RÉSEAU CANADIEN DES MALADIES RARES

CANADIAN RARE DISEASE NETWORK

Rare Lives, Shared Strength

REPORT TO THE COMMUNITY

2024-25

one child every child | UNIVERSITY OF CALGARY

BUILDING MOMENTUM

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ABOUT CRDN

Who We Are

The Canadian Rare Disease Network (CRDN) is a pan-Canadian network that aims to bring together the country's leading clinical, scientific, and patient expertise from across Canada around one common goal: **addressing the unique challenges faced by millions of children and adults affected by rare diseases in Canada**. Currently administratively housed at the Alberta Children's Hospital Research Institute and enabled through support from the University of Calgary's One Child Every Child initiative, CRDN is co-led by a diverse team of experts.

Francois Bernier
Alberta Children's Hospital, University of Calgary

Kym Boycott
Children's Hospital of Eastern Ontario, University of Ottawa

Leanne Ward
Children's Hospital of Eastern Ontario, University of Ottawa

Ian Stedman
York University

Durhane Wong-Rieger
Canadian Organization for Rare Disorders (CORD)

Vision

Innovative care and research in Canada so that all patients and families affected by a rare disease are empowered to live their full potential.

Mission

Establish a growing network that builds connections across geographies and disease boundaries to enable timely diagnosis, screening, and access to treatment, facilitate best care, support and empowerment, and global sharing of best practices for patients and their families in Canada, ultimately enhancing their quality of life.

How We Work

CRDN is committed to driving meaningful change by working **through partnerships** with key stakeholders across Canada and globally. Rather than duplicating efforts, we aim to leverage existing expertise and resources, align priorities, and foster collaboration to maximize impact for the rare disease community. Every contributor, from researchers to those delivering care, and from patients sharing their experiences to those advocating for change, and everything in between, plays an essential role in driving CRDN's mission and vision forward in the years to come.

Our Objectives

Our work is strategically centered around 3 interconnected pillars that are focused on addressing the key challenges faced by rare disease patients and families, and a 4th 'enabling' pillar that spans across all areas and aligns efforts and partnerships at both the national and global level:

PILLAR 1: DIAGNOSTICS & REGISTRIES

Reducing the time it takes to identify rare diseases

PILLAR 2: INNOVATIVE THERAPIES

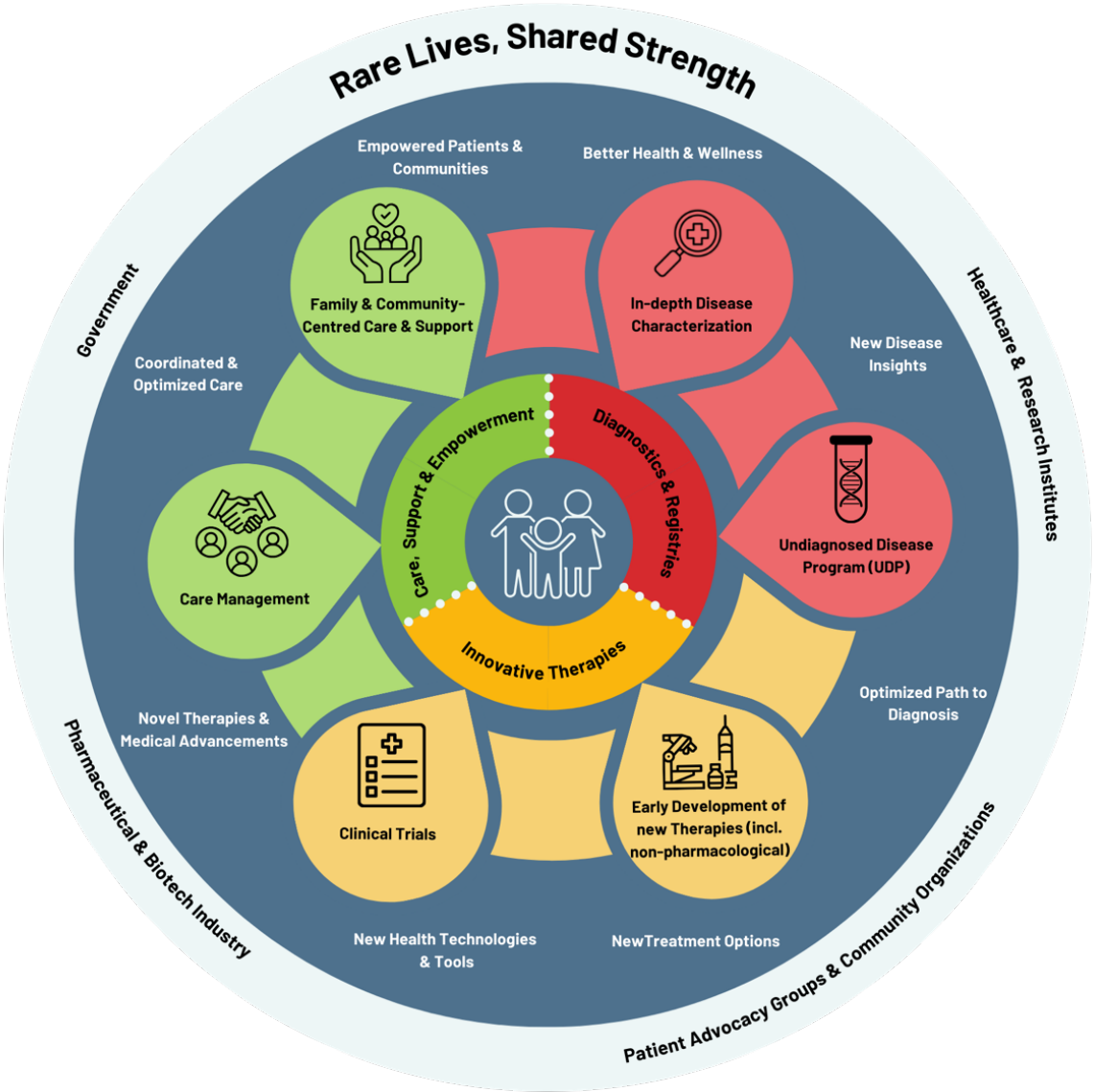
Expanding treatment possibilities

PILLAR 3: CARE, SUPPORT, & EMPOWERMENT

Supporting patients and their families

PILLAR 4: NATIONAL & GLOBAL COLLABORATION

Connecting Canada to drive rare disease breakthroughs



Support

CRDN is currently enabled by the University of Calgary's One Child Every Child research project supported by the Canada First Research Excellence Fund (CFREF).

MESSAGE FROM THE CHAIR & MANAGER

What an exciting first year it has been for the Canadian Rare Disease Network (CRDN), and what an honour to present our inaugural report to our community. This past year has been foundational for CRDN in establishing operations, building a strong team, including our Pillar Groups and administrative team, and co-developing our strategic roadmap for the coming years.

One of our first steps was to articulate CRDN's vision and mission, setting a clear direction for our work. Through extensive consultations, we co-developed a strategic plan built around four key pillars: Diagnostics and Registries, Innovative Therapies, Care, Support, and Empowerment, and National and Global Collaboration. These pillars will guide our efforts and set a clear course for achieving meaningful, long-term impact.

This year has also been about connection – building relationships across sectors, initiating partnerships, and fostering a collaborative spirit. Notably, we've begun coordinating efforts with other organizations, researchers, and patient groups, ensuring that CRDN complements and works in synergy with existing initiatives to maximize collective impact

Since launching, we been generating national engagement and some exciting progress towards our four strategic objectives.

As we look ahead, our focus remains on deepening collaborations, supporting innovation, and advancing equity in rare disease care and research. CRDN is still in its early stages, and while there is much work to be done to ensure we address the challenges faced by people affected by rare diseases in Canada, we have built momentum to move ahead and gathered learnings that we will leverage. We are proud of what we have accomplished together thus far and deeply grateful to all of you who have contributed your time, expertise, and passion to support CRDN's mission.

We look forward to continuing this journey with you – working together to build a stronger, more connected, and more impactful network that enables innovative care and research so that all patients and families affected by rare diseases are empowered to live their full potential.

