

RÉSEAU
CANADIEN
DES MALADIES
RARES



CANADIAN
RARE
DISEASE
NETWORK



RARE DISEASE DAY®

~ Rare Lives, Shared Strength ~

Francois Bernier, Kym Boycott, Leanne Ward, Thierry Lacaze-Masmonteil, Breanne Stewart, Ian Stedman, & Durhane Wong-Rieger on behalf of the CRDN Steering Committee

CORD Rare Disease Summit
February 28-29, 2024, Ottawa, ON





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What are your hopes for the future?

Waiting for responses ...

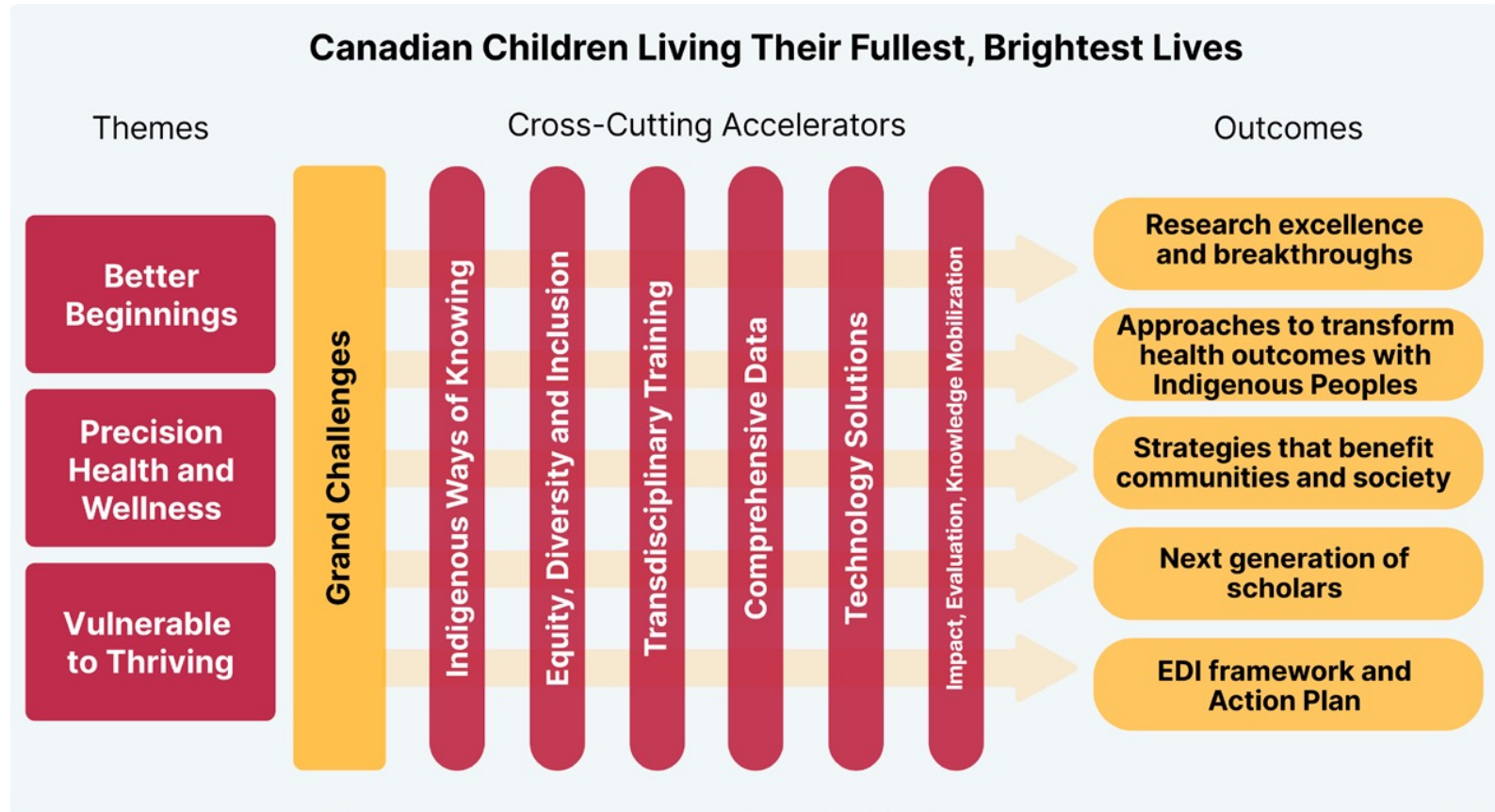


Acknowledgements

one child 
every child



A Canada-first research initiative, with a vision for all children to be healthy, empowered, and thriving.





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



Benefits



Coordination & Collaboration

Ensuring efforts are aligned and complementary rather than fragmented and possibly duplicated, thus empowering the rare disease community.

Resource Sharing

Efficient sharing of resources, such as expertise, best practices, and funding that can enhance learning and save costs.

Increased Impact

Greater visibility of individual networks and initiatives as well as coordinating and amplifying collective efforts to achieve larger-scale, cross-sectoral outcomes.

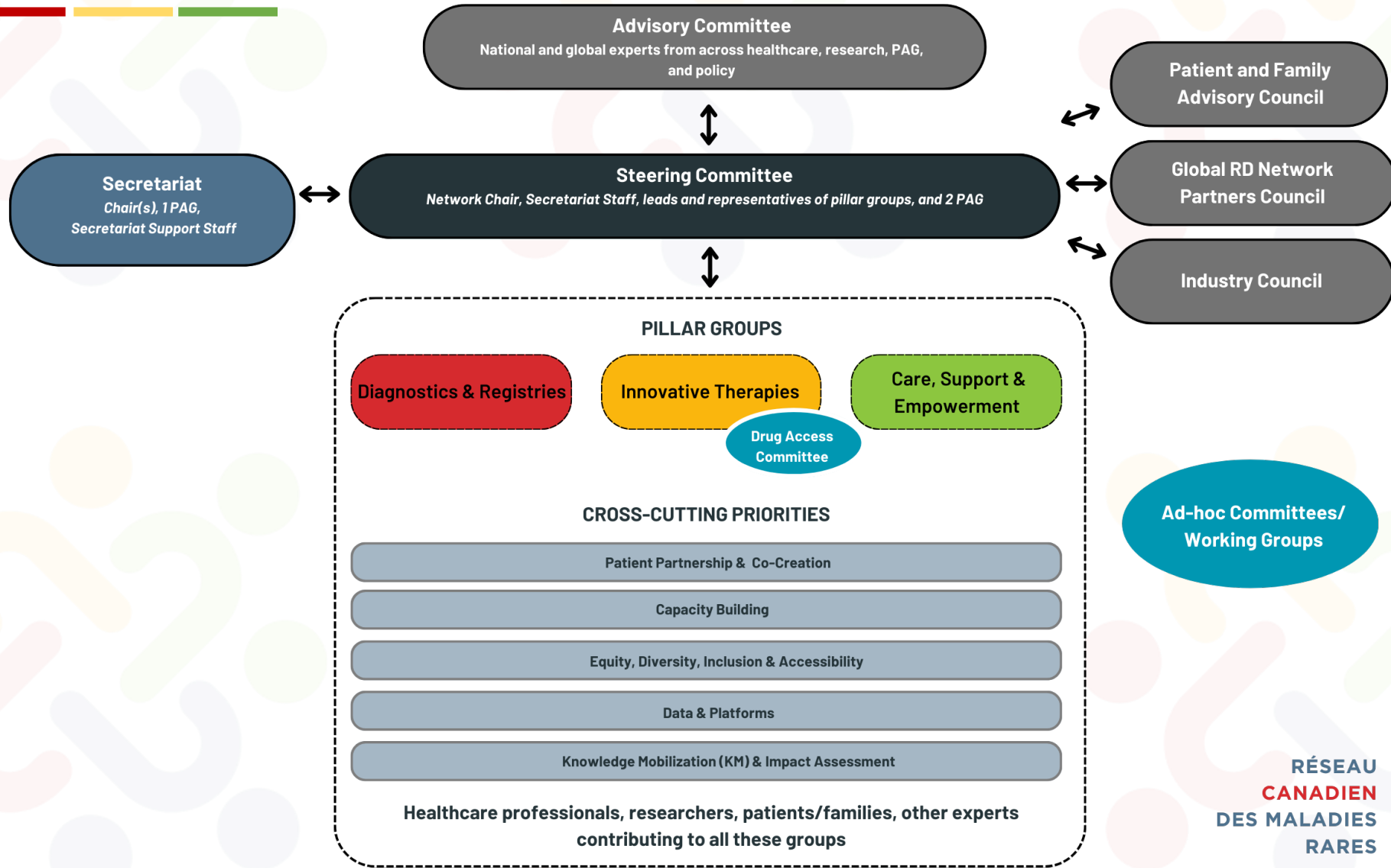
Capacity Building

Opportunities for capacity building and professional development, benefitting all members.

