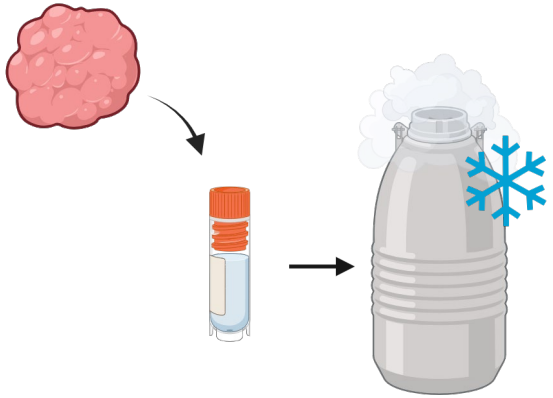


# Case Study: PRO-00828 – Timeline and outcomes



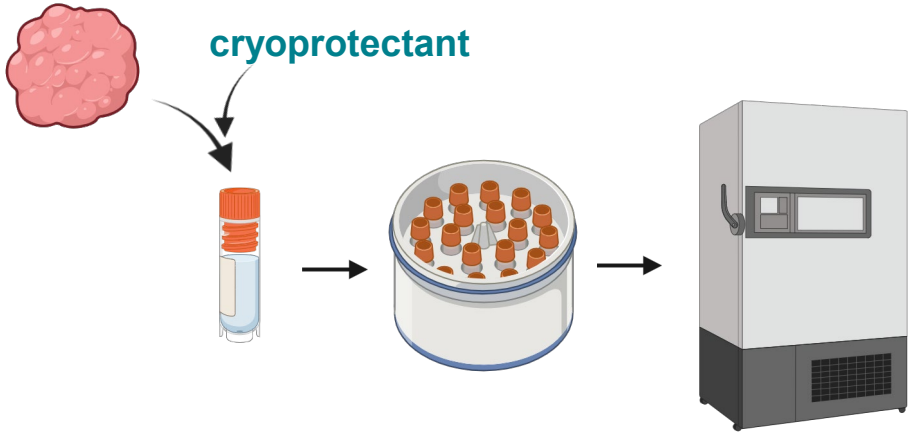
# Tissue Preservation Strategies for Functional Modelling

## Flash-frozen



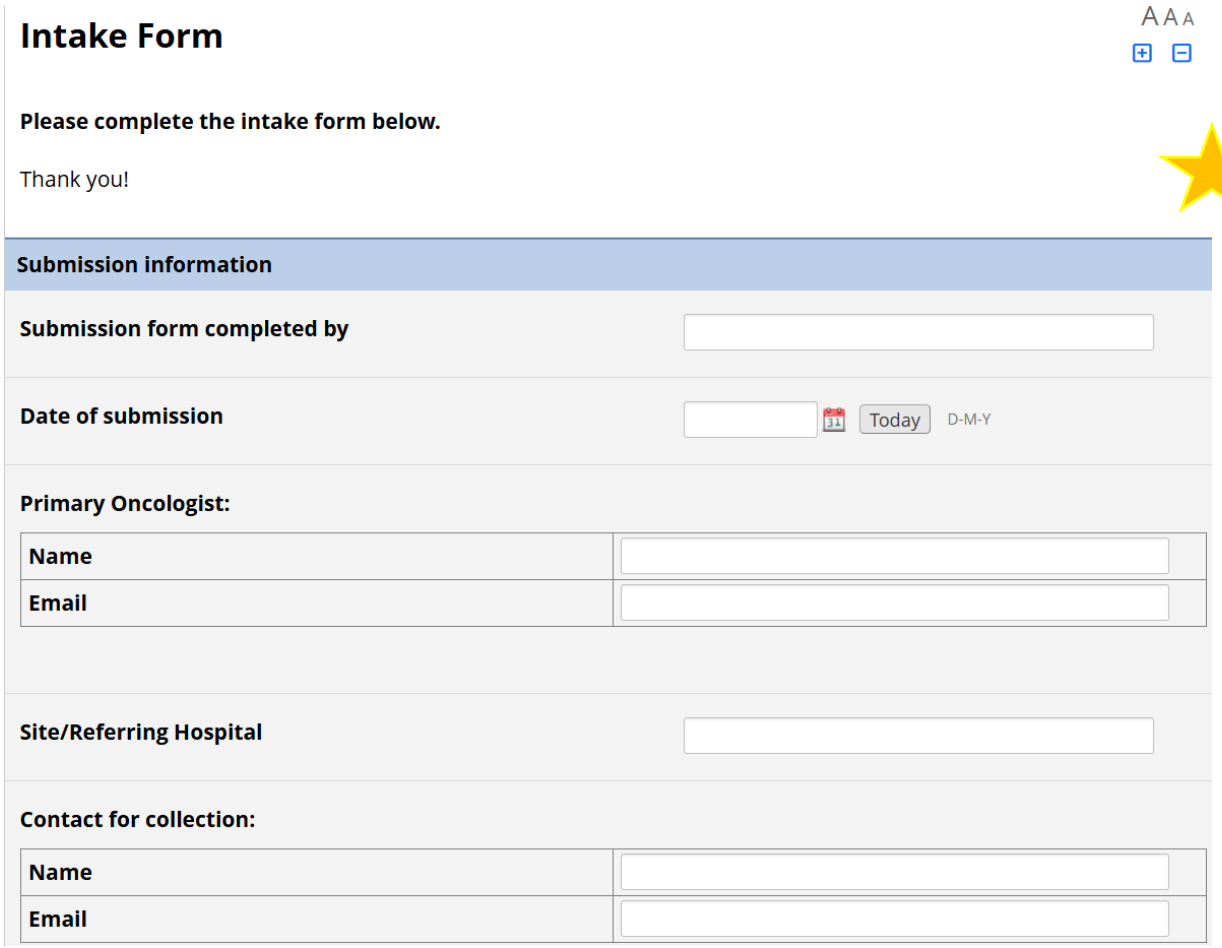
- Excellent for DNA, RNA & protein analysis
- Preserves molecular profile at time of collection
- BUT, cells are non-viable
- Cannot generate patient-derived preclinical models

## Viable Cryopreservation




- Maintains viable tumour cells
- Enables establishment of:
  - In vivo PDX models (e.g., mouse)
  - Organoids and 3D cultures
- Allows functional drug testing
- Protocol support (contact us!)

# Intake form – Modelling program



## Intake Form

AAA

**Please complete the intake form below.**

Thank you!

**Submission information**

**Submission form completed by**

**Date of submission** Today D-M-Y

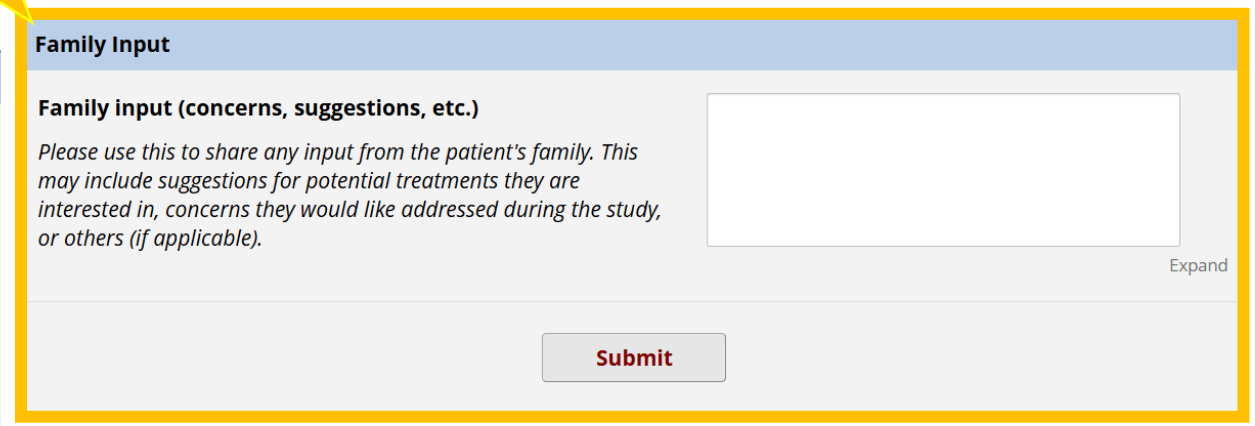
**Primary Oncologist:**

| | |
|---|---|
| **Name** | |
| **Email** | |

**Site/Referring Hospital**

**Contact for collection:**

| | |
|---|---|
| **Name** | |
| **Email** | |



**Family Input**

**Family input (concerns, suggestions, etc.)**

*Please use this to share any input from the patient's family. This may include suggestions for potential treatments they are interested in, concerns they would like addressed during the study, or others (if applicable).*

