September 13th, 2025

How the Patient Voice Enhances Research from Bench to Bedside

A Healthier World

UHN Canada's Hospital

Sonya MacParland, PhD
Ajmera Transplant Centre
University of Toronto

UNIVERSITY OF TORONTO

SickKids

HUMAN CELL ATLAS LIVER

CHAN ZUCKERBERG INITIATIVE

What is Patient Partnered Research?

"Patient-oriented research is about engaging patients, their caregivers, and families as partners in the research process. This engagement helps to ensure that studies focus on patient-identified priorities, which ultimately leads to better patient outcomes.

Citation: CIHR's Strategy for Patient-Oriented Research - Patient Engagement Framework (2014)

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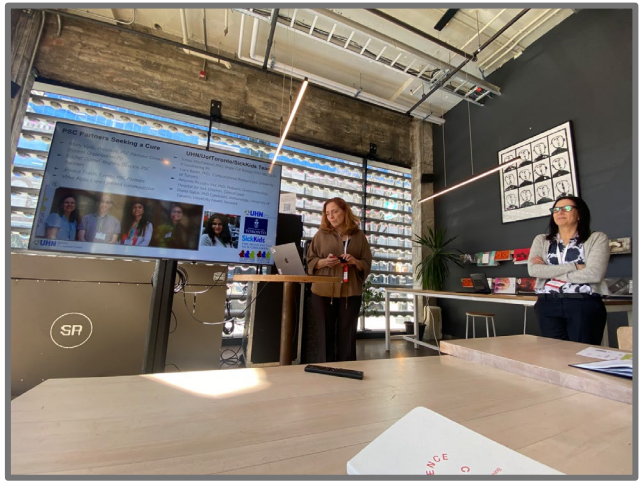
The **Real** Power of Patient Stories

It is important for researchers to place the same importance on the **things that can be learned from patients** as they do on raw scientific data.

-Healthcare research can be much more impactful by ‘doing things with people’ vs. ‘to people’.

-Having the patient voice throughout all aspects of the research grounds the researchers in the “WHY”.



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Guiding Principles of Patient Engagement

Inclusiveness: Patient engagement in research integrates a diversity of patient perspectives and research is reflective of their contribution – i.e., patients are bringing their lives into this.

Support: Adequate support and flexibility are provided to patient participants to ensure that they can contribute fully to discussions and decisions. This implies creating safe environments that promote honest interactions, cultural competence, training, and education. Support also implies financial compensation for their involvement.

Mutual Respect: Researchers, practitioners and patients acknowledge and value each other's expertise and experiential knowledge.

Co-Build: Patients, researchers and practitioners work together from the beginning to identify problems and gaps, set priorities for research and work together to produce and implement solutions.

Citation: CIHR's Strategy for Patient-Oriented Research - Patient Engagement Framework (2014)

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Mary Vyas

Parent of a Young Adult with PSC

Co-Founder and President of PSC Partners Canada

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PSC Partners Seeking a Cure

- *Mary Vyas, President, PSC Partners Canada (Patient Organization PI)*
- *Rachel Gomes, Board of Directors, PSC Partners Canada*
- *Jessica Travis, Events, PSC Partners*
- *Virve Aljas, Expert Patient Communicator*

UHN/UofToronto/SickKids Team

- *Sonya MacParland, PhD, Single Cell Biology (Wet lab) UHN (Coordinating PI)*
- *Gary Bader, PhD, Computational Biology Lead, University of Toronto*
- *Amanda Ricciuto, Md, PhD, Pediatric GI, Hospital for Sick Children, Clinical Lead*
- *Diana Nakib, PhD Candidate, Immunology, University Toronto, University Health Network*

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Patient Organization Research Collaborators

PSC Partners Seeking a Cure

Key Team Members Involved:

- *Mary Vyas, Co-Founder and President, PSC Partners Canada*
- *Rachel Gomel, Board Member, PSC Partners Canada*
- *Virve Aljas, Expert Patient Communicator*
- *Stephen Rossi, PharmD, Chief Scientific Officer, PSC Partners*

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Structure of Our Collaboration

PSC Partners Activities:

- *Organize and host conferences*
- *Lead focus groups*
- *Develop educational resources*
- *Present to diverse audiences*
- *Recruit patients to our Registry and to research studies*
- *Share resources (e.g. CZI, RAO, NORD)*
- *Organize and join working groups*
- *Welcome collaboration*

UHN /UofT Research Team Activities:

- *Generating tools to share data collected accessibly (shiny apps)*
- *Creating avenues for collaboration with adult liver, inflammation and pediatric liver communities*
- *Updating/ sharing protocols and open sharing of data across organ networks*

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Joint Activities

Joint Activities:

- *Provide input at monthly research meetings*
- *Support public outreach and knowledge dissemination through lay presentations, interviews, and PSCP Canada-organized events*
- *Leveraging the PSCP Registry to support recruitment of additional and diverse study subjects*

