RareKids CAN

STRATEGIC PLAN 2025-2029

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

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MICYRN
Maternal Infant Child and Youth Research Network

MICYRN is the RareKids-CAN host institution. Thank you to our member institutions for their support and in kind contributions.

MESSAGE FROM THE NOMINATED PRINCIPAL INVESTIGATOR AND NETWORK DIRECTOR

We are happy to share the revised RareKids-CAN Strategic Plan. This plan reflects our evolution into a more focused, coordinated, and impact-driven national network. It responds to feedback from researchers, patient and family partners, and our leadership team.

Our initial grant application, supported by a Clinical Trial Operations and Coordinating Hub and fifteen sub-platforms, set out an ambitious, condition- and intervention-agnostic national infrastructure to deliver coordinated pre- and post-award support for investigator-initiated and industry sponsored Pediatric Rare Disease Clinical Trials (PRDCTs) across Canada.

RareKids-CAN supported fourteen clinical trial projects and six registry projects in our first year. These early activities confirmed the value of centralized national infrastructure while highlighting the need for greater focus, coordination, and system-level alignment.

Our revised strategy shifts from a project-by-project model to a system-enabling approach, prioritizing initiatives that can deliver national-scale outcomes and support long-term sustainability.

We are grateful to the patient and family partners, researchers, institutions, funders, policy leaders, and collaborators whose insights have shaped this evolution. Together, we look forward to advancing a more connected, efficient, and equitable clinical trials ecosystem for children and families affected by rare diseases in Canada.

Sincerely,

DR. THIERRY LACAZE, Nominated Principal Investigator

BREANNE STEWART, Network Director

THE CHALLENGE

Children and families affected by rare diseases face uniquely urgent and complex challenges. In Canada, pediatric rare disease clinical trials are often difficult to launch, slow to scale, and unevenly available across regions. This creates barriers to participation, delays access to innovation, and places unnecessary burdens on families.

These challenges are not confined to individual studies or institutions. They reflect broader system-level gaps in trial readiness, coordination, regulatory alignment, and equitable access. Addressing them requires more than project-by-project solutions, it demands a coordinated national approach that connects people, strengthens infrastructure, and modernizes how pediatric rare disease research is conducted and integrated into care.

ACCELERATING ACCESS THROUGH COLLABORATION

RareKids-CAN brings together researchers, health care teams, patients and families, policymakers, and industry partners across 16 child health research institutions in Canada to simplify and strengthen the pediatric rare disease research landscape.

Our network is powered by a centralized Clinical Trial Operations and Coordinating Hub based at MICYRN, supported by 15 expert-led sub-platforms with leads and teams across Canada. Together, they translate strategy into action by providing specialized expertise across key domains of pediatric rare disease research.

Our network is further strengthened by 16 Clinical Trial Navigators embedded across participating child health research institutions, who provide essential on-the-ground coordination and expertise. They connect investigators to resources, facilitate information sharing, identify trial opportunities, and support study start-up and execution.

To ensure that lived experience meaningfully shapes research priorities, trial design, and system improvements, more than 40 patient and family partners are actively engaged across our initiatives.

By connecting people and systems, we accelerate access to clinical trials and innovative treatments while reducing duplication, regional inequities, and the travel burden placed on families. Through improved research readiness, trial matching, and supportive policy frameworks, RareKids-CAN helps ensure therapies reach children and families sooner and more equitably. The network also enhances Canada's capacity to deliver high-quality pediatric rare disease trials.

ABOUT REKIDS-CAN

MISSION

Advancing pediatric rare disease research and treatment through cross-jurisdictional collaboration — strengthening trial readiness, connecting patients and sites, and driving regulatory innovation to improve access to advanced therapies for children and families across Canada.

VISION

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

OUR STRATEGIC SHIFT

EARLY MOMENTUM & KEY LEARNINGS

In RareKids-CAN's first year, we established ourself as a national convener and catalyst. The network united researchers, clinicians, patient and family partners, institutions, and sponsors from across the country to strengthen support for pediatric rare disease clinical trials. Through centralized national services, RareKids-CAN helped study teams navigate regulatory requirements, build high-quality data and registry systems, identify trial opportunities, and move studies forward more efficiently.

Early demand for these services confirmed a clear national need for shared infrastructure and coordinated support. Just as importantly, RareKids-CAN's early work provided critical insight into where the network can have the greatest impact. Experience across multiple trials and registries highlighted persistent challenges, including long study start-up timelines, difficulty identifying and enrolling participants, and variability in how clinical research is embedded into care across provinces. These challenges are systemic in nature — and they require systemic solutions.