Expand

Submit

**Intake form - PPMP**

https://redcap.link/x8wv64zx

# Acknowledgment

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**Pediatric Preclinical Modelling Program**

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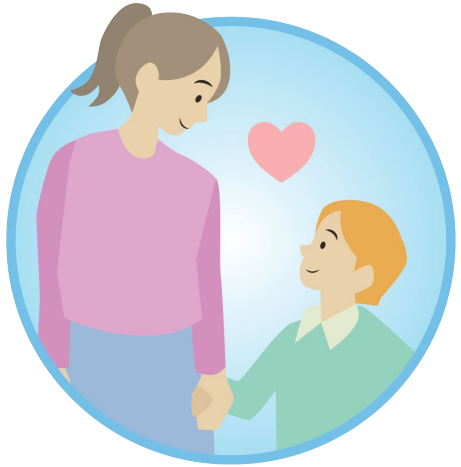
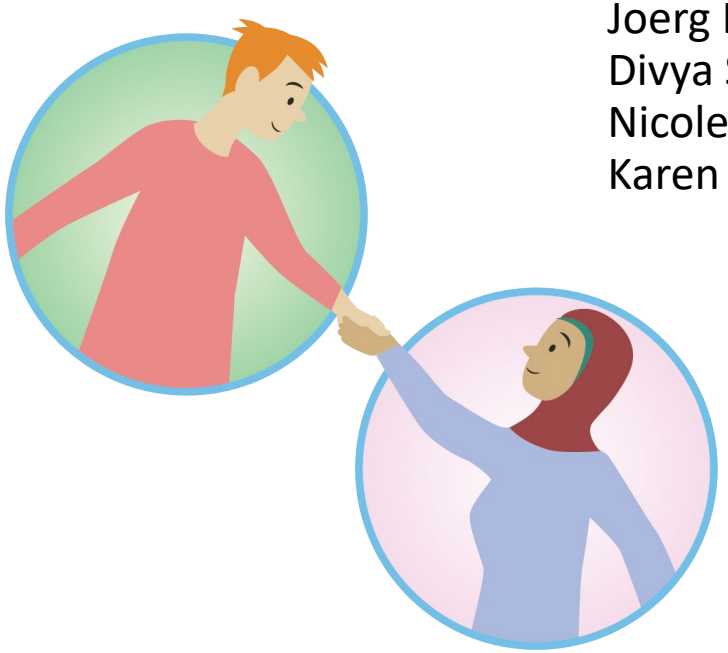
Vicky Ling Li

**Poster Presentation** entitled "A National Pediatric Preclinical Modelling Program to Advance Precision Oncology for Childhood Cancers in Canada" – **Presenter: Nadine Azzam**

# Development of a Canadian Pediatric CAR T-Cell Network for Manufacturing and Local-Regional Access and Delivery of Novel CAR T-Cell Therapies





Joerg Krueger, Henrique Bittencourt, Michel Duval, David Mitchell, Ashley Chopek, Divya Subburaj, Greg Guilcher, Victor Lewis, Ravi Shah, Geoff Cuvelier, Sunil Desai, Nicole Prokopishyn, Karin Hermans, Michael Chu, Ashley Townson, Erilda Kapllani, Karen Sherwood, Donna Wall, Kirk Schultz, Amanda Li

On behalf of the *Cell Therapy Transplant Canada Pediatric Committee*

ACCESS Meeting
March 10th, 2026
Toronto, Ontario

# Access Challenges to Novel Cellular Therapies for Canadian Children

- CAR T cells therapies are improving outcomes for patients with cancer (and other non-malignant diseases)

- Licensed CAR T-cell products
  - Manufactured by pharmaceutical companies
  - Currently only one product licensed for children (Anti-CD19 CAR for r/r B-ALL)

- Pediatric cancers are comparatively rare
  - Industry R&D increasingly focusing on adult malignancies, increasing reticence to commit to pediatric trials
  - "…pediatric CAR development at crucial crossroads…limited availability of CAR T-cell products to academic study group…products for pediatric cancer population by pharmaceutical companies is unlikely to return investment…call for a concerted academic action.."[1]

*=>To be able to offer novel Cellular Therapies with equity of access for children in Canada, we advocate for:*

1. Industry-independent local CAR manufacturing capabilities

2. Improved care delivery with decreased barriers to CAR T cell therapy access, local-regional treatment focus and decreased burden to patient families

1. Rossig et al., JCO 2023

# Canadian Pediatric Blood and Marrow and Cellular Therapy Group

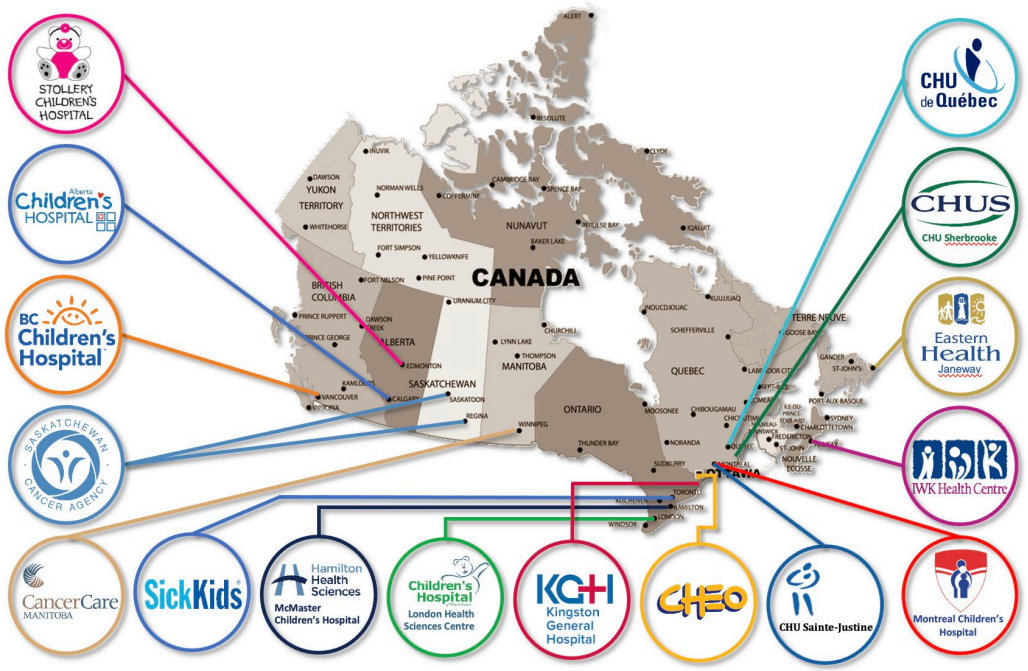
- Cell Therapy Centers

CTTC CELL THERAPY TRANSPLANT CANADA
TRANSPLANTATION ET THÉRAPIE CELLULAIRE CANADA

- BC Children's Hospital, Vancouver, BC
- Alberta Children's Hospital, Calgary, AB
- Cancer Care Manitoba, Children's Hospital, Winnipeg, MB
- The Hospital for Sick Children, Toronto, ON
- CHU Sainte-Justine, Montreal, QC
- Montreal Children's Hospital, QC