Centering the voice of the PSC community:

- *Expand existing channels of PSC patient support and engagement with the research community in the US and Canada*
- *Drive knowledge translation with a focus on DEI and accessibility*

Anticipated Challenges:

- *The diversity of languages spoken in patient populations (recruitment materials)*

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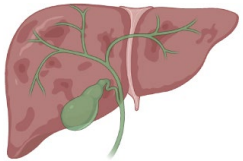
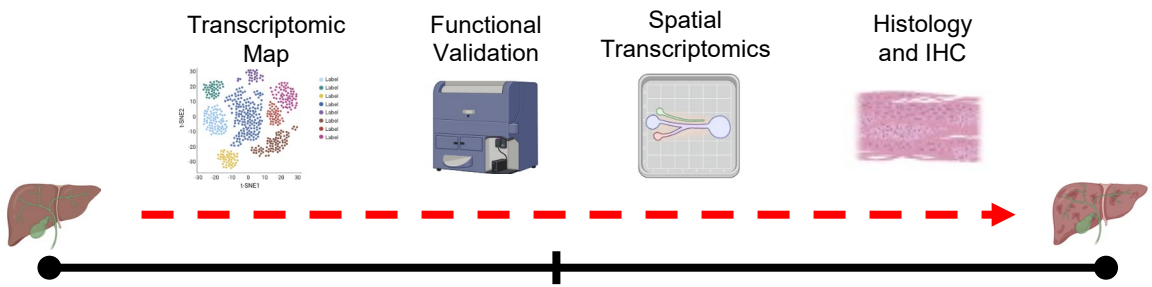
Characterization of PSC at different stages of progression



Explanted PSC Liver

Signatures belonging to:
PSC, End-Stage Liver Disease, and Fibrosis



Diagnostic Biopsies (Pediatric)

Dr. Amanda Ricciuto

SickKids

Research-only Biopsies (Pre-transplant hepatology clinic)

Dr. Gideon Hirschfield

Toronto Centre for Liver Disease

Dr. Amanda Ricciuto

SickKids

Transplantation (Transplant clinic)

Dr. Ian McGilvray

Mark Cattrall

Dr. Blayne Sayed

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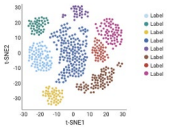
CZI Patient-Partnered Collaboration: Comprehensive characterization of pediatric PSC and PSC-IBD at diagnosis

Chan Zuckerberg Initiative

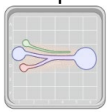
Patient-Partnered Collaborations for Single-Cell Analysis of Rare Inflammatory Pediatric Disease

Functional Validation

Transcriptomic Map



Spatial Transcriptomics



Histology and IHC

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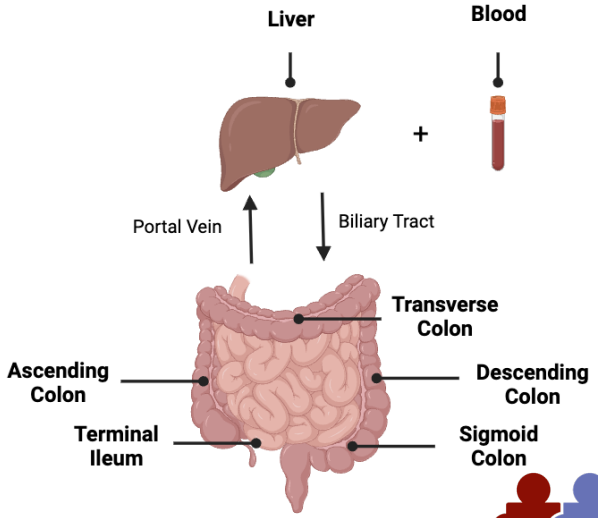

Sampling of matched liver, blood, and gut at diagnosis

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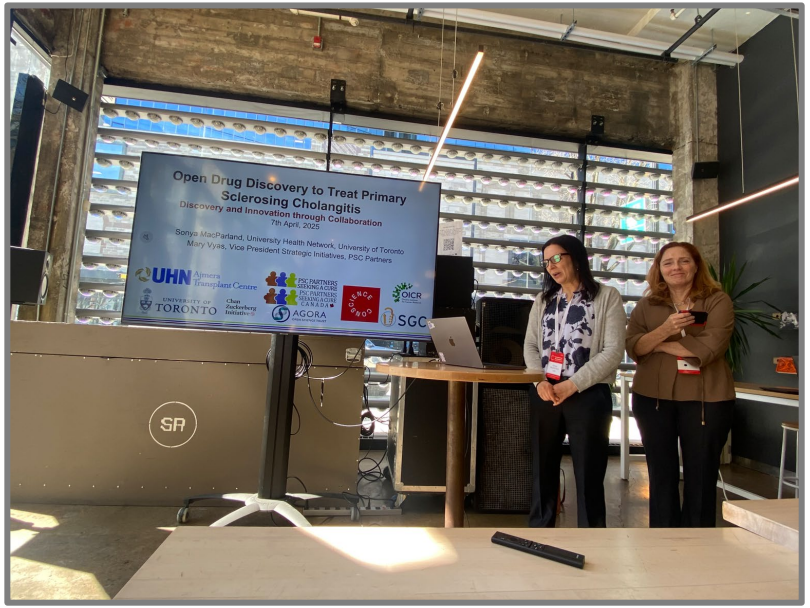
Knowledge Translation Activities