Global Engagement

Support international engagement and partnerships, fostering collaboration and knowledge-sharing for research, innovation, care, and advocacy.

Governance



Steering Committee Members



Francois Bernier, MD
Alberta Children's Hospital
Research Institute
(ACHRI),
University of Calgary



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



Jim Dowling, MD, PhD
Sick Kids Hospital,
University of Toronto



Kim M Boycott, MD, PhD
Children's Hospital of
Eastern Ontario (CHEO)
Research Institute,
University of Ottawa



Jacques L. Michaud
Centre de recherche du
CHU Sainte Justine,
Université de Montréal



Gail Ouellette, PhD
iRARE Centre, RQMO



Lawrence Korngut, MD
Hotchkiss Brain Institute,
University of Calgary



Angela Genge, MD
Montreal Neurological
Institute – Hospital,
McGill University



Jonathan Pratt
Regroupement Québécois
des maladies orphelines
(RQMO)



Craig Campbell
Children's Hospital LHSC,
Western University



Leanne Ward, MD
Children's Hospital of
Eastern Ontario (CHEO)
Research Institute,
University of Ottawa



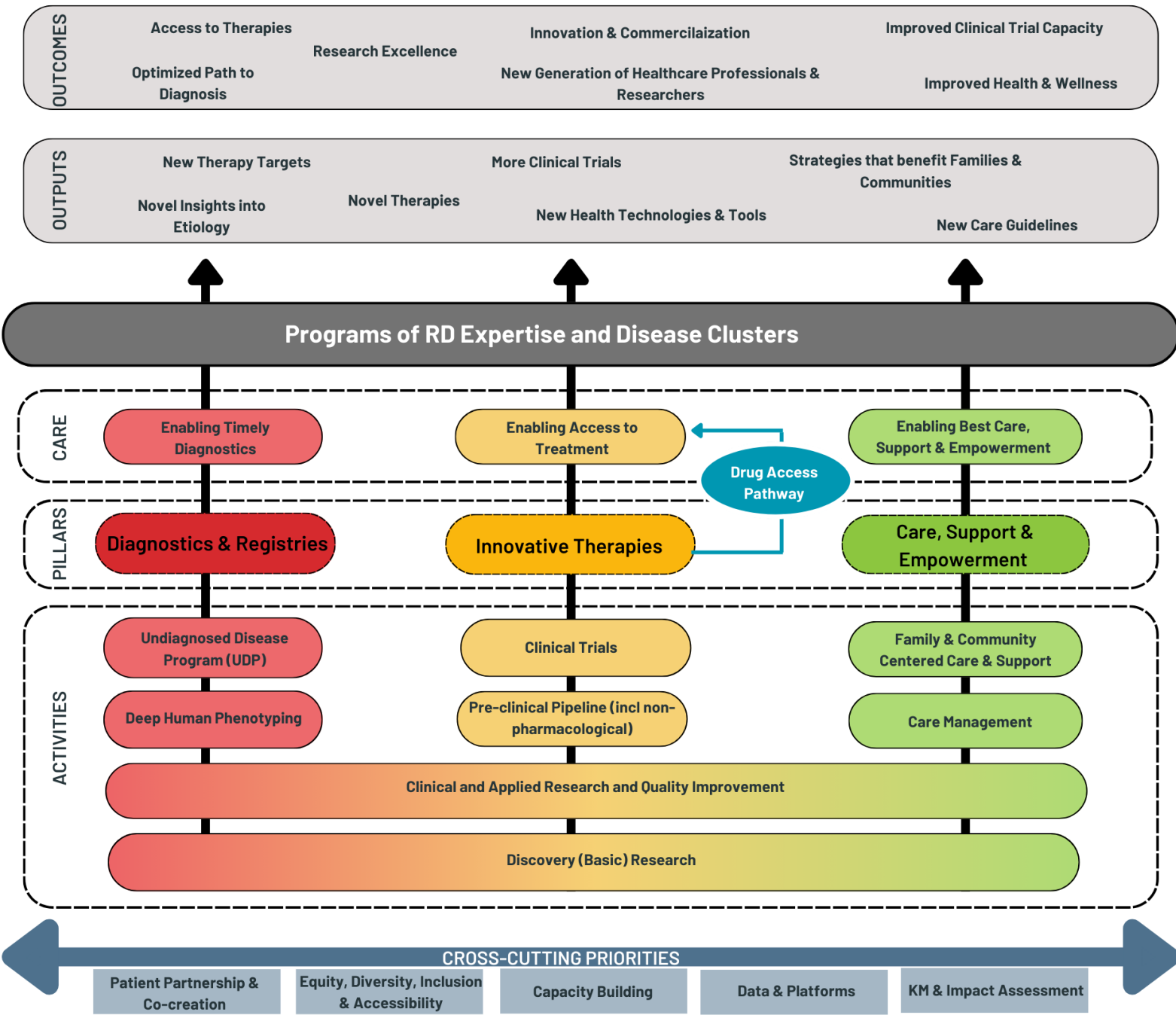
Ian Stedman, PhD
York University



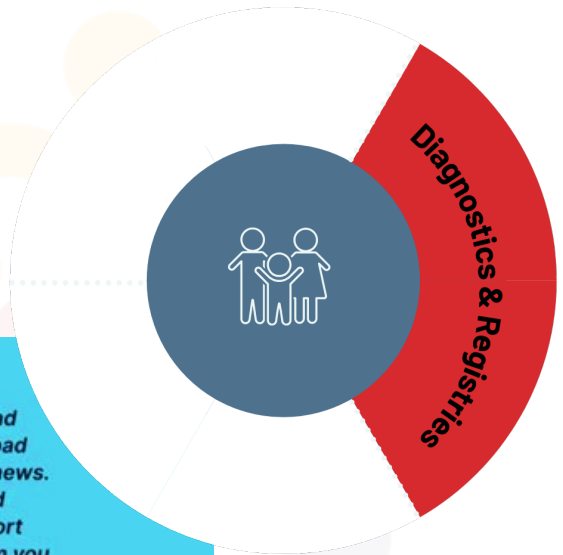
**Thierry Lacaze-
Masmonteil, MD, PhD**
University of Calgary;
Maternal, Infant, Child
and Youth Research
Network (MICYRN)



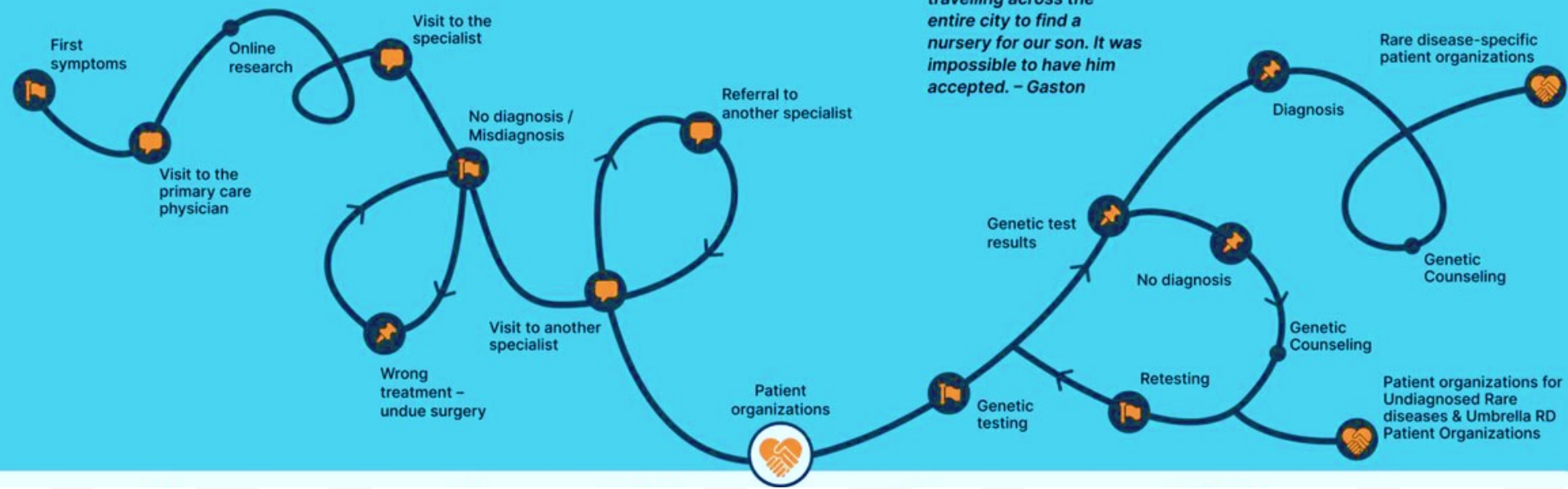
Deborah Marshal, PhD
Alberta Children's Hospital
Research Institute (ACHRI),
University of Calgary



