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Pediatric Rare Disease Clinical Trials and Treatment Network

CORD Conference
April 29, 2025

ABOUT US

CIHR Team Grant: National Pediatric Rare Disease Clinical Trials and Treatment Network 2023-10-24 in partnership with the Government of Canada's National Strategy for Drugs for Rare Diseases

Date: January 2024 – December 2028

Administering Institution: Maternal Infant Child and Youth Research Network (MICYRN)

Nominated Principal Investigator: Dr. Thierry Lacaze

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

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MISSION

Establish a cross-jurisdictional platform that develops, manages, and executes cutting-edge clinical trials, providing treatment options and support for children, adolescents, young adults, and their families affected by rare diseases in Canada

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VISION

To ensure that every child, adolescent, and young adult in Canada affected by rare diseases has access to effective and innovative treatments

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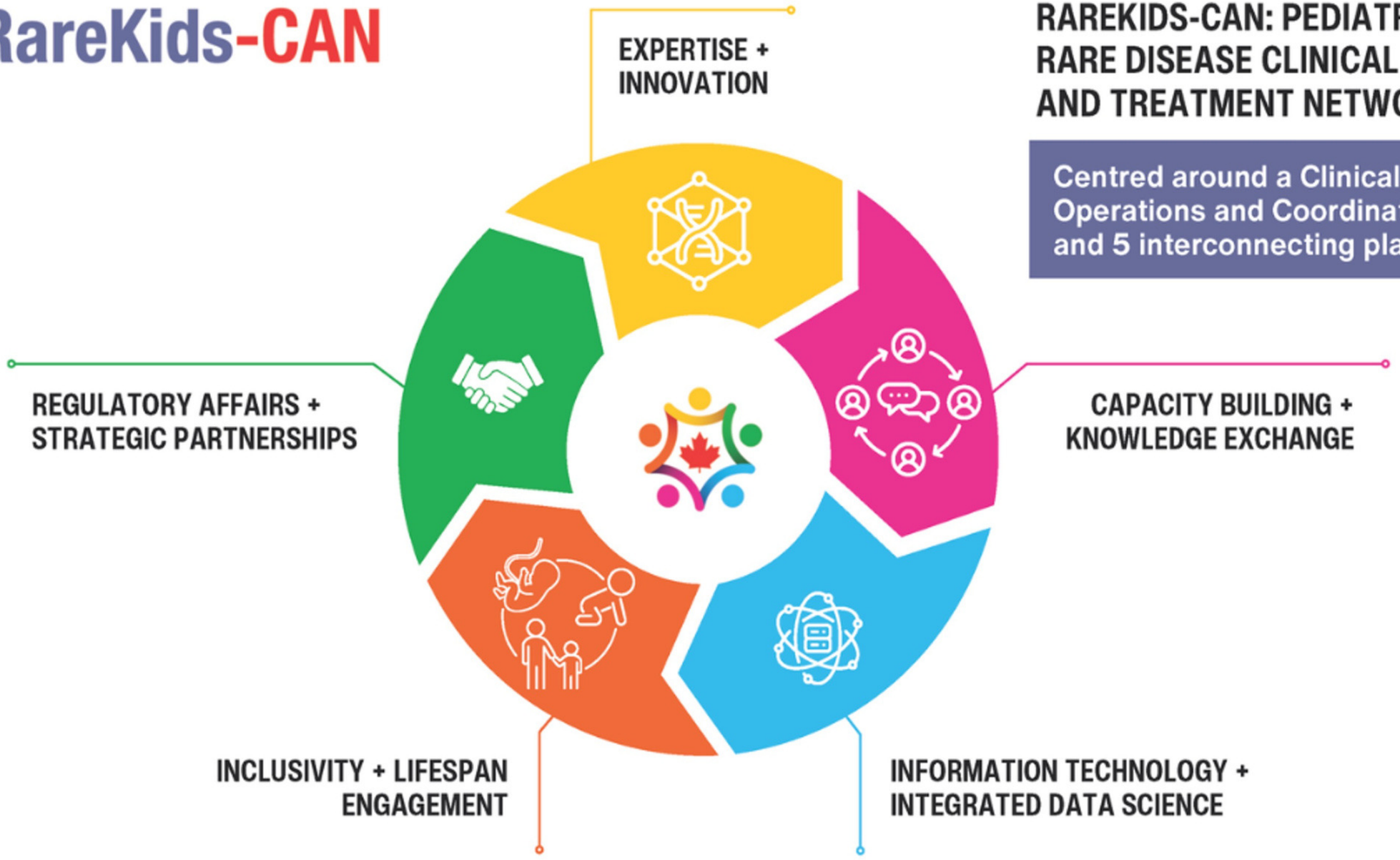
RAREKIDS-CAN: PEDIATRIC RARE DISEASE CLINICAL TRIALS AND TREATMENT NETWORK

