RARE DISEASES INTERNATIONAL | CORD Canadian Organization for Rare Disorders

# A Rare International Dialogue – AGENDA

**May 10 – 12, 2019**

Sheraton Centre Toronto Hotel
123 Queen Street West | Toronto, ON

1st Time in North America!

Three days of dialogue and deliberation on all things rare: from research and policy to action and better outcomes

The largest and most significant gathering of rare disease advocates from patients to policy makers, from gene manipulators to AI gurus, from local support groups to international research networks coming together to Create a Vision for Rare Disease in the 21st Century and Develop Action Plans from Local to Global

Hosted by Rare Disease International & Canadian Organization for Rare Disorders (CORD)

## Friday, May 10, 2019: Vision for Rare Disease in 21st Century

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| **8:00 a.m. – 8:30 a.m.** | **Registration and Breakfast: Grand Ballroom** |
| **8:30 a.m. – 10:15 a.m.** | **Welcome to Rare Disease in the 21st Century**<br>Welcome: Why Rare Disease Matters<br>• Durhane Wong-Rieger, Chair, Rare Disease International<br>• Maureen Smith, Secretary, Canadian Organization for Rare Disorders<br>• Wayne Critchley, Chair, Canadian Organization for Rare Disorders<br>• Honourable Ginette Petitpas Taylor, Minister of Health (Canada)<br>• Honourable Navdeep Bains, Minister of Innovation, Science and Economic Development Canada (TBC)<br>• Christine Elliot, Ontario, Minister of Health (TBC)<br>• Senator Mary Harney, Former Minister of Health (Ireland)<br>• Peter Goodhand, CEO, Ontario Institute for Cancer Research, Board Member, Global Alliance for Genomics & Health (5 min)<br>• Chris McMaster, Scientific Director, CIHR Institute of Genetics<br>Advances in Rare Disease<br>Facilitator: Bill Dempster, 3Sixty Public Affairs<br>• David Malkin, The Hospital for Sick Children<br>• Gareth Baynam, Western Australia Health Department (Australia)<br>• Eileen Treacy, National Rare Diseases (Ireland)<br>• Hartman Wellhoefer, Takeda Pharmaceuticals |
| **10:15 a.m. – 10:45 a.m.** | **Plenary: How Advocates Transformed the Rare Disease Landscape**<br>Introduction: Transformative Role of Advocacy in Advancing Rare Disease - Bruce Bert, Pfizer<br>**1. Celebration of Rare Disease Heroes (and their impact)**<br>• Yann LeCam, Europe |

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| | • Marlene Haffner, USA<br>• Min-Chieh Tseng, Taiwan<br>• Anders Olauson, Sweden |
| **10:45 a.m. – 11:15 a.m.** | **Networking Break** |
| **11:15 a.m. – 12:30 p.m.** | **2. How Rare Disease Helped Transform the Healthcare Landscape**<br>Facilitator: Cate McCready, BIOTECanada<br>• Diagnostic Breakthroughs: From Newborn Screening to Next Gen Sequencing - Kym Boycott, CHEO<br>• Transforming Rare Disease Research through Transnational Funding - Daria Julkowska, E-Rare Net<br>• Using Genomics to Match Rare Disease Patients to Therapies - Jan Friedman, University of British Columbia<br>• Applying Digital Technologies to Rare Disease Diagnosis, Care, Treatment, and Cure - Michael Liebman, IPQ Analytics, LLC<br>• Patient Communities: How AI is Transforming the Patient Experience - Lynda Brown-Ganzert, Curatio |
| **12:30 p.m. – 1:30 p.m.** | **Lunch** |
| **1:30 p.m. – 2:00 p.m.** | **Voice of the Patients**<br>Introduction: Gareth Baynam, University of Western Australia<br>• Tori Lacey, Canada<br>• Sherry Caldwell, Canada<br>• Eda Selebatso, Botswana<br>• Migdalia Denis, Latin America |
| **2:00 p.m. – 4:00 p.m.** | **Patient-Focused Drug Development - *Joint afternoon session with RE(ACT)***<br>Introduction: Olivier Menzel, Blackswan Foundation |

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| | **Researchers and Regulators**<br>• Marlene Haffner, USA<br>• Nick Sireau, UK<br>• Larissa Lapteva, USA<br>• Calvin Ho, USA<br>• Soo-Kyung Lee, USA<br>**Patients, Parents, and Patient Groups**<br>Facilitator: Pamela Graves-Moore, Sanofi Genzyme<br>• Tracy Kirby, Batten Disease Support and Research Foundation<br>• John-Peter Bradford, Life-Savings Therapies Network<br>• Tanya Collin-Histed, International Gaucher Alliance<br>• Christina Mutena, Rare Disease Kenya<br>• Tatiana Kulesha, Ukrainian Association of Pulmonary Hypertension |

**Please note:** Events below are separate ticketed events, which are **NOT** included with the conference registration fee.