Sincerely,

Francois Bernier
Chair, CRDN

Svenja Espenhahn
Manager, CRDN

YEAR IN REVIEW - HIGHLIGHTS 2024-2025

Launching on Rare Disease Day 2024 & Building Momentum

CRDN builds on years of dedicated advocacy by many patient organizations across Canada. Momentum surged with the federal government's historic $1.5 billion investment in *the National Strategy for Drugs for Rare Diseases* in 2023 — creating a critical opportunity to unite diverse stakeholders around a shared goal: improving the lives of individuals and families affected by rare diseases in Canada.

The foundation for CRDN was laid in June 2023 at a Canadian Organization for Rare Disorders (CORD) conference, where key leaders from research, healthcare, and patient organizations came together to shape its vision. Shortly after, a **15-member Steering Committee** was formed, bringing together a coalition of experts, including national patient organizations such as CORD and Regroupement québécois des maladies orphelines (RQMO), committed to building a collaborative, impactful, and inclusive network. Since then, the Steering Committee has convened 6 times, focused on establishing a strong foundation for action and aligning efforts.

This momentum culminated in **CRDN's official launch on February 29, 2024**, in front of 250+ attendees of the CORD conference in Ottawa. It was here that CRDN's leadership presented CRDN's mission for the first time, and invited attendees to share their hopes for the future of rare diseases in Canada. The launch was also celebrated with a press conference at the National Press Theatre in Ottawa, where our Chair François Bernier alongside Steering Committee member, Durhane Wong-Rieger from CORD, marked Rare Disease Day and spoke to the launch of CRDN. The launch, amplified by various news outlets (e.g., Canada Newswire, CTV News), helped build our initial momentum, but was just the beginning of our journey to improve the lives of those affected by rare diseases across Canada.

"By uniting patient, healthcare, academic, and advocacy organizations from across Canada under a common banner, the network seeks to amplify its impact on the entire spectrum of rare diseases – from diagnosis to treatment, care, and psychosocial support."
Gail Ouellette, iRARE Centre, RQMO

Building our Roadmap Together

In our first year, an essential part of our work was to **identify strengths and gaps** within the rare disease community. We brought together 34 individuals from across Canada and various sectors to form our three Pillar Committees – Diagnostics and Registries; Innovative Therapies; Care, Support, and Empowerment. These committees met a total of 15 times, contributing over **1,000 minutes of meaningful dialogue to shape CRDN's strategic priorities** and strategies, and ensuring that CRDN works in a complementary, synergistic fashion with existing efforts.

To broaden input, we hosted a virtual 1-hour **town hall** on October 31, 2024, sharing the draft strategic plan with the rare disease community. Over 260 people registered, and 160 attended live, participating in a Q&A session and engaging with the plan.

During the town hall, we launched a **public feedback survey**, offered in both English and French, to provide an opportunity to the broader rare disease community to share their feedback and thoughts on our strategic plan. The survey received **115 responses from individuals across 10 out of Canada's 13 provinces and territories** and from across research, healthcare, patient organizations, industry, and government.

This valuable input refined our strategic plan, ensuring it reflects the voices of those we serve and supports meaningful progress for Canada's rare disease community. Our strategic plan will allow us to move forward with a strong understanding of needs and opportunities, guiding our collaborative efforts.

"I'm thrilled that psychosocial support has been thought of and included... Well done!"
Anonymous survey respondent

"The more I read the CRDN's Strategic Plan, the more elated I became. It is incredibly encouraging."
Anonymous survey respondent

Branding our Network & Launching Communication Channels

Prior to our official launch, we set out to create a recognizable identity for CRDN that reflects the diversity and unity of the rare disease community. Through a collaborative process involving public engagement, we developed a logo and visual identity that would resonate with our audiences across Canada.

Our logo, featuring an array of vibrant colors, is more than a visual mark—it symbolizes the interconnectedness, diversity, and shared purpose of the rare disease community. The public vote to select the logo wasn't just about design preferences; it was an opportunity to involve patients, caregivers, researchers, healthcare professionals, and advocates in shaping the identity of a network built for and by them. This co-creation process emphasized our emphasis and commitment to inclusivity and partnership.