Pillar 1



Patient Journey through diagnosis



“ It’s a waiting game, but you tell a mum to wait when she’s waited 15 years. It’s difficult. – Nuria

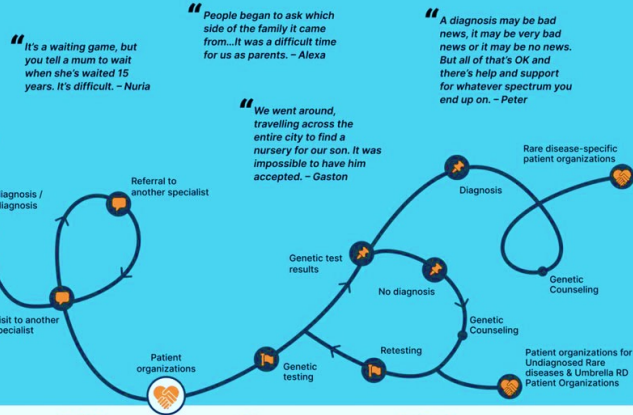
“ People began to ask which side of the family it came from...It was a difficult time for us as parents. – Alexa

“ We went around, travelling across the entire city to find a nursery for our son. It was impossible to have him accepted. – Gaston

“ A diagnosis may be bad news, it may be very bad news or it may be no news. But all of that’s OK and there’s help and support for whatever spectrum you end up on. – Peter

Pillar 1

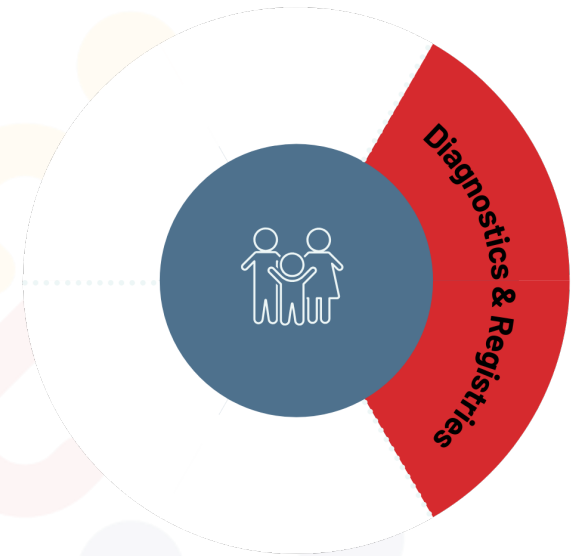
Patient Journey through diagnosis



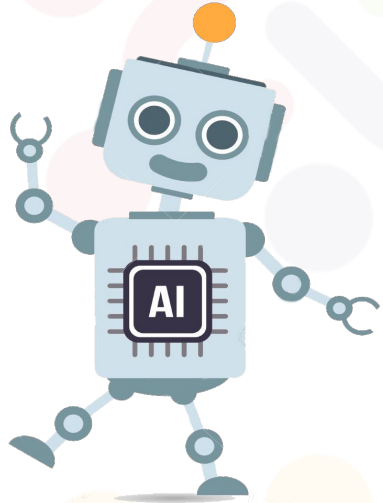
Facilitate timely diagnosis and optimal delivery

Understand the pathogenesis of undiagnosed RDs

In-depth understanding of all RDs



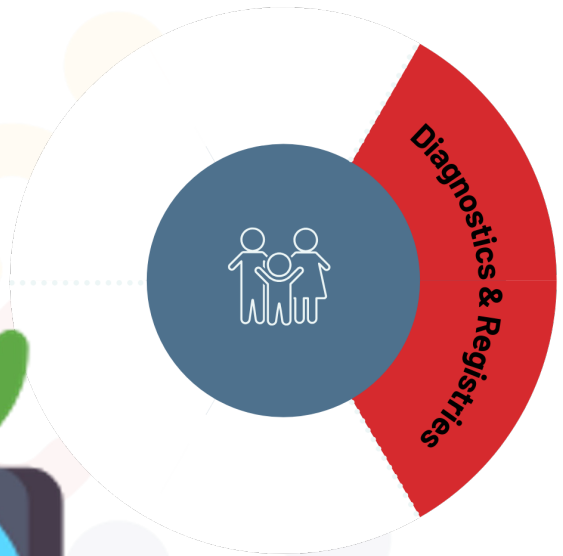
Pillar 1



Undiagnosed Disease Program

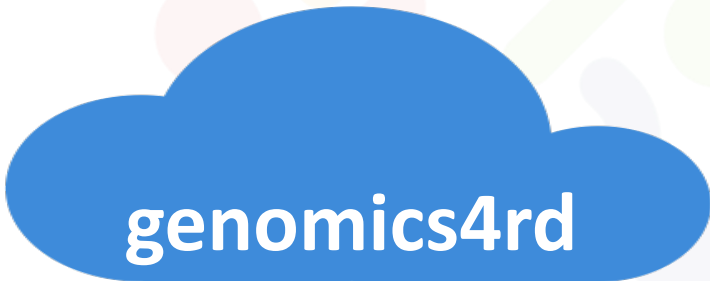


In-depth Disease Characterization

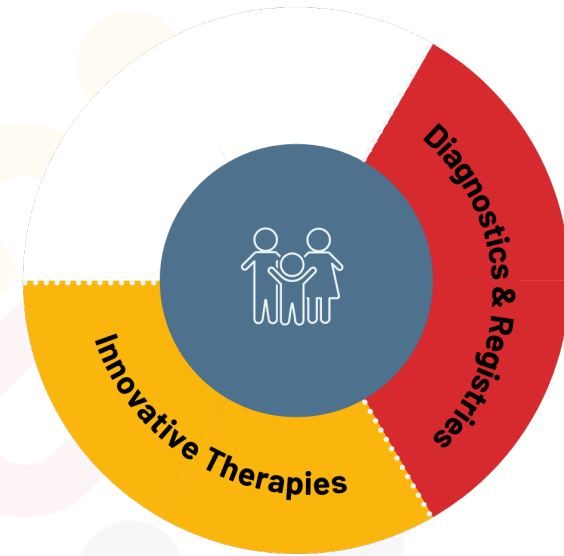
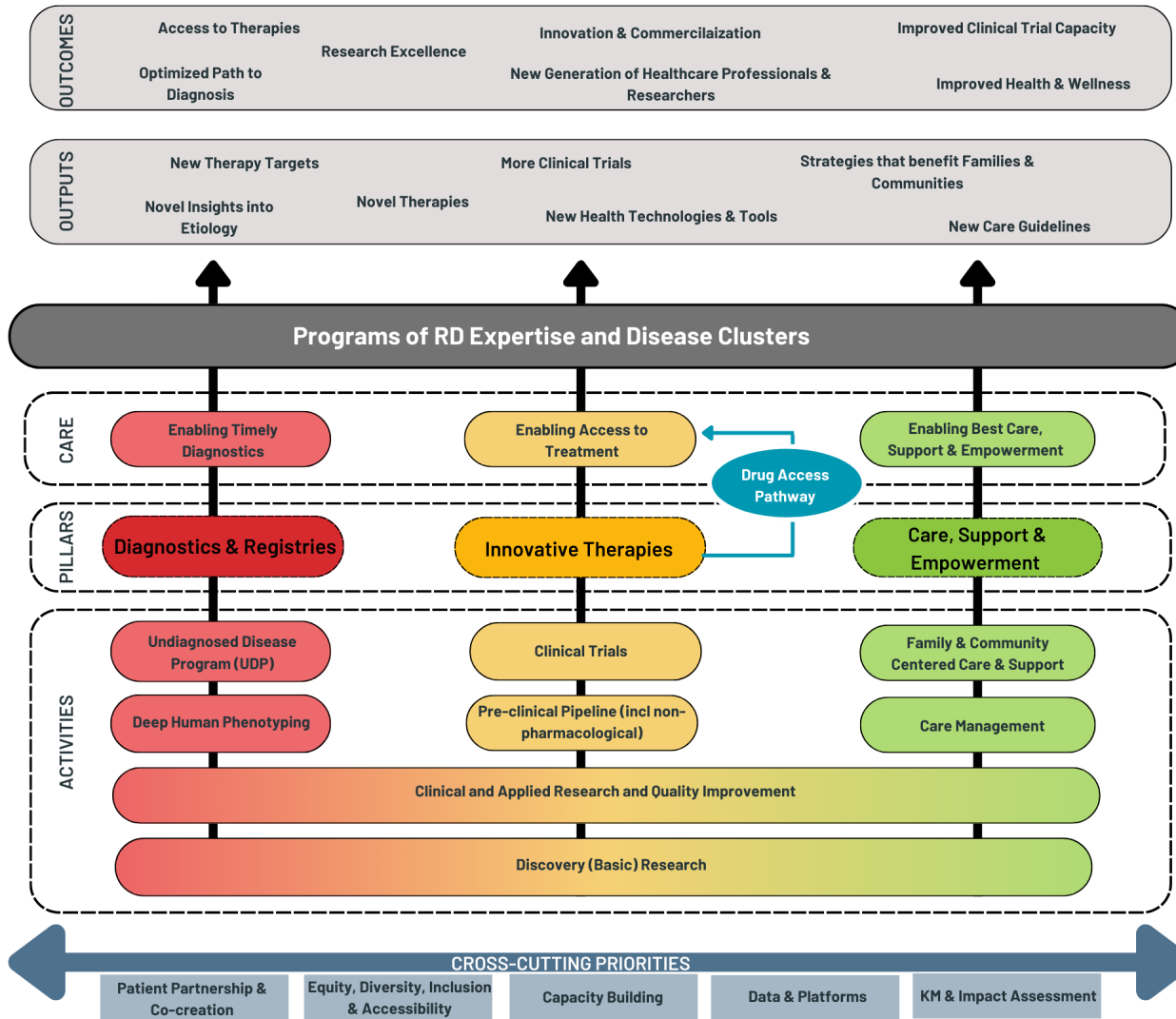