- Referring centers

Children's Cancer & Blood Disorders



# Pediatric CAR T-Cell Network for Manufacturing and Local-Regional Access and Delivery of Novel CAR T-Cell Therapies *CAR4ALL / CAR POUR TOUS*

- **Aim 1**
  - Pediatric Point of Care Manufacturing Capabilities
- **Aim 2**
  - Countrywide pediatric network to promote (novel) cellular therapies
    - Advocacy, Care Pathways, Standard and Educational Resources
- **Aim 3**
  - Access and development of Canadian based testing for Next Generation Sequencing Minimal Residual Disease Post CAR T cell treatment

*Guiding Principles*

- National lens
- Collaboration with relevant stakeholders
- Not-for-profit
- Involvement of Patients and Families with lived experience (PWLE)

# Aim 1. Develop Point-of-Care CAR T-cell Manufacturing

**Goal:** Manufacture CD19 CAR T cell product with Miltenyi CD19 vector, using the Miltenyi CliniMACS Prodigy closed cell processing system

- Perform 3 validation manufacturing runs with central quality control testing
- Manufacturing sites: BC Children's Hospital, Alberta Children's Hospital, Cancer Care Manitoba, Hospital for Sick Children, Ste-Justine

## Progress

**Selection of first CAR vector**

- Met with stakeholders, Canadian consortia, industry partners, and international key opinion leaders

**Cell facility/manufacturing laboratory group formed meeting regularly**

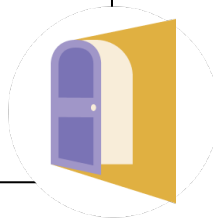
- Lead: Dr. N. Prokopishyn
- Monthly Meetings
- Cell Facility benchmarking
- National standardized SOPs

**QC testing**

- Requires validated assays for microbacterial testing, cell viability, cytokine assays, replication competent lentiviral enumeration, etc.

**Logistical and Regulatory Benchmarks/Progress**

- Import Licenses
- Biosafety licenses



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- BC Children's Hospital, Alberta Children's Hospital, Cancer Care Manitoba, Hospital for Sick Children, Ste-Justine

**Progress**

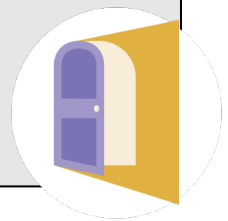
**In-person peer-to-peer training for first manufacturing run**

- Validation against Health Canada approved manufacturing process in Edmonton (Adult program, Dr. M. Chu)
- Co-funded by CTTC [Thank you!]

=> Calgary April 2026 !!!

**Impact/Outcomes**

- Develop local infrastructure and technical expertise to perform CAR T-cell manufacturing on a bench-top manufacturing platform within clinically-based laboratories.
- Foundational preparation for a Canadian CD19-CAR T-cell pediatric trial
  - ALL first relapse number 1 unmet need. No Canadian center for upcoming COG trial
- Proof-of principle for platform for other targets/diseases



# Aim 2. Improve Equitable Access and National Standards of CAR T-cell Therapy Delivery

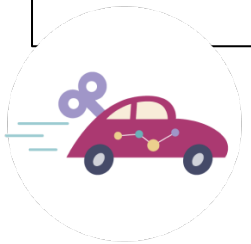
**Goal: Develop the Canadian Pediatric CAR T-cell Network (CPCN):**

- Bring together local-regional pediatric oncologists, CT/BMT 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.

**Progress**

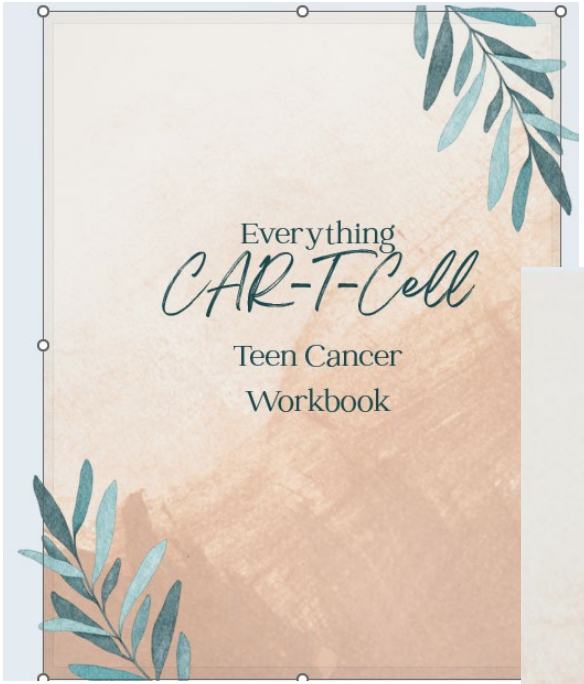
- **Consensus Statement Guidelines for use of CAR T cell therapy in children in Canada** beyond existing commercial label.
  - Draft version under review

- **National Needs Assessment of Pediatric CAR T-cell Treating and Referring Sites**
  - *Led by Drs. Divya Subburaj, Victor Lewis*
  - Online Survey of clinical teams (MDs, nurses, patient navigators, and social workers) conducted.
    - Data analysis pending



# Aim 2. Improve Equitable Access and National Standards of CAR T-cell Therapy Delivery

- **Patients with Lived Experience (PWLE) Advocacy**
  - Partnered with a young adult PWLE from BC to produce an AYA specific CAR T cell patient handbook
    - Handbook finalized
    - Revisions in progress
  - Have met in focus group meeting with 2 additional PWLE families (1 Ontario, 1 BC), who will continue to provide feedback on this project





- *Why did you write this book?*
- *Who did you write it for?*
- *What impact do you hope this book has for other people?*

# CAR-T-Cell Therapy

CAR T CELL THERAPY IS A TYPE IMMUNOTHERAPY THAT USES THE PATIENT'S OWN CELLS, TO ATTACK CANCER CELLS.

STEP 1: T CELL COLLECTION
A TUBE IS INSERTED INTO YOUR NECK OR ARM TO REMOVE BLOOD CELLS.
AN APHAERESIS MACHINE SEPARATES YOUR T CELLS.
THE REST OF YOUR BLOOD AND FLUIDS GO BACK INTO YOUR BODY.

STEP 2: YOUR T CELLS GET MODIFIED
IN A LABORATORY SCIENTISTS ENGINEER YOUR T CELLS BY ADDING A CHIMERIC ANTIGEN RECEPTOR (CAR), THIS RECEPTOR CAN FIND CANCER CELLS BECAUSE THEY HAVE A PROTEIN BIOMARKER ON THEIR SURFACE (LIKE A FLAG)

STEP 3: GETTING YOUR T CELLS BACK
INFUSION!: YOUR T CELLS (NOW CAR T CELLS) ARE ADDED BACK INTO YOUR BLOOD.
THERE MAY BE WEIRD TASTE OR SMELL WHEN THIS HAPPENS!

STEP 4: IMMUNE ACTIVATION
YOUR NEW CAR T CELLS WILL HUNT AND KILL CANCER CELLS.
THIS IMMUNE RESPONSE IS CALLED CYTOKINE RELEASE SYNDROME (CRS).
SOME PATIENTS FEEL LIKE THEY HAVE A BAD FLU DURING THIS TIME, AND SOME PATIENCES GET NO SYMPTOMS AT ALL..