Centred around a Clinical Trial Operations and Coordinating Hub and 5 interconnecting platforms



NETWORK MEMBERS

217 RareKids-CAN Members

45 Onboarded in Paid Positions

33 Pt/Family Partners

8 Trainees

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YEAR 1 HIGHLIGHTS

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Patient & Family Engagement

COHORT SPONSORSHIP

- RKC sponsored 24 spots in the 10-week McMaster-based Family Engagement in Research Course
- 12 individuals were pt/family partners
- 12 individuals were RKC members and affiliated researchers

Training & Mentorship

SALARY AWARD

1 salary award through IMPaCT Training Program

WEBINARS

Supported 2 webinars as part of the IMPaCT program related to pediatric rare diseases

Registry-Led

LIVING SCOPING REVIEW

Scoping review underway to develop a living inventory of pediatric rare disease clinical trials, providing a comprehensive overview and supporting future research on trial design and methodology.

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ACADEMIC RESEARCH ORGANIZATION

MICYRN
Maternal Infant Child and Youth Research Network

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PRE-AWARD SERVICES

- CONSULTATIONS
- GRANT APPLICATION REVIEW
- BUDGET SUPPORT
- LETTER OF SUPPORT

*NO COST FOR ACADEMIC/INVESTIGATOR INITIATED PROJECTS

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POST-AWARD SERVICES

- Consultations
- Monitoring
- Data Safety Monitoring Board
- Site & Investigator Identification
- Ethics Submissions
- Knowledge Translation
- Regulatory Submissions
- Contracts Facilitation
- Clinical Trial Sponsorship
- Database Build & Management
- Project Management

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PROJECTS

20
Projects

Registry Only Projects

6/20

Clinical Trial Projects

14/20

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CLINICAL TRIAL NAVIGATORS

- Hired 16 Clinical Trial Navigators across the 16 affiliated pediatric institutions
- Assist with industry sponsored rare disease feasibility assessments/site & investigator identification
- Host monthly virtual meetings to share learnings across 16 sites

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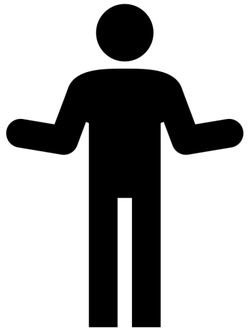
SUCCESS STORY



Initial Pharma Outreach:

- **Investigators: 35**
- **No response/delayed response: 9**
- **Pursuing: 1**

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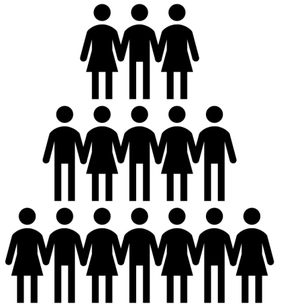


- Leveraged expertise database to identify relevant experts
- Reached out to investigators
- Shared information of clinical trial
- Supported completion of feasibility assessment

Clinical Trial Navigators

- **Pending response: 4 (-5)**
- **Pursuing: 6 (+5)**

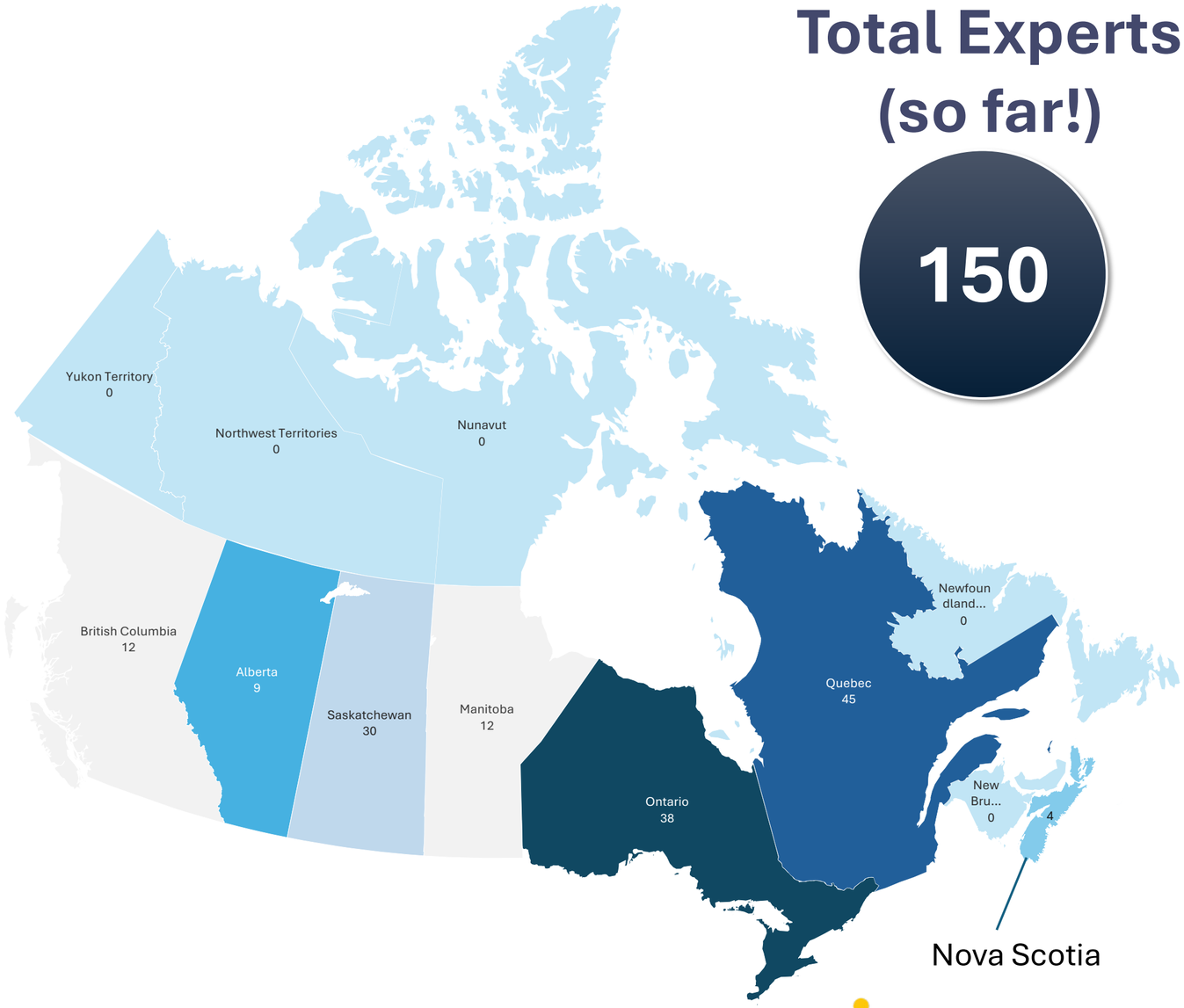
EXPERTISE DATABASE

We've developed a national database of neonatal and pediatric clinical/methodological experts to:

- Join Data Safety Monitoring Boards (DSMB)
- Advise on protocol development
- Serve as site investigators for rare disease trials
- Be contacted as a clinical expert to advise on CDA/ INESSS reviews

Total Experts (so far!)

150



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SUPPORT for INDUSTRY

Experts for Protocol Development (including pt/family partners)

Site & Investigator Identification

Feasibility Assessment

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Health Canada Relations and Advocacy

Lead: Charlotte Moore Hepburn

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SickKids | Child Health Policy Accelerator

ADVOCACY POLICIES

Policy 1:

PRACTIcE Trials-

Pediatric Routinely Administered Clinical Therapeutics In Everyday practice

- Health Canada should establish a full exemption pathway for low-risk pediatric clinical trials involving drugs routinely used in clinical practice.
- White paper drafted, circulated for endorsement

Policy 2:

Special Access Program (SAP) vs. Single Patient Study (SPS)

- Exploring models in other jurisdictions
- Meeting with stakeholders

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STRATEGIC PLANNING

Unfit for Commercialization

Biotech/ Industry Driven

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THANK YOU

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