Two-way knowledge translation (Patients present with researchers at research-focused conferences and vice versa)



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Knowledge Translation

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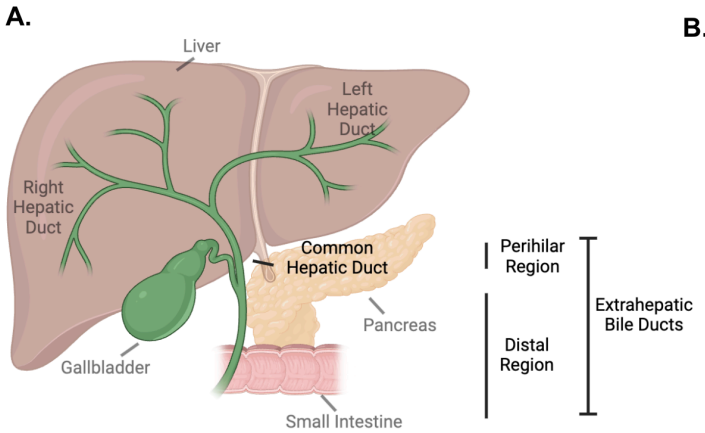
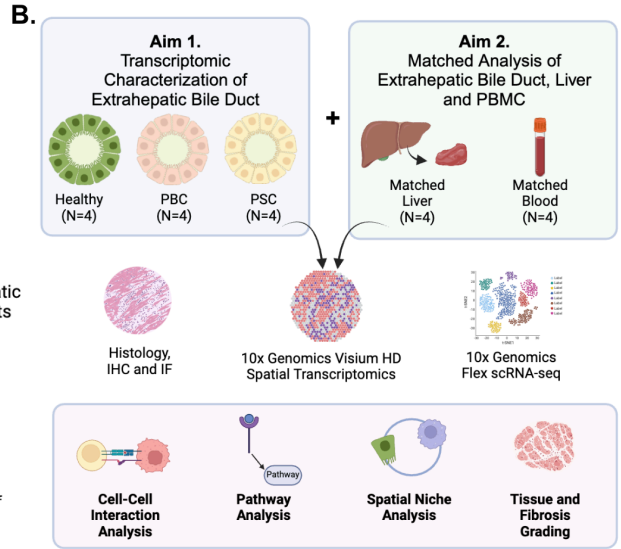
PSCP Funded Research Project: Investigating the extra hepatic bile duct niche





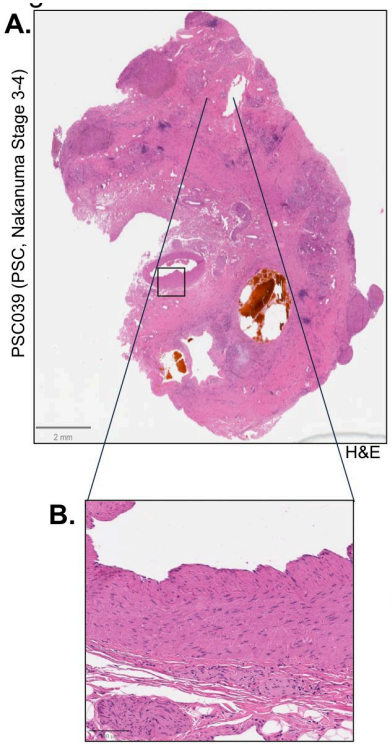


Figure 1. Schematic of sampling strategy and outline of project experimental aims. **A.** Schematic of the biliary tract system in humans. We have collected the distal region of neurologically deceased donor (NDD), PSC and PBC extrahepatic bile ducts at the time of transplantation. **B.** Outline of project aims and sample collection (top), experimental approaches to employ (middle), examples of computational analyses to perform on generate data (bottom).

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CANADA RESEARCH CHAIRS

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Toronto General & Western Hospital Foundation UHN

KNOWLEDGE LIVES HERE.

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

IMMUNOLOGY
FACULTY OF MEDICINE
UNIVERSITY OF TORONTO

NSERC CRSNG

CanHepC
Canadian Network on Hepatitis C
Réseau Canadien sur l'Hépatite C

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