DAY 1: DELIVERING RARE ALLIANCE CANADA

Objectives:
Mobilize all stakeholders around common vision for Rare Alliance Canada
Develop consensus on a “best-fit” model for Canada’s rare disease network
Create a guiding framework to map current resources, define gaps, identify opportunities and challenges, and prioritize needs
Articulate next steps; gain commitment to action; define success factors, and outcome measures

What is Rare Alliance Canada?
We are patient and family driven
We bring together all stakeholders, all disciplines, all activities, all services
We work toward rare disease sites linked from coast to coast
We envision centres of excellence empowering GPs, therapists, teachers, and support workers to effective serve rare disease patients and families in their own community
We will enhance Canada’s reputation as a leader in rare diseases

DAY 2: DELIVERING THERAPIES FOR LIFE

Objectives:
Share vision of possibilities for health with innovative therapies of today and tomorrow
Challenge all stakeholders to identify and “go beyond” barriers to providing access to old and new therapies for life
Develop consensus on a “best-fit” framework for “life-long” access to innovative therapies
Articulate next steps; gain commitment to action; define success factors, and outcome measures

What are Principles of Canada’s Therapies for Life Program?

Principles: patient-driven, comprehensive, responsive, responsible access to therapies for unmet needs
Innovative: Canada is favourable environment for R&D, clinical trials, timely access, patient support
Patient-driven: Patients at all levels from policy, program development, criteria for specific drug access, patient participation in registration, monitoring, and data collection, reassessment of drug place in therapy
Priced Right: Canadian pricing and financing (non-excessive for payers, incentive for further and affordable access for patients)
## DAY 1: DELIVERING RARE ALLIANCE CANADA

### Session 1: Approaches to Developing Rare Disease Networks

**Panel:** 5-min overview of origin and vision (why and so what)
- Vancouver Vision: Opportunities and Challenges in Canadian Landscape (Durhane)
- Children’s National Rare Disease Institute (Children’s National Health System and NORD)
- Ontario Rare Disease Framework (Ronald Cohn, The Hospital for Sick Children)
- European Reference Networks (Louise Clément, Health Standards Organization)

**Panel discussion on strengths, risks, and outcome**
- What is the intended impact?
- What will it take to make it work?
- What are indicators working or not working?

**Audience discussion on relevance for Canada**
- What are the relative merits of each model, and how what do these suggest for “Rare Alliance Canada?”
- What would a “best of” look like for Canada?

**Session Chair:** Durhane Wong-Rieger, CORD

### Session 2: Rare Gems in Canada’s Research Landscape

**Panel 1:** Research and clinical expertise (30 min)
- CIHR RD Emerging Team Grants and Contributions to Canadian Excellence
- Autosomal Recessive Spastic Ataxia of Charlevoix-Saguenay (Bernard Brais, McGill University)
- Chronic Childhood Vasculitis: Characterizing the Individual Rare Diseases to Improve Patient Outcomes (David Cabral, University of British Columbia / Susa Benseler, Alberta Children's Hospital)
- The FACTs Project: Fabry Disease Clinical Research and Therapeutics (Jeffery Medlin, University Health Network / Tony Rupar, Children’s Health Research Institute)
- Hereditary Spastic Paraplegia (Guy Rouleau, Centre hospitalier de l'Université de Montréal (CHUM))

**Panel 2:** Research Improving Care and Treatment (30 min)
- Improving diagnosis (NBS, genetics and genomics)
- RD-Connect / PhenomeCentral / MatchMaker Exchange (Michael Brudno, University of Toronto/ Orion Buske, University of Toronto)
- Diagnosis to treatments
- CHEO (Kym Boycott)
- Scleroderma Patient-centered Intervention Network (Brett Thombs, Jewish General Hospital)

**Session Chairs:** Alex MacKenzie (CHEO) & Etienne Richer (CIHR)
### Session 3: Rare Disease Research and Clinical Centres of Excellence/Networks

**Patients (TBD)**

**Panel 1: Disease Specific Networks**
- Cystic Fibrosis
- Hemophilia
- Sickle Cell Disease
- Dravet Syndrome
- Rhett Syndrome

**Panel 2: RD Clusters**
- Neuromuscular
- Metabolic networks
- Rare Cancers

**Panel 3: Multiple disease networks**
- MYCRN (Anne Junker, BC Children’s Hospital)
- Genomics (Monica Justice, The Hospital for Sick Children)
- Community-based RD Networks (Shikha Mittoo, Mount Sinai Hospital)

**Session 4: Designing Rare Alliance Canada**

**Panel: Key elements**
- Essential features
- Criteria, quality, standards, guidelines
- Outreach, education, training
- Design work (small group)

**Plenary: Coming Together for Next Steps**

### DAY 2: DELIVERING THERAPIES FOR LIFE

**Session 1: How Patients Drive Access**
- Parent and Patient Advocacy (Cystinosis)
- Engaging with the System (SMA)
- Driving the System (Pat Furlong, Parent Project Muscular Dystrophy)

**Session 2: Innovative Therapies Demand Innovative Access Solutions**

**Panel 1: What are the BIG innovations in therapies?**
- Drugs for rare diseases
- Cellular therapies: gene and stem cell replacement
- Biologics and biosimilars

**Panel 2: How is “usual approach” blocking access?**
- Patient perspectives
- Clinician perspectives
- Private payer perspectives
- Public payer perspectives
- Industry perspectives

**Discussion: What does success look like?**
- What if we didn’t focus just on the drug but all of the healthcare services?
- What if we really replaced “cost” with “value” in all of our therapeutic assessments?
- What if patients really did have the last word in deciding access?

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<tr>
<th>Session 3</th>
<th><strong>Initiatives to Address Unmet Need</strong></th>
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<td>Panel: Patients, Health Canada, PMPRB, CADTH, IMC, BIOTECanada, pCPA, Provincial Drug Programs, Private Insurers</td>
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<td></td>
<td>Critique of Models</td>
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<td>- EMA: ADAPT SMART: Overall objective of an adaptive pathway is to agree the optimal strategy for the regulatory and HTA/payer evidence requirements to be met in the most efficient and effective way possible to facilitate development of and access to new medicines in high unmet need.</td>
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<td>- USA: Early Access Programs, Right to Try</td>
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<td>- Global: Compassionate Use, Global Access Programs</td>
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**Realizing Canada’s Life-Cycle Approach**
- Regulatory: Vanessa’s Law and Orphan Drug Regulatory Framework/Roadmap
- Pricing: PMPRB Amendments to Pharmaceutical Drug Regulations
- HTA: CADTH “early submission” and early advice (parallel processing)
- pCPA: Negotiated access (including price/access conditions)
- Provincial Managed Access Programs (including cancer drugs)
- Expensive Drugs for Rare Diseases Program (pilot initiatives)

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<th>Session 4</th>
<th><strong>Making “Therapies for Life” a reality</strong></th>
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<td>Working Groups: How could Life-Cycle Approach work with various therapies in various settings?</td>
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<tr>
<th>Wrap Up</th>
<th><strong>Plenary Discussion: Next Steps for Canada</strong></th>
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**Session Chair:** TBD