Follow us & help build our momentum

To connect with and inform our varied audiences, we launched **multiple communication channels**, most of these being bilingual (English and French):

- Our **website** is being viewed across Canada, USA, Europe, UK, India, and beyond and also hosts our **Rare Disease Events Calendar** – a one-stop shop for all rare disease-related happenings, from webinars and conferences, to workshops and other events taking place in Canada and beyond;
- Our monthly **Rare Insights newsletter**, which has gained 360+ subscribers during the first year and highlights key developments and initiatives across rare disease research, healthcare, patient advocacy, and more;
- And our **social media feeds** (LinkedIn and X), with over 2,100 followers gained in our first year.

Growing Awareness

As a new initiative, CRDN is still largely unknown to many in the healthcare, patient, and research communities. Thus, the CRDN leadership team actively participated in a number of speaking engagements throughout the year and representing CRDN at various events to help grow awareness, foster engagement, and shape conversations around the strength, needs, and opportunities within Canadia's rare disease community.

National

CRDN's manager attended the *inaugural RareKids-CAN conference* in Ottawa and the *Neuromuscular Diseases Network for Canada (NMD4C) annual scientific meeting* in Calgary.

Members of the leadership team contributed to events such as the *CORD conference* in Montreal, the *Canadian College of Medical Geneticists' (CCMG) annual scientific meeting* in St. John's, the *Pompe Empowerment workshop* in Vancouver, *RQMO's month of Zebruary program* (online), and *Rare Disease Day and World Birth Defects Day Symposium* in Calgary. Each event provided a platform to exchange knowledge, discuss challenges, and explore meaningful and synergistic collaborations.

Global

CRDN's participation extended beyond Canada, with representation at the *Sidra Medicine Precision Medicine and the Future of Genomics conference* in Doha and the *2025 International Genomics Education and Training Summit* in Greece to bring back valuable global perspectives and best practices to Canada.

CRDN also gained global visibility through notable features, including being 1 of 12 initiatives featured in the *Rare Diseases International (RDI) Mapping Rare Project*, mentions on the *International Rare Diseases Research Consortium (IRDiRC) website*, and inclusion in the *European Orpha News*.

Engaging in Discussions to Strengthen Rare Disease Care

CRDN has been actively contributing to important discussions around improving rare disease care and access to lifesaving therapies across Canada and remains committed to driving positive change at both the provincial and national levels.

Alberta Rare Disease Framework Discussions

For example, CRDN participated in a two-day workshop, hosted by *BioAlberta*, *the Pivot Group*, and *Innovative Medicines Canada*, aimed at assessing Alberta's rare disease landscape. The workshop brought together key stakeholders to develop recommendations for a more coordinated, patient-centered care system. The final recommendations from this workshop will be presented to the Alberta Ministry of Health, contributing to the development of a comprehensive rare disease framework.

Cell and Gene Therapies Discussions

At the national level, CRDN took part in roundtable discussions on cell and gene therapy for rare diseases, hosted by *Advancing Cell & Gene Therapy (ACGT)* – a group of leading pharmaceutical companies. These initial discussions emphasized the importance of ensuring patient voices are central as these innovative therapies evolve.

Fostering Global Alignment

Alongside *RareKids-CAN: Pediatric Rare Disease Clinical Trials and Treatment Network* and the *Maternal, Infant, Child and Youth Research Network (MICYRN)*, we are proud to co-lead the creation of a **Canadian National Mirror Group (NMG)** as part of the *European Rare Disease Research Alliance (ERDERA)*. The Canadian NMG will bring together representatives from federal government agencies, research funders and networks, academic institutions, and patient organizations to coordinate national efforts and fostering collaboration, both nationally across sectors and globally.

ERDERA
European Rare Diseases Research Alliance

A global initiative uniting 170 partners from 37 countries to accelerate research, improve diagnostics, and advance treatments for rare diseases

Over the past year, the Canadian NMG has convened twice to discuss its structure and function, engage with ERDERA activities, and amplifying opportunities for Canada's rare disease community. By participating in this global initiative, Canada will align its rare disease efforts with global strategies, strengthen its role in the global rare disease community, and gain access to global resources and partnerships that can accelerate breakthroughs in research and patient care.