# Possible Side Effects (CAR-T-Cell)

CYTOKINE RELEASE SYNDROME (CRS): OCCURS WHEN THE T CELLS RELEASE CYTOKINES, IN THE BODY. THIS RELEASE CAN CAUSE YOUR IMMUNE SYSTEM TO REACT.
This reaction is different for everyone, but in most cases it's like having a bad cold or flu.

It's important to be able to identify and describe what is happening with your body. here are some useful adjectives (feel free to add more!)

PAIN CAN BE:
sharp
dull
stabbing
throbbing

OUR BODY CAN FEEL:
unsettled
restless
tired
exhausted
detached

SWEATY
CHILLED
SHAKY

NAUSEATED
CLAMMY
TINGLING

OUR BRAIN CAN FEEL:
fuzzy
confused
tired
cloudy
DRAINED
SLUGGISH

# CORE Values

WHEN LIFE GETS HARD, KNOWING WHAT REALLY MATTERS TO YOU — YOUR PERSONAL VALUES — CAN GUIDE YOUR DECISIONS, KEEP YOU GROUNDED, REMINDING YOU OF WHO YOU ARE

VALUES ARE THE BELIEFS AND PRINCIPLES THAT GUIDE YOUR ACTIONS AND DECISIONS. THEY'RE LIKE AN INNER COMPASS

# Aim 3. Improve post CAR T-cell Therapy Monitoring

**Goal: Bring Next-Generation Sequencing Minimal Residual Disease (NGS MRD) testing to Canada:**

- Cross-validate 20 post-CAR T-cell Therapy samples on commercial and non-commercial NGS MRD platform

**<u>Progress</u>**

- NGS MRD Working Group formed, regular meetings every 6 weeks
  - Vancouver (E. McGinnis, K. Sherwood, A. Li)
  - Sick Kids Hospital (J. Bartram, J. Krueger)
  - Ste-Justine (T. Tran)
  - Project Management by Dr. Karen Sherwood (Vancouver General Hospital). Two potential sites to develop NGS MRD capabilities – Vancouver General Hospital, SickKids Hospital
- Ongoing
  - NGS MRD Technique
    - Children's Oncology Group based trials using ClonoSeq by Adaptive Biotec (Seattle, USA), which is a proprietary methodology, difficult to validate as an external site.
    - **<u>Changing political landscape</u>**

    ⇒ Develop and validate EuroMRD approach for clinical use [Dr. J. Bartram]

    ⇒ Lab assessment and implementation May 2026 [Toronto]
- Protocol Development (R. Shah)

# Acknowledgements & Collaborators


## CTTC CAR T-Cell Steering Committee

Henrique Bittencourt

David Mitchell

Ashley Chopek

Divya Subburaj

Victor Lewis

Ravi Shah

*And many others!*

## Patients And Families with Lived Experience, including:

JL and family

JD and family

KL and family

LE and family

## C17 CTTC Pediatric BMT Working Group and Leadership

Michel Duval

Kirk Schultz

Donna Wall

Greg Guilcher

Tony Truong

Geoff Cuvelier

Sunil Desai

Deanna Hockley

Stephanie Maier

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Kyle Potts

Karin Hermans

Audi Setiadi

CTTC Cellular Therapy Lab Group

## NGS MRD Task Force

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Eric McGinnis

Jack Bartram

Mohammed Abdulhaleem

Thai Tran

## CLIC Network and BioCanRx Consortia

Natasha Kekre

Kevin Hay

Rob Holt

Brad Nelson

Julie Nielsen

**This research has been funded by the Canadian Institutes of Health Research (184352)**

# Education and Training

ACCESS Summer Research Program

ACCESS Pediatric Hematology Oncology Career and Education Skills Development (PHOCESD) Conference (Fellows Conference)

Persons With Lived Experience (PWLE) Subsidy Program

Chiquita Hessels, Dawn Pickering, Meera Rayar, Laura Wheaton

# Summer Student Research Program

A virtual education program that is available to any student (undergraduate, graduate, health care professional) working within the ACCESS community. It runs from annually from May to mid-August.

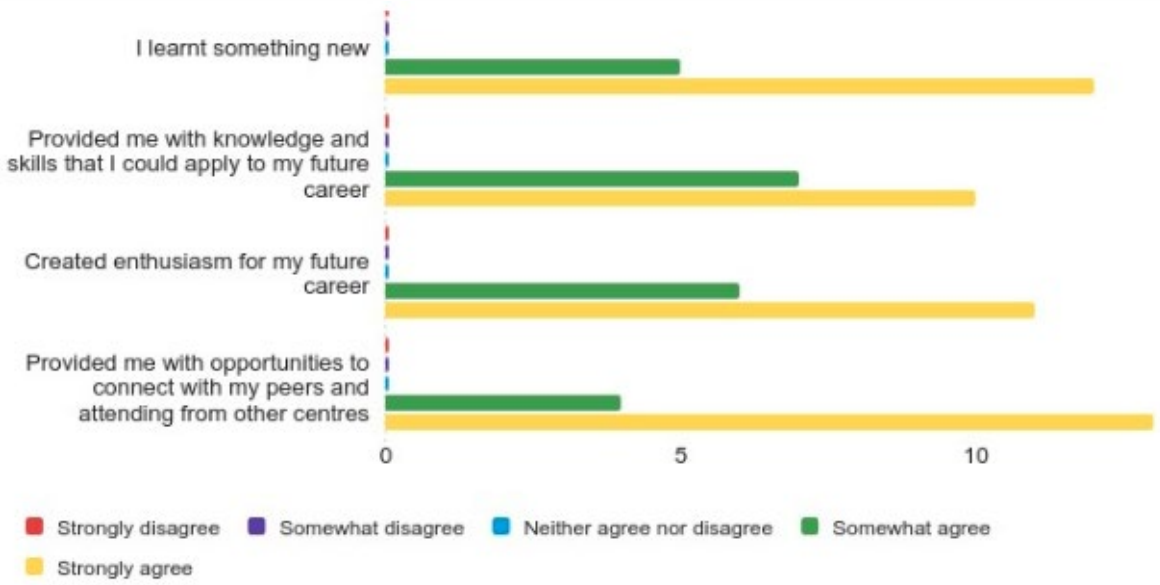

In Summer 2025:

- 43 trainees from across the country
- 15 Undergraduate Students
- 18 Graduate Students
- 1 Post-Graduate Students
- 9 Clinical Trainees

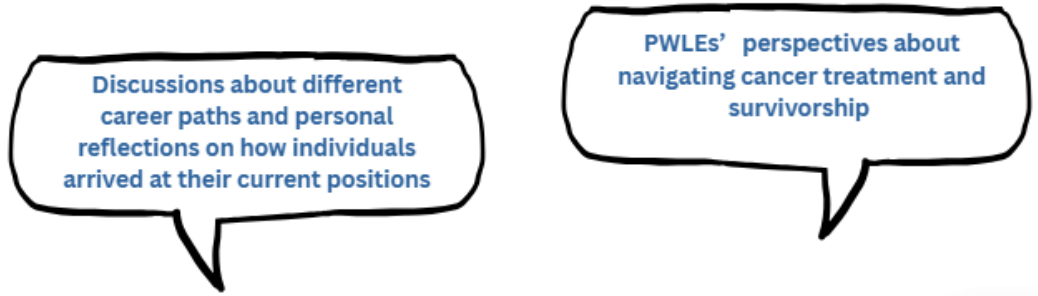
# Pediatric Hematology/Oncology Career Education and Scientific Development Conference