Pan-Canadian Genome Library

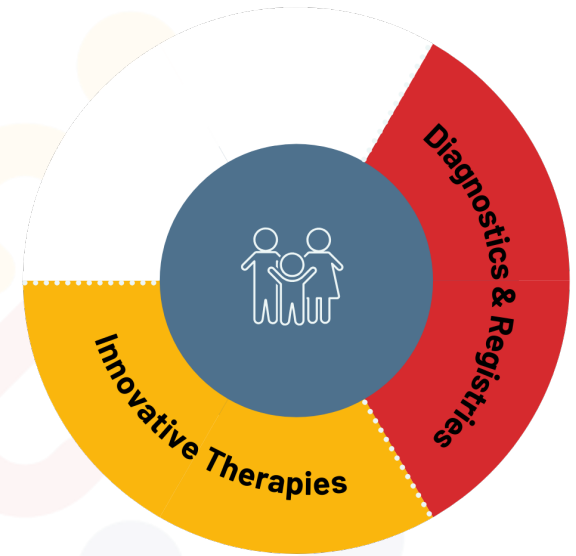
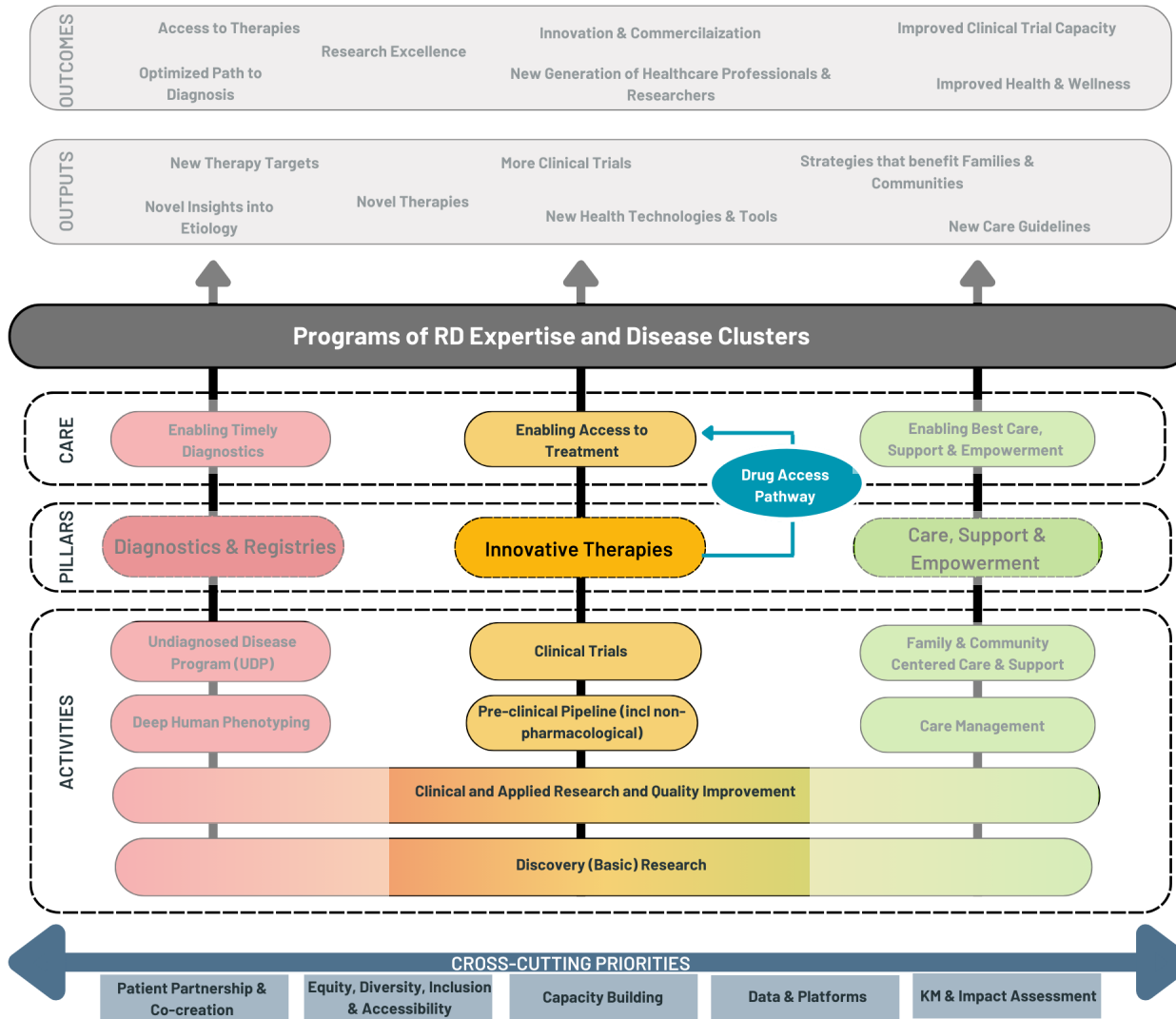
Registries