RARE DISEASES INTERNATIONAL

A global alliance of patient-driven organizations to advocate for rare diseases as an international public health priority

The RDI-Lancet Commission on Rare Diseases has officially launched, co-chaired by CRDN's own Dr. Kym Boycott, to develop evidence-based policy solutions that enhance healthcare for Persons Living with a Rare Disease (PLWRD) worldwide.

The Rare Diseases International (RDI)-Lancet Commission on Rare Diseases is a new initiative dedicated to improving the lives of Persons Living with a Rare Disease (PLWRD) globally by generating evidence-informed recommendations that can be implemented in all countries.

LOOKING AHEAD

CRDN's journey has only just begun, but our first year has demonstrated the power of collaboration, shared vision, and collective action. As we move forward, we remain committed to advancing research and innovation for better health, strengthening partnerships, and ensuring that the voices of the rare disease community continue to shape our priorities.

Over the past year, CRDN has built strong connections with government agencies, research networks, and patient organizations to elevate rare disease as a national priority. We know that lasting impact comes from working together. In the year ahead, we will continue to foster and seek new partnerships, driving forward initiatives that enhance diagnostics, improve access to innovative therapies, and strengthen support for patients and families across Canada. By expanding our network and leveraging collective expertise, we aim to create meaningful change that benefits the entire rare disease community.

A key milestone in the coming year will be our inaugural Canadian Rare Disease Innovation Showcase. This event will highlight groundbreaking research and innovative approaches to care while fostering new connections across sectors. It will serve as a platform to inspire action, share knowledge, and strengthen the rare disease ecosystem in Canada.

As we look ahead, we are grateful for the support of our partners and the dedication of the rare disease community. Together, we are building a future where every person affected by a rare disease has access to the care, treatment, and resources they need. We invite you to join us in shaping this future — through collaboration, advocacy, and continued innovation.

OUR TEAM

Steering Committee

Francois Bernier
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Kim M Boycott
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Craig Campbell
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Jim Dowling
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Angela Genge
Montreal Neurological Institute – Hospital, McGill University

Lawrence Korngut
Hotchkiss Brain Institute, University of Calgary

Thierry Lacaze Masmonteil
University of Calgary; Maternal, Infant, Child and Youth Research Network (MICYRN)

Deborah Marshall
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Jacques L. Michaud
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Jonathan Pratt
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Ian Stedman
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Breanne Stewart
RareKids-CAN: Pediatric Rare Diseases Clinical Trial and Treatment Network

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Durhane Wong-Rieger
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Jodi Warman Chardon
Ottawa Hospital, University of Ottawa

OUR TEAM

Pillar 2 – Innovative Therapies

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Jim Dowling
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Thierry Lacaze-Masmonteil
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Breanne Stewart
RareKids-CAN: Pediatric Rare Diseases Clinical Trial and Treatment network

Risini Weeratna
National Research Council (NRC)

Durhane Wong-Rieger
Canadian Organization for Rare Disorders (CORD)

OUR TEAM

Pillar 3 – Care, Support, & Empowerment

Ian Stedman
York University

John Adams
Canadian PKU and Allied Disorders (CanPKU+)

Jillian Banfield
Canadian Institutes for Health Research – Institute of Genetics (CIHR IG)

Brad Crittenden
Canadian Association of Pompe

Deborah Marshall
University of Calgary

Homira Osman
Muscular Dystrophy Canada

Gail Ouellette
iRARE Centre, RQMO

Stephen Parrott
Kidney Cancer Canada Board

Jonathan Pratt
Regroupement Québécois des maladies orphelines (RQMO)

Nicola Worsfold
World Duchenne Organization

Help us build on our momentum by raising awareness and sharing knowledge to improve rare disease research and care.

Visit our website and sign-up for our newsletter

Join the conversation on social media!
#RareLivesSharedStrngth #Canada4Rare

SCAN ME