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| **6:00 p.m.** | **Wine Reception (outside Grand Ballroom)** |
| **7:00 p.m.** | **Rare Disease Awards Gala Dinner Celebration: Grand West Ballroom** |

## Saturday, May 11, 2019: Educational Forum

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| **8:00 a.m. – 8:30 a.m.** | **Continental Breakfast: Grand East Ballroom** |
| **8:30 a.m. – 10:15 a.m.** | **Opening Plenary: Embracing New Opportunities and Challenges for Rare Disease in 21st Century**<br>Success Stories: Patients, Treaters, Researchers, Policy Makers<br>1. Patients: Dorica Dan, Romania, K.P. Tsang, Hong Kong<br>2. Alliances: Gail Ouelette, Regroupement Québécois des Maladies Orphelines<br>3. Industry: Donatello Crocetta, Takeda<br>Panel Discussion: Building on Success and Tending to Unintended Consequences<br>Facilitator: Jane Farhnam, CORD<br>1. Trends in Rare Disease Treatment - Murray Aitken, IQVIA<br>2. Rare Disease by the Numbers - Debbie Lambert, Orphanet<br>3. Researcher/Developer - Michael Hayden, BC Children's Hospital<br>4. Rare Disease Treatment Access - Judith Glennie, PRISM |
| **10:15 a.m. – 10:30 a.m.** | **Refreshment Break (participants move into dialogue streams)** |
| | **Three Parallel Dialogue Streams (Overview)**<br>**STREAM ONE** (Grand East Ballroom)<br>**Translating Research into Care and Treatment**<br>• Ending the Diagnostic Odyssey: Genomics, AI, Databases<br>• Tools and Support for Patient and Parent Engagement<br>• Empowering the Whole Person<br>**STREAM TWO** (Grand Centre Ballroom)<br>**Innovations in Therapy: Treating More and Curing Some** |

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| | • Curative Therapies: Next Step for Rare Diseases?<br>• Strategies for Access to Innovative Therapies<br>• Drivers of Drug Development<br>**STREAM THREE** (Grand West Ballroom)<br>**Addressing Issues of Diversity and Equity in Rare Disease**<br>• Strategies for Addressing Rare Disease in Emerging Healthcare Systems<br>• Grassroots Strategies Across the Globe<br>• Challenges of Rare Disease Specific to Underserved Populations | | |
| **10:30 a.m. – 12:00 p.m.** | **STREAM ONE:**<br>ROOM: Grand East Ballroom<br>**Translating Research into Care and Treatment**<br>**Winning the Race to Diagnosis**<br>Facilitator: Alex MacKenzie, CHEO<br>1. Reducing the Diagnostic Odyssey for Children: How A Global Commission is Helping Shape the Path Forward - Peter Jones, Microsoft Health<br>2. How structured data is streamlining genomic diagnosis as part of the first pan-Canadian rare disease data repository, Genomics4RD - Orion Buske, PhenoTips<br>3. CHEOmics Clinic - Alison Eaton - CHEO<br>4. GenCOUNSEL: Optimizing Genetic Counselling with the Clinical Implementation of Genome-Wide Sequencing - Alison Elliott, University of British Columbia | **STREAM TWO:**<br>ROOM: Grand Centre Ballroom<br>**Innovations in Therapy: Treating More and Curing Some**<br>**Curative Therapies: Next Step for Rare Diseases?**<br>Facilitator: Ian Stedman, York University<br>1. Using CRISPR to Unravel Gene Networks in Rare Disorders - Kristin Kantautus and Ashwin Seetharaman, University of Toronto<br>2. Gene Therapy for Single Gene Defects: Example of SMA - Susan Manganaro, AveXis<br>3. How CAR-T Cells are Killing Tumours and Saving Lives - Ronan Foley, Juravinski Hospital, McMaster University<br>4. Advancing Cell and Gene Therapies for Hemoglobinopathies - Kevin Kuo, University Health Network | **STREAM THREE:**<br