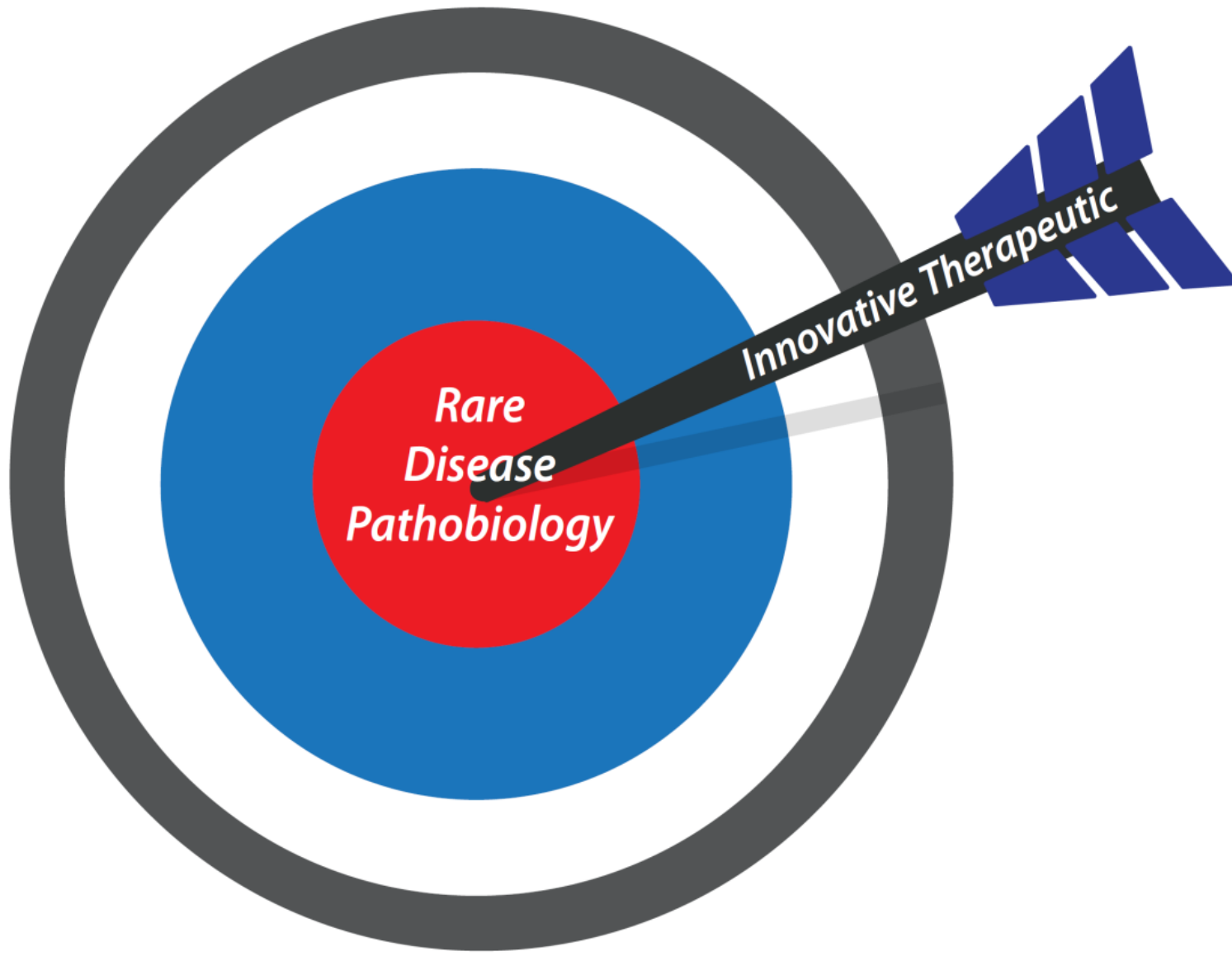


Pillar 2




Pillar 2






Effective, Innovative Therapies



Treatment



Control



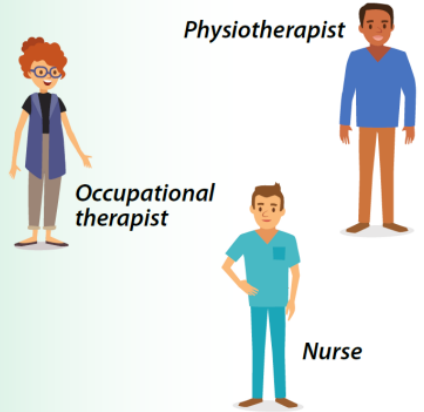
Pre-Clinical Pipeline




Clinical Trials



Drugs + Therapeutics Access Pathway

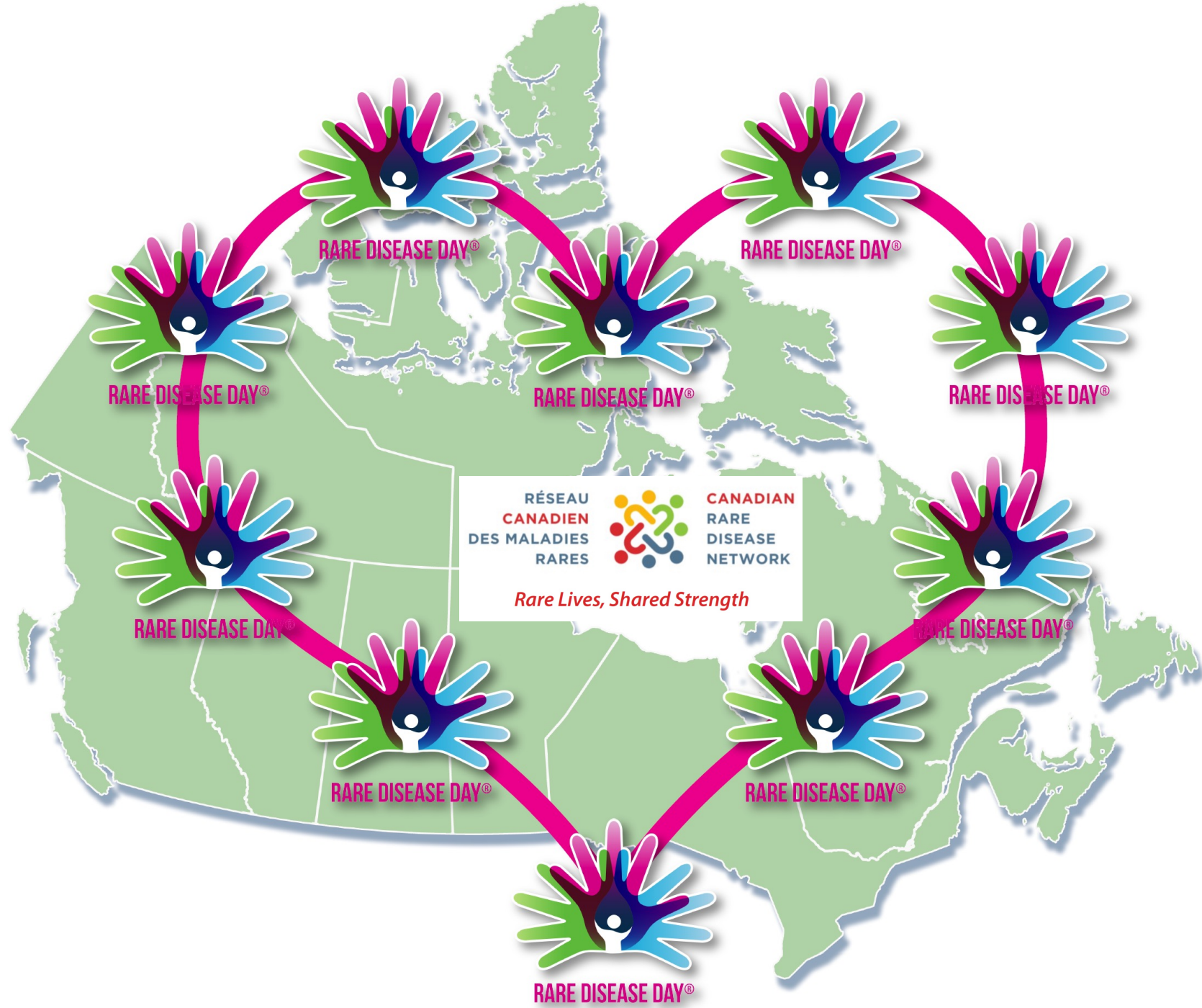


RARE DISEASE CLINIC



Drugs + Therapeutics Delivery

4108



RareKids-CAN

**Pediatric Rare Disease
Clinical Trials and
Treatment Network**

RareKids-CAN



- **Funding Opportunity:** CIHR Team Grant: National Pediatric Rare Disease Clinical Trials and Treatment Network 2023-10-24
- **Sponsor:** The CIHR Rare Disease Research Initiative, under the scientific leadership of the CIHR Institute of Genetics, and in partnership with the Government of Canada's National Strategy for Drugs for Rare Diseases
- **Amount:** \$19,999,845
- **Date:** January 2024 - December 2028
- **Administering Institution:** Maternal Infant Child and Youth Research Network (MICYRN)
- **Nominated Principal Applicant:** Thierry Lacaze-Masmonteil

Objectives RareKids-CAN

| | |
|-----------------------|--|
| Infrastructure | Develop a platform to support pediatric rare disease clinical trials (PRDCT) in Canada |
| Training | Increase the capacity to perform PRDCTs in Canada by offering training and mentorship opportunities |
| Attraction | Attract international PRDCTs, increasing investments in Canada and providing earlier access to innovative therapies to people living in Canada |
| Inclusivity | Foster diversity and inclusion in and improve access to PRDCTs |
| Data | Implement processes to acquire data from clinical trials, real world registries, health economics evaluation, and post-marketing surveillance to inform assessment reviews throughout the whole drug/therapy cycle |
| Authorization | Increase the number of new rare disease drug submissions for authorization of commercialization to Health Canada |

Current Composition of Network (and Growing!)