FROM INDIVIDUAL PROJECTS TO SYSTEM-LEVEL IMPACT

Informed by lessons from year one, and guided by ongoing engagement with patient and family partners, researchers, institutions, and advisors, RareKids-CAN has refined its approach. This strategic plan reflects a deliberate shift from supporting individual projects in isolation to strengthening the conditions that allow pediatric rare disease clinical trials to succeed at scale.

RareKids-CAN remains committed to supporting individual clinical trial projects; however, our strategic focus is expanding to prioritize national readiness, system alignment, and equitable access across the pediatric rare disease clinical trials ecosystem. By addressing shared barriers, such as fragmented infrastructure, inconsistent processes, and misaligned policies, RareKids-CAN strengthens Canada's capacity to attract and deliver innovative pediatric rare disease trials, including advanced therapy medicinal products, and enables access to treatment. Our revised strategy is anchored around three priorities: 1) Strengthening Pediatric Rare Disease Clinical Trial Capacity and Advanced Therapy Medicinal Product Readiness; 2) Optimizing Participant/Site/Trial Matching; 3) Driving Regulatory Reform and System Innovation.

75% OF RARE DISEASES MANIFEST DURING CHILDHOOD, YET FEWER THAN 5% HAVE AN APPROVED TREATMENT.

STRATEGIC PRIORITY 1

STRENGTHENING PRDCTs CAPACITY AND ADVANCED THERAPY MEDICINAL PRODUCTS (ATMPs) READINESS

Strategic Priority One focuses on strengthening the capacity of Canadian pediatric research institutions and affiliated hospitals to lead and deliver high-quality, innovative PRDCTs, while building readiness for the safe and effective administration of ATMPs (ex vivo and in vivo gene therapies, genome editing, antisense oligonucleotides therapies, and cell therapies) across research and clinical settings. This priority is advanced through the following initiatives:

ACADEMIC LEADERSHIP, GUIDANCE & TRAINING

Foster excellence in PRDCTs design, conduct and execution by supporting, creating, expanding, and disseminating new knowledge.

CENTRALIZED SUPPORTS FOR PRDCTs

Through centralized and subsidized services, we reduce costs, improve efficiency, and accelerate trial initiation.

ATMPS TRAINING MATERIAL & STANDARD OPERATING PROCEDURES (SOPs)

Develop materials to support the safe and effective handling and administration of ATMPs.

NETWORK OF PEDIATRIC ATMPs CLINICAL TRIAL AND DELIVERY-READY UNITS

Support the development of a Community of Practice who share a common interest in the ATMPs clinical trial and delivery space.

INNOVATIVE MODELS

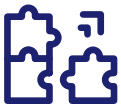
Advance decentralized and patient-centered clinical trial models to enhance accessibility, equity, and participation in PRDCTs.

1 IN 12 CANADIANS ARE AFFECTED BY A RARE DISEASE, TWO-THIRDS OF THEM ARE CHILDREN

STRATEGIC PRIORITY 2

OPTIMIZING PARTICIPANT/ SITE/TRIAL MATCHING

Strategic Priority Two focuses on optimizing participant, site, and trial matching to improve the efficiency of PRDCT initiation and delivery, while increasing Canada's attractiveness for investment. This priority aims to create an integrated ecosystem that supports patient identification, recruitment, feasibility assessment, site and investigator identification, and registry use, and is advanced through the following initiatives:



TRIAL MATCHING & PATIENT IDENTIFICATION

Develop a centralized, data-driven portal that supports trial planning, attracts new PRDCTs, and enables optimized patient-trial matching across Canada to enhance PRDCT visibility and feasibility.

REGISTRY DEVELOPMENT SUPPORT

Advance the development, maintenance, and use of patient registries and real-world data to support the full pediatric rare disease clinical trials life cycle.

NATIONAL EXPERTISE CATALOGUE

Develop and maintain a centralized database of methodological and RD clinical experts to assist with protocol development and site and investigator identification.

ONLY 60% OF TREATMENTS FOR RARE DISORDERS MAKE IT INTO CANADA

STRATEGIC PRIORITY 3

DRIVING REGULATORY REFORM AND SYSTEM INNOVATION

Strategic Priority Three focuses on driving regulatory reform and system innovation to enable timely access to pediatric rare disease therapies in Canada. Through active engagement with Health Canada and international partners, this priority aims to reduce regulatory barriers across clinical trial development, conduct, and treatment access, and is advanced through the following initiatives:

REGULATORY PATHWAYS

Identify policy objectives that promote a competitive Canadian regulatory environment aligned with international best practices and reduce barriers to pediatric rare disease treatments.