- The PHOCESD Conference took place in Toronto over 2 days in May 2025
- 21 trainees and 3 Program Directors attended the conference

**Conference Evaluation**




**When asked about their experience, attendees particularly valued sessions exploring:**



# PWLE Subsidy Program

Knowledge Translation Professional Certificate™

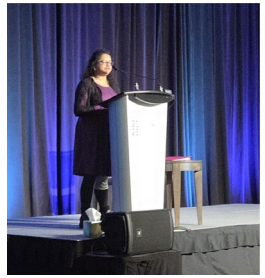
SIOP 2025
INTERNATIONAL SOCIETY OF PAEDIATRIC ONCOLOGY
AMSTERDAM, THE NETHERLANDS | OCTOBER 20-23, 2025

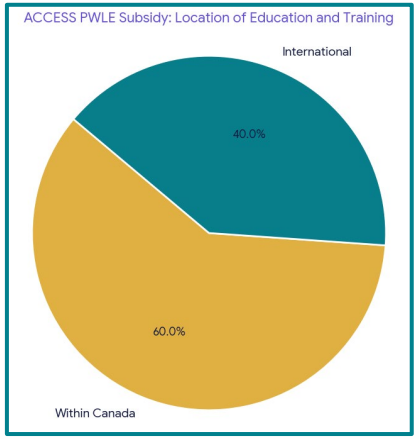
REACH 24
research • education • advocacy • community • hope
7TH INTERNATIONAL
LFS ASSOCIATION SYMPOSIUM
Hosted by Penn Medicine/CHOP and in partnership with the LiFE Consortium

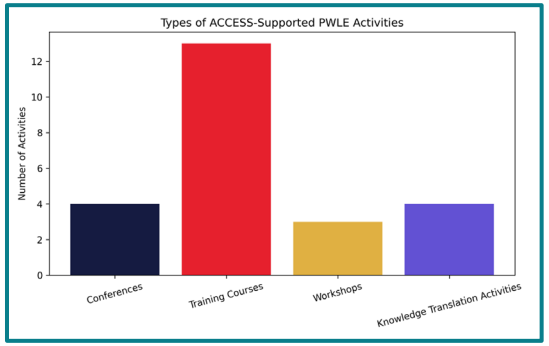

POGO
PEDIATRIC ONCOLOGY GROUP OF ONTARIO
FOR KIDS WITH CANCER
FOR NOW, FOR LIFE.


CCRC 2025
THE CANADIAN CANCER RESEARCH CONFERENCE | LA CONFÉRENCE CANADIENNE SUR LA RECHERCHE SUR LE CANCER

Isber
MONTRÉAL CANADA
MAY 12-16, 2025
ANNUAL MEETING





# Thank you and Acknowledgements


- This research has been funded by the Canadian Institutes of Health Research (184352)
- The PHOCESD was generously also supported by: The Charles Bruneau Foundation, Garron Family Foundation, and Jazz Pharmaceuticals


access acces

CIHR IRSC Canadian Institutes of Health Research Instituts de recherche en santé du Canada

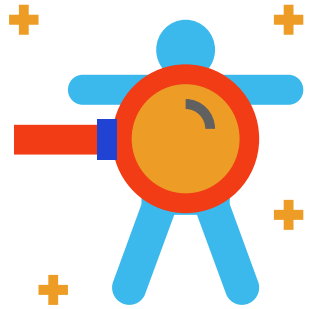
# Health system implementation for innovative diagnostics in childhood cancer

TM4: Avram Denburg & Celine Cressman

CIHR IRSC Canadian Institutes of Health Research
Instituts de recherche en santé du Canada

# Research Context

- Molecular diagnostics offer **incredible promise** for precision oncology
- Substantial **uncertainty** of long-term value
- Little regulation and policy to direct broader clinical implementation
- Policy void is especially relevant for CAYA (clinical, economic, ethical)

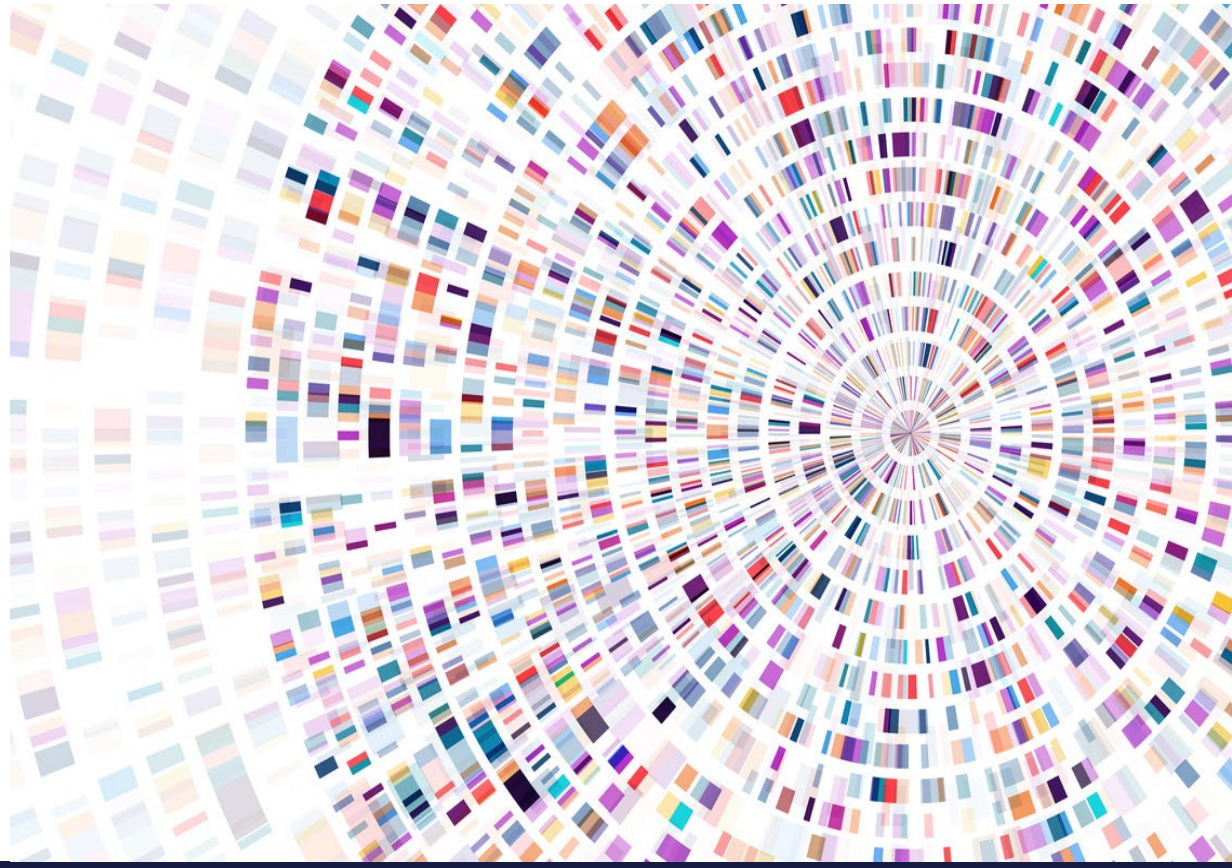


# Project Aims

(i) Map the ***current state*** of policy and system implementation for innovative diagnostic technologies in childhood cancer in Canada

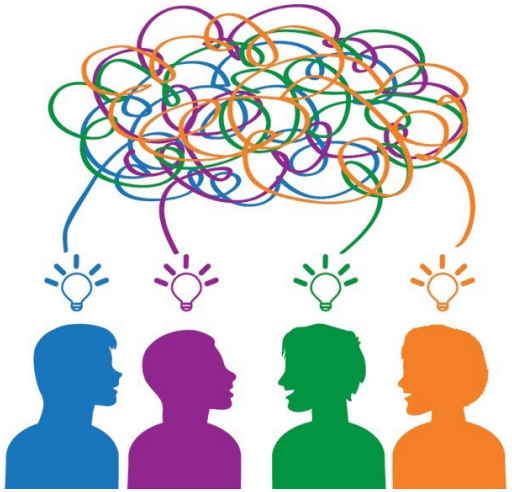
(ii) Generate lessons for an ***ideal future state***

- Focus on
  - exemplar technologies
  - access and equity
  - Canadian setting



# Methods & Design

1. **Survey** of genome sequencing availability
    - clinical and research pathways
    - source of funding
    - location of testing
    - turn-around times
2. **Semi-structured key informant interviews**
    - experience accessing molecular diagnostics for pediatric oncology
    - current challenges and successes
    - ideal future state



# Early Findings: Test Access is Highly Variable

| Type of test | | Range of responses |
|---|---|---|
| WGS | Clinically available | No |
| | Funding | - |
| | Location | - |
| | Turnaround | - |
| | Available via research | PROFYLE |
| | Funding | Research |
| | Location | Ste-Justine/SickKids/BCCH |
| | Turnaround | 6-12 |
| Exome | Clinically available | QC=yes; rest of country=no |
| | Funding | Province; - |
| | Location | In-house / in-province; - |
| | Turnaround | 4; - |
| | Available via research | SIGNATURE/APEC/MCI |
| | Funding | Research |
| | Location | Local to international |
| | Turnaround | 3-8 |

| Type of test | | Range of responses |
|---|---|---|
| DNA | Clinically available | Yes |
| | Funding | Province |
| | Location | Local to international |
| | Turnaround | 1-2 |
| | Available via research | PROFYLE/KiCS/LIBERTY |
| | Funding | Research |
| | Location | Ste-Justine/SickKids/BCCH |
| | Turnaround | 2-8 |
| RNA | Clinically available | Yes |
| | Funding | Province |
| | Location | Local to international |
| | Turnaround | 2-4 |
| | Available via research | PROFYLE/KiCS/APEC/MCI |
| | Funding | Research |
| | Location | Local to international |
| | Turnaround | 3-12 |
| Cyto-genetics | Clinically available | FISH/single gene/microarray |
| | Funding | Province |
| | Location | Local to international |
| | Turnaround | 1-3 |

# Early Findings: Key Informant Interviews

- *N=27*
- *oncology, pathology, bioinformatics, policymakers*

## Experience of the current state:

- An evolving landscape
- Complex patchwork of access
  - Large variation in lab organization & capacity; funding structures
- Absence of standards
- Disparities in access via clinical pathways
  - Quebec vs rest of Canada; larger vs smaller sites
- Greater equity of access via research pathways (PROFYLE, MCI)
  - Inherent uncertainty

**We find it piecemeal… all of a sudden a study closes and we're all kind of going 'what are we going to do?' …It's great as long as it's here**

*–Pediatric oncologist, ON*

# Perceived Challenges in the Current State

a) Policy & System
- blurred boundary between “standard of care” and “research”
- lack of standards
- funding risks

b) Organizational
- institutional and geographic variation
- tissue scarcity and test prioritization
- tension between broad and targeted approaches to sequencing

c) Human
- workforce capacity and expertise bottlenecks
- reliance on individual expertise and comfort

# An Ideal Future Means Equal Access

## Necessary structural & organizational changes:

a. Standardize testing and referral pathways
   - clarity and equity
   - national resource (e.g. Hawkins lab)

b. Expand access pathways for AYA

c. Centralized labs (AB & QC) or distributed model with regional centers
   - efficiency and expertise

**We really need to make sure that if something is accessible in Toronto, it should also be accessible for patients in the Yukon.**

*-Pediatric Oncologist, SK*

**The ideal system would be—not that everyone has the same technology—but everyone has access to the same technology.**

*-Pathologist, BC*

# An Ideal Future Requires Investments in Human Resources

a. Pathologists, technologists, genomic analysts, bioinformaticians to interpret and translate tests
   - Manage the large volume of results
   - Improve TATs

b. Genetic counsellors and researchers to navigate consent and disclosure
   - Decrease burden on clinicians
   - Translate the findings to providers, patients, and families

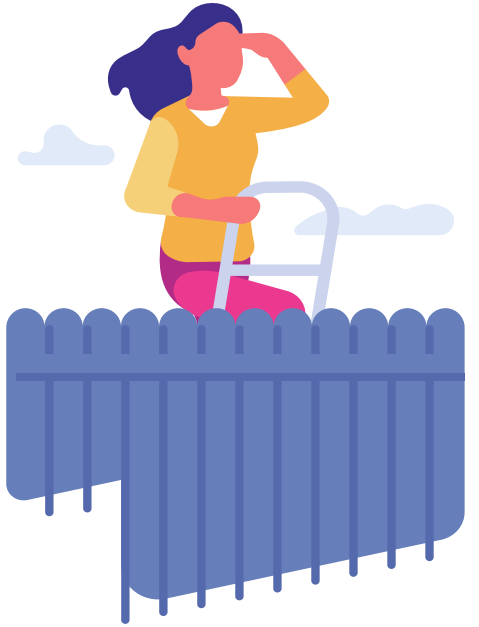
c. Education programs to improve awareness of testing availability
   - Decrease disparities in access based on provider knowledge
   - Increase clinician comfort and confidence with genome diagnostics

# Next Steps

1. Finalize mapping the current and ideal states for Canada
   - cross-provincial data analysis

2. Cross-case comparison: Canada, Australia, UK, Sweden

3. Policy recommendations to decision-makers about how to achieve that ideal state



# Thank you & Acknowledgements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access acces

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# Pan-Canadian Approaches to Data Sharing & Access

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# Introduction

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# Core Elements of Good Data Governance

- **Transparency**
  - Develop clearly defined and accessible information on data sharing, including purpose, processes, procedures and overall governance.
  - Nature of the data.
  - Ensure that a fair process for access adjudication is in place.

- **Accountability**
  - Institute best practices for monitoring and responding to non-compliance
  - E.g. data misuse, data breach, failure to obtain prior ethics review, non compliance with security procedures.

- **Engagement**
  - Facilitate contributions & co-design with patients/participants/PWLE
  - E.g. citizen engagement

    - Global Alliance Framework for Responsible Data-Sharing, Accountability Policy, Consent Policy

# Core Elements of Good Data Governance

- **Data Quality and Security**
  - Store and process data in an accurate, verifiable, unbiased, proportionate and current manner.
  - Ensure feedback on the quality and security
  - Establish proportionate data security measures.
  - Be cognizant of the issues related to lawful requests for access to data.

- **Privacy, Data Protection and Confidentiality**
  - Be cognizant of laws and regulations in your jurisdiction.
  - Privacy and data protection safeguards should be proportionate to the nature and use of the data (open, controlled, registered access).

- **Risk-Benefit Analysis**
  - Undertake a proportional assessment of the benefits and risks of harm in data sharing;
  - Conduct Data-Sharing in a view to minimize harms and maximize benefits to donors, society and the health care system.

- Global Alliance Framework for Responsible Data-Sharing, Accountability Policy, Consent Policy

# Core Elements of Good Data Governance

- **Recognition and Attribution**
  - Design systems of data sharing with a view towards recognition and attribution to all who contributed the results (e.g. ICMJE, etc.)
  - COBRA guidelines for acknowledgments for bioresources (e.g. BRIF, etc.)
  - Acknowledgements, co-authorships, referencing.

- **Accessibility and Dissemination**
  - Maximize the accessibility of data through streamlined processes (while ensuring both ethical and scientific integrity);
  - Promote collaborative partnerships that can generate maximum benefits;
  - Centralized vs. Federated models

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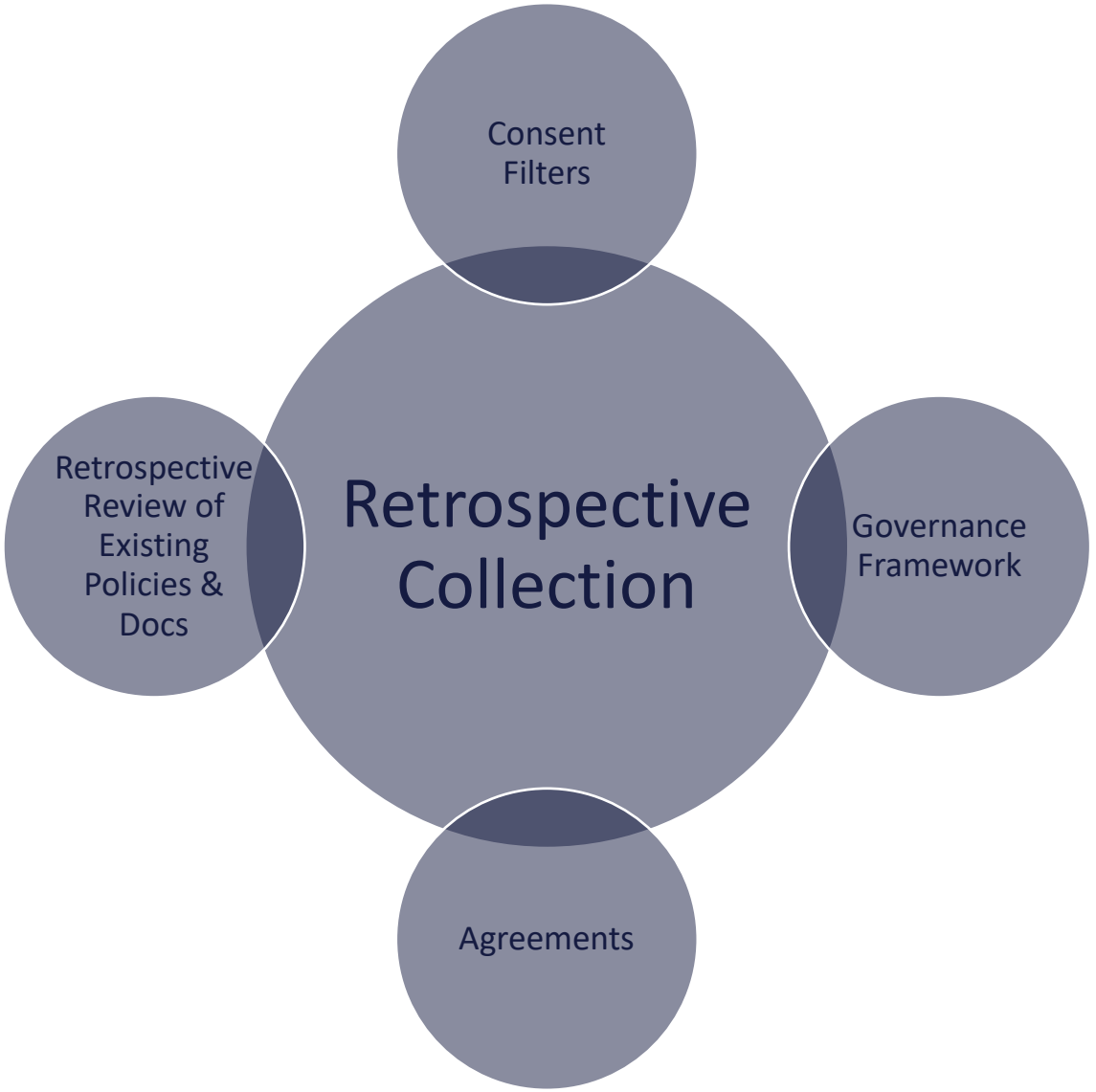
# Prospective Collection

# Retrospective Collection



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# Our Activities

## ACTIVITY 1:
**Facilitating Data Governance and Sharing Across ACCESS Members**

**Participants:** Researchers, legal representatives, and contracts staff across ACCESS partner institutions (target n = 25-30).

**Focus:** The survey examines professional perspectives on data sharing, including its role, definitional clarity, ethical principles, compliance measures, and the use of multi-institutional and external data sharing agreements. It also explores challenges and barriers in data sharing, their impacts on research, and potential solutions.

## ACTIVITY 2:
**Fostering Communication of Patient Data and Access to Results**

**Participants:** Adult survivors of childhood cancer and parents/guardians (target n=30-40).

**Focus:** The survey examines needs, experiences, perspectives, and barriers related to access to medical records and research data. It also explores experiences with return of results, interest in and accessibility of research information, and supports towards meaningful data access.

**PWLE engagement:** Across survey design, deliverables, and dissemination.

# Activity 1 – Preliminary Results

## Perceived Importance and Scope of Data Sharing

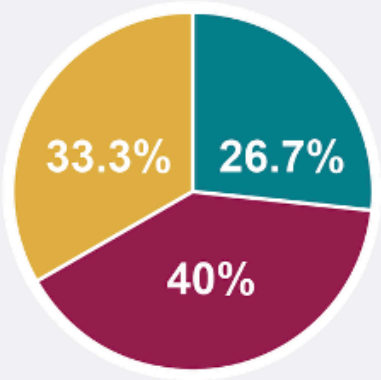
Data sharing was consistently framed as foundational to childhood cancer research. Respondents emphasized the system-wide nature of data sharing impacts, spanning scientific progress, ethics, and public trust.

## Ethical Principles

Open-text responses emphasized the need to operationalize ethical principles through streamlined processes, reduced bureaucracy, trusted coordinating bodies, and alignment with patient and family expectations for socially beneficial data sharing.

## Lack of Shared Understanding

While all sites reported use of data sharing agreements (DSAs), conceptual misalignment persists. Only 26.7% of respondents believed stakeholders share a clear, common definition of a “data sharing agreement”.

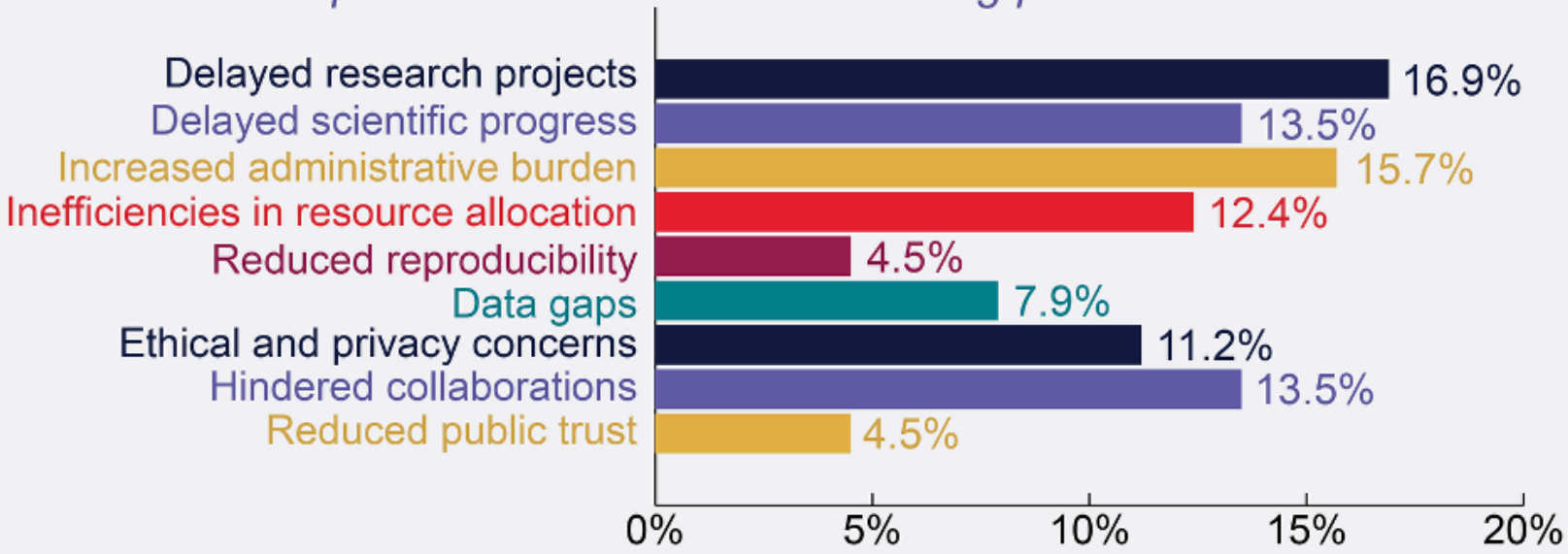



*Do stakeholders have a clear and common definition of a “data sharing agreement”?*

- Yes
- No
- Somewhat

# Activity 1 – Preliminary Results

*What is the impact of issues with data sharing processes on research?*




**Key Resource Constraints**

The most significant resource constraints were funding and training/expertise. Similarly, limited technical expertise and administrative burden were rated as the concerns that most impact the uptake of data sharing.

# Activity 2 – Horizon Scan Findings

**Right of access established, but variably implemented:**

Patients have a recognized legal right to access medical records across Canada; however, procedural requirements and thresholds for refusal vary by jurisdiction and institution.[3]

**Research data present additional barriers:**

Unlike clinical records, research data often lack standardized access routes. Return of individual results is not guaranteed and may depend on clinical relevance, institutional policy, privacy legislation, or "actionability" thresholds.[4]

**Persistent gap between formal rights and lived experience:**

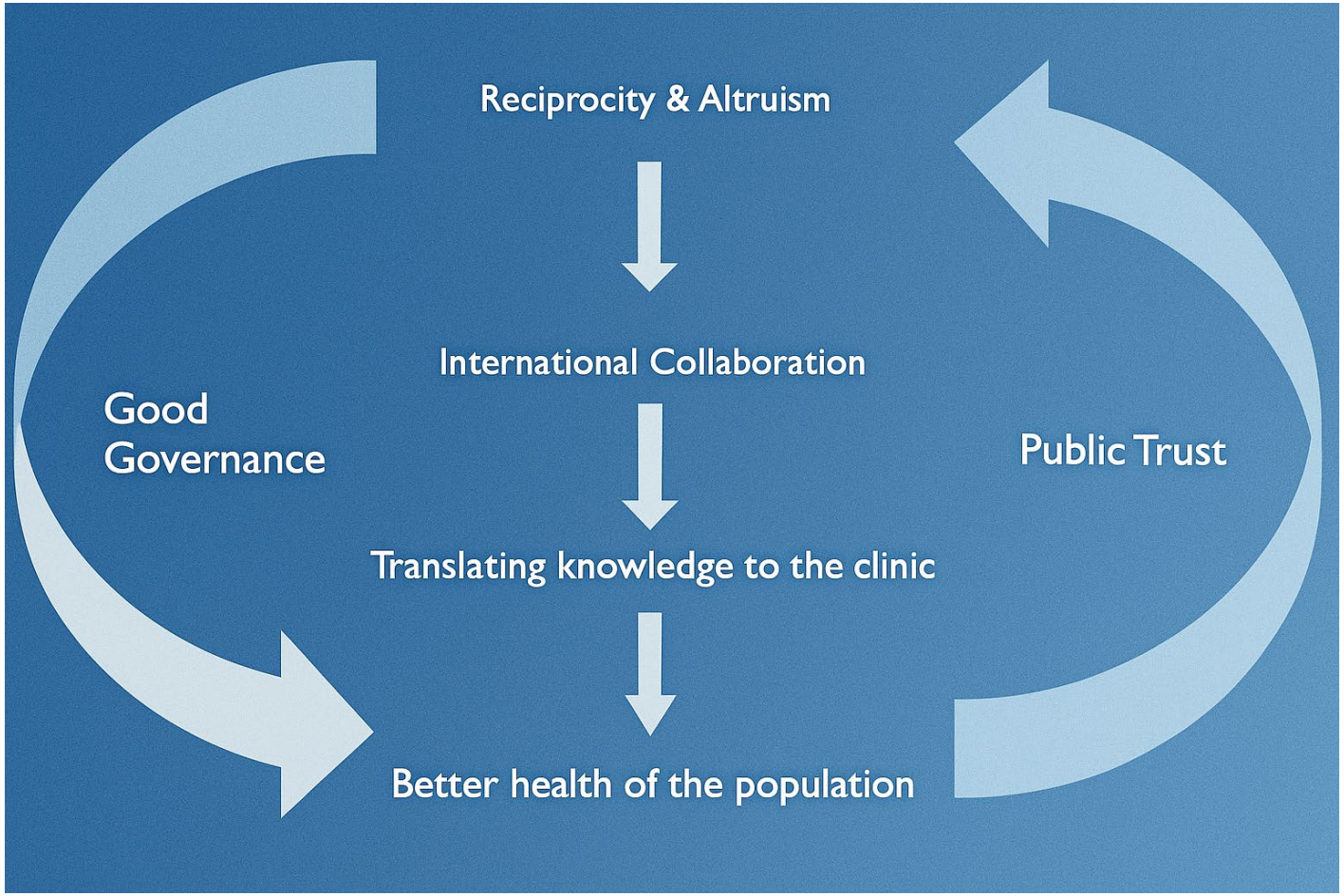
Survivors and families report fragmented systems, administrative burden, dispersed records, and psychosocial barriers (e.g., trauma, research fatigue, survivorship transitions), contributing to unmet information needs.[5]

Interested in participating in one of our surveys?

Contact terese.knoppers@mcgill.ca or man.zawati@mcgill.ca

[3]McInerney v. MacDonald, [1992] 2 SCR 138. https://decisions.scc-csc.ca/scc-csc/scc-csc/en/item/884/index.do; [4]Jarvik, G. P., et al. (2014). Return of genomic results to research participants: The floor, the ceiling, and the choices In between. The American Journal of Human Genetics, 94(6), 818–826; [5]Ilic, A., et al. (2023). The information needs of relatives of childhood cancer patients and survivors: A systematic review of qualitative evidence. Patient Education and Counseling, 114, 107840

# Conclusion: Maintaining the Balance



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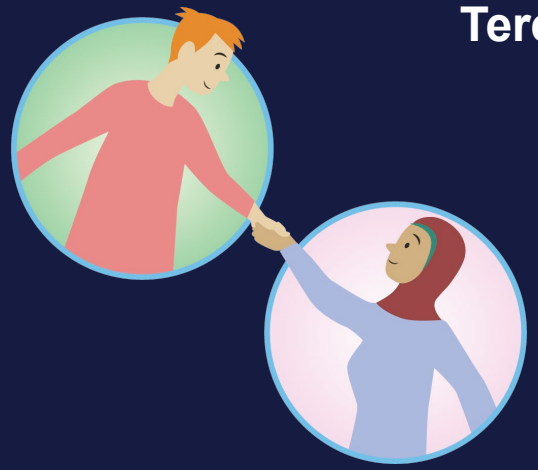

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