Scope



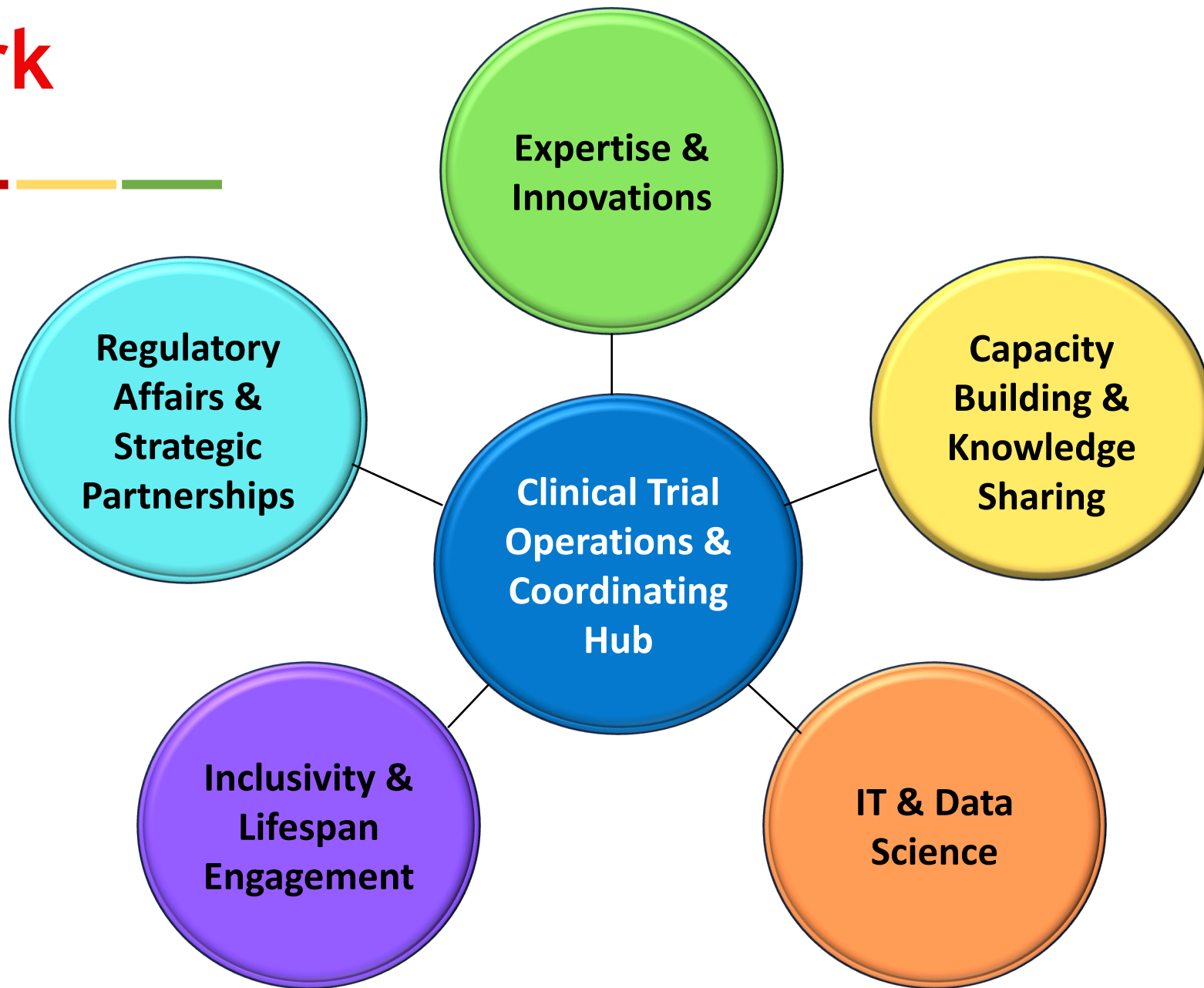
Pediatrics

Investigator Initiated (Academic)

Industry and Biotech

Primarily Non-Oncology

Network



Clinical Trial Operations and Coordinating Hub (CTOCH)



Central Intake

Expansion of ARO Services:

- Consultation
- Regulatory submissions
- Ethics navigator
- Contracts facilitation
- Quality assurance and monitoring
- Standing Data Safety Monitoring Board (DSMB)

Clinical Trial Navigators- 17 sites across Canada (Local CTU Point of Contact: Site identification, Feasibility, Readiness)

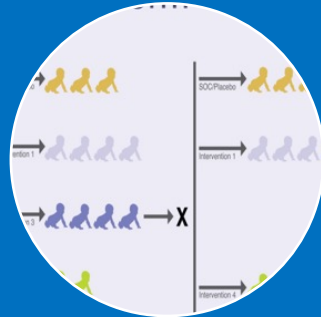
Guidance Documents and Shared SOPs: Advanced Therapeutic Products (modified cell, stem cell, gene therapies)

Clinical Trial Sponsorship

Expertise and Innovations Platform



Statistics



Methods



Pharmacology



Capacity Building & Knowledge Sharing Platform



Training and Mentorship

Knowledge Synthesis & Mobilization

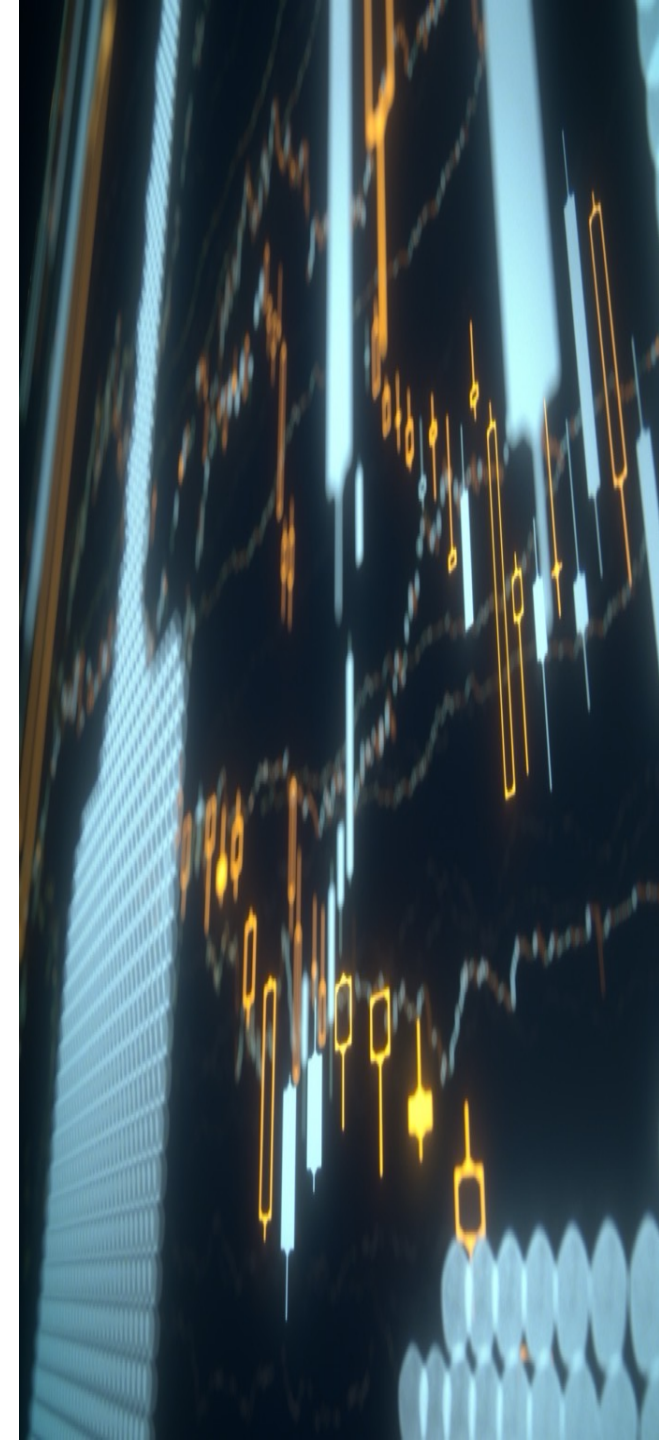
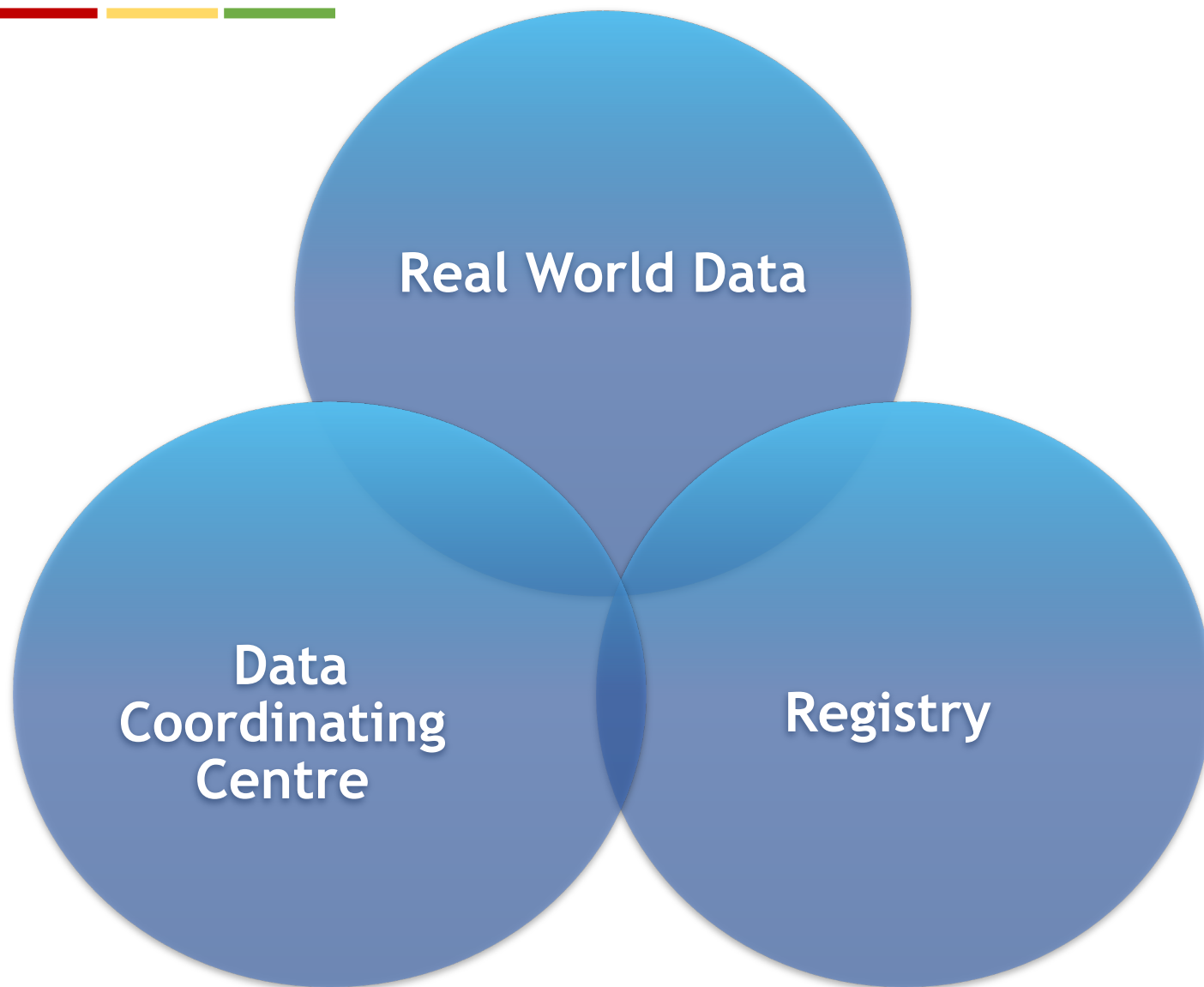


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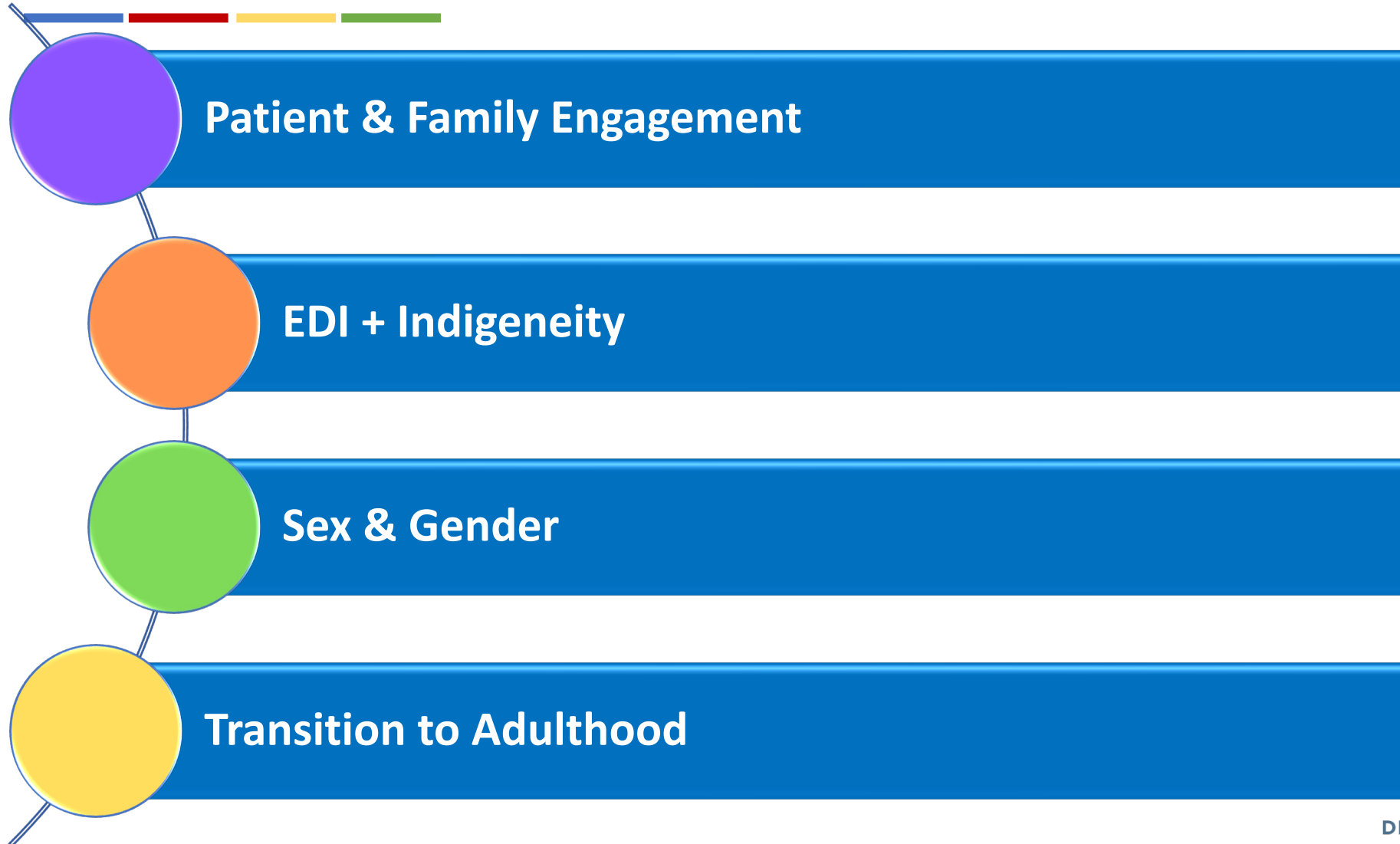


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IT & Data Science Platform



Inclusivity & Lifespan Engagement Platform



Regulatory Affairs & Strategic Partnerships Platform



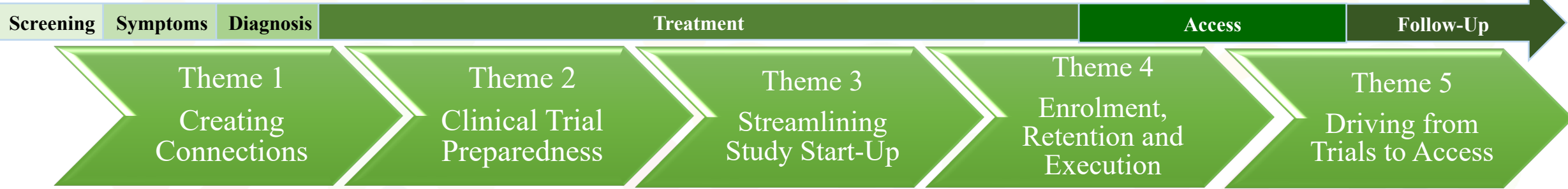
Health Canada Relations & Advocacy

Health Economics, HTA, & Reimbursement

Sustainability & Business Development



Patient Journey



Clinical Trial Operations and Coordinating Hub (CTOCH) & Cross-Cutting Platforms

Expertise & Innovation

- Design & Methods
- Statistical Methods
- Pharmacology

Capacity Building & Knowledge Sharing

- Training & Mentorship
- Knowledge Synthesis & Mobilization

IT & Data Science

- Data Coordinating Centre
- Real World Data & Evidence
- Registry

Inclusivity & Lifespan Engagement

- Pt & Family Engagement
- EDI+I
- Sex & Gender
- Transition to Adulthood

Regulatory Affairs & Strategic Partnerships

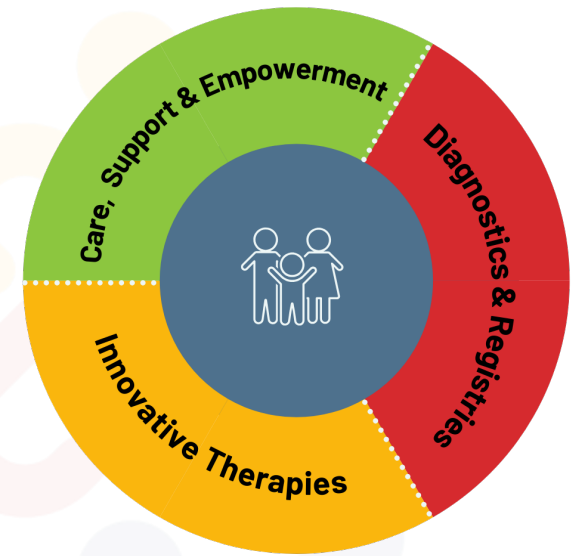
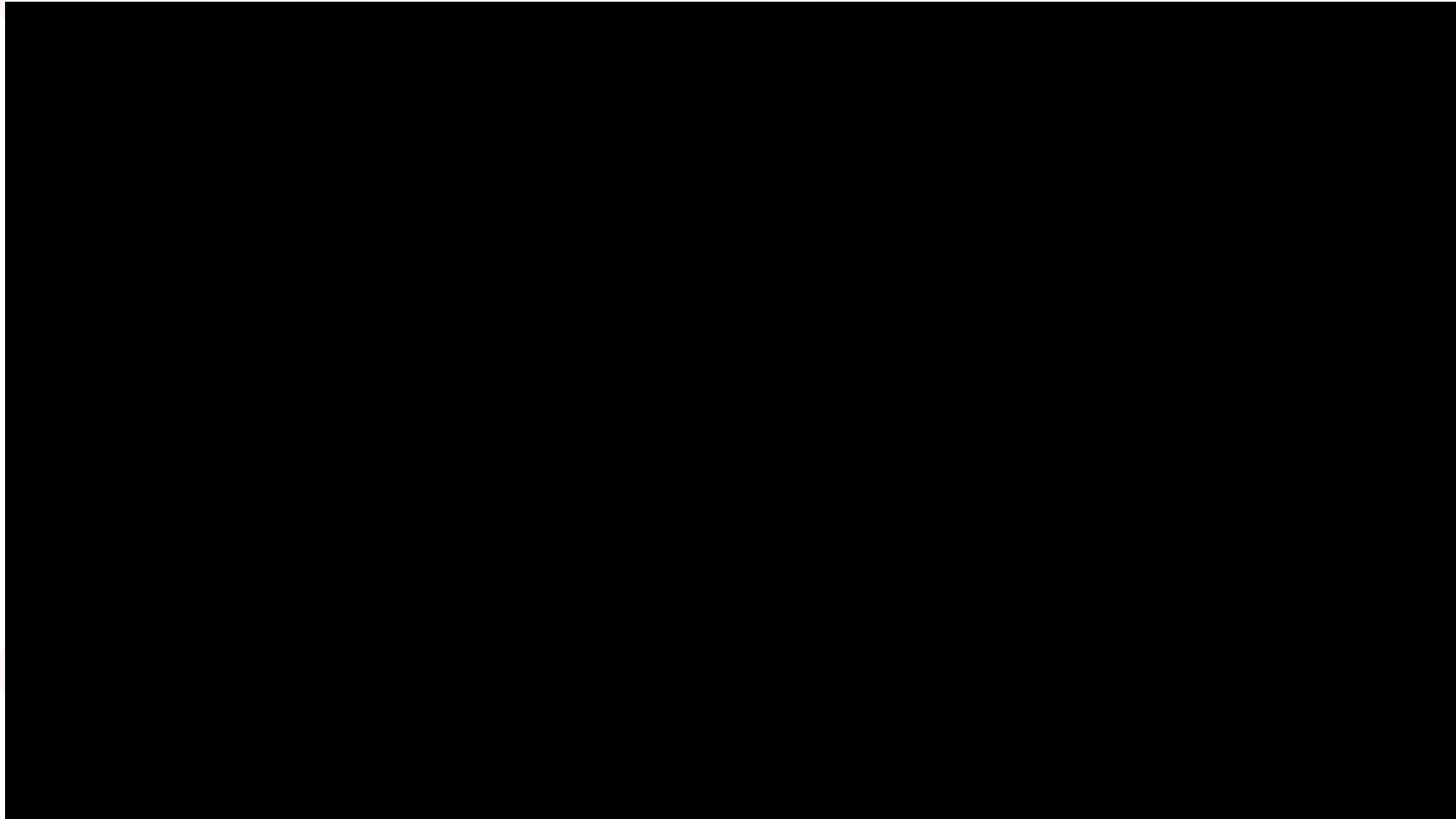
- Health Canada Relations & Advocacy
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Contact



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breanne.stewart@micyrn.ca

Pillar 3



Follow Sylvie on Instagram: [@silverrlinings63](https://www.instagram.com/silverrlinings63)

Co-Creation and Partnerships



Questions?

Join us!



#CRDN

#Canada4Rare

#RareLivesSharedStrength

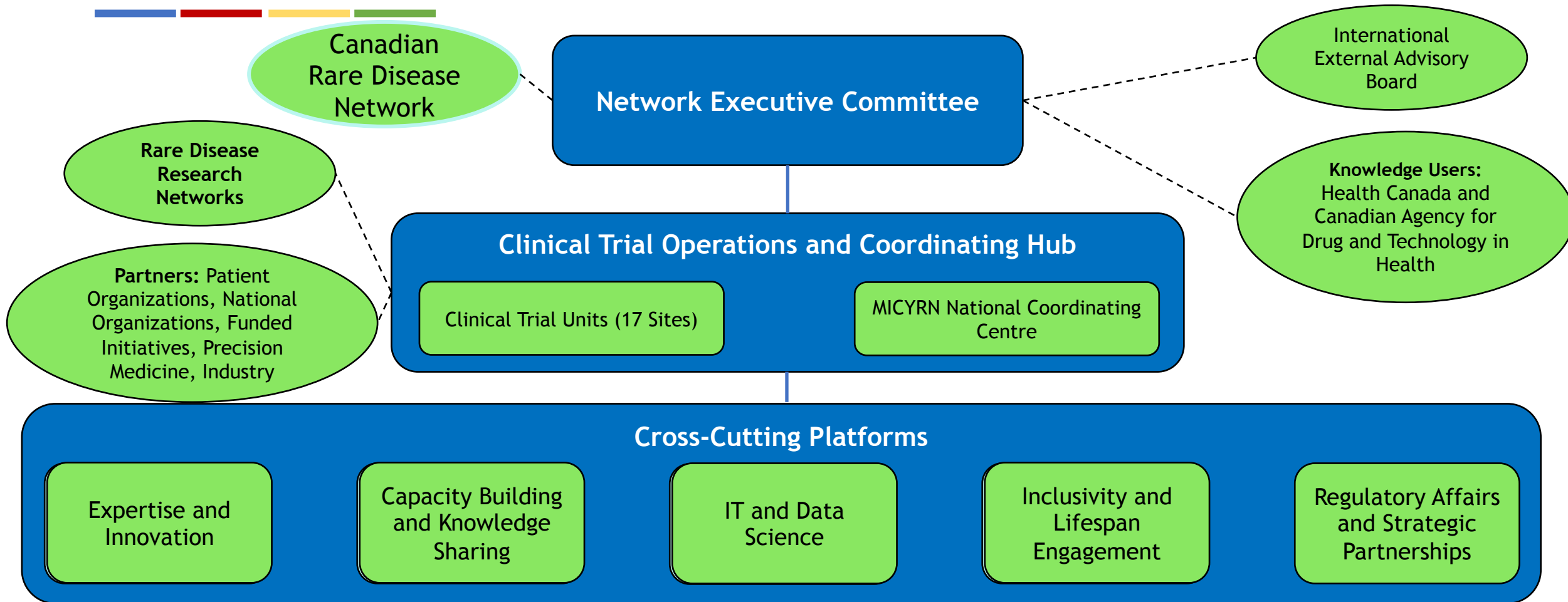
#RareDiseaseDay



Back-up slides



RareKids-CAN Governance



Rare Lives, Shared Strength