GLOBAL ENGAGEMENT

Continue to build on international collaborations to increase access to innovative PRDCTs and improve pediatric rare disease outcomes in Canada.

ACCESS PATHWAYS FOR THERAPIES UNFIT FOR COMMERCIALIZATION

Advance initiatives that support the development and implementation of therapies that are unlikely to be commercially viable but offer meaningful benefits for rare disease patients and their families.

LOOKING AHEAD

This strategic plan positions RareKids-CAN to deliver meaningful, long-term impact; supporting today's clinical trials while building a stronger, more connected foundation for the future of pediatric rare disease research in Canada. By strengthening national coordination, advancing regulatory and system innovation, and centring the needs of children and families, RareKids-CAN is helping ensure that promising therapies are not only developed but delivered more equitably and efficiently.

OUR IMPACT

RareKids-CAN's three strategic priorities are designed to generate meaningful, measurable, and lasting impact for children and families affected by rare diseases across Canada.

STRENGTHENED NATIONAL CAPACITY & ATMP READINESS

Through Strategic Priority 1, we will strengthen Canada's ability to attract, develop, and conduct high-quality, innovative PRDCTs, including ATMPs. By enhancing institutional readiness and increasing the number of highly qualified personnel in PRDCTs, Canada will be better positioned to participate in and lead transformative research. This increased capacity will reduce delays, improve trial quality, and ensure that Canadian children can safely access cutting-edge therapies within both research and clinical environments.

AT THE END OF YEAR 5, WE WILL HAVE DEVELOPED:

- A searchable, living national inventory of PRDCTs to support collaboration, design optimization, and performance monitoring
- Comprehensive toolkits, online modules, and guidance resources co-developed with patient partners
- A travel fund to reduce participation barriers for patients and families
- Standard operating procedures (SOPs) to support domestic and cross-border shipping, handling, and administration of advanced therapies
- A formal Community of Practice on advanced therapy trials involving at least half of Canada's pediatric research institutions

MORE EFFICIENT TRIAL IMPLEMENTATION & EQUITABLE ACCESS

Through Strategic Priority 2, we will improve how patients are identified, matched, and connected to relevant clinical trials. By building a more integrated national ecosystem for patient identification, recruitment, feasibility assessment, site and investigator engagement, and registry support, the network will reduce inefficiencies in trial start-up and execution. This infrastructure will also address inequities driven by geography, awareness, and resource limitations — ensuring that participation in research is not determined by postal code. By improving trial efficiency and visibility, Canada will become a more attractive environment for investment while expanding access to therapies for families nationwide.

AT THE END OF YEAR 5, WE WILL HAVE DEVELOPED:

- A national conceptual portal to support patient identification and therapy development
- A searchable catalogue of active and recruiting PRDCTs and registries in Canada to help patients and families identify participation opportunities
- Tools and guidance documents to support project teams developing a pediatric rare disease registry

MODERNIZED REGULATORY AND POLICY ENVIRONMENT

Through Strategic Priority 3, we will drive regulatory reform and system innovation to enable more timely access to pediatric rare disease therapies. By actively engaging with Health Canada, provincial and territorial partners, and international collaborators, the network will work to reduce regulatory barriers to trial development, oversight, and treatment access. This includes advancing policy discussions related to clinical trial oversight, the Special Access Program, single patient study models, investigational device testing, pediatric development planning, and therapies not suited for commercialization. We will also advocate for the integration of clinical trials into standard of care and explore innovative reimbursement pathways, including hospital exemption models.

AT THE END OF YEAR 5, WE WILL HAVE DEVELOPED:

- Five policy objectives supported by white papers
- Strengthened international collaborations to align regulatory and research pathways
- A feasibility study exploring delivery pathways for therapies not suited for commercialization

OVERALL IMPACT

Collectively, these priorities will transform Canada's pediatric rare disease ecosystem — strengthening infrastructure, accelerating trial delivery, modernizing regulatory pathways, and improving equitable access to innovative therapies. The ultimate impact will be a more responsive, coordinated, and sustainable system in which every child, adolescent, and young adult living with a rare disease in Canada has a meaningful opportunity to access safe, effective, and innovative treatments.

RareKids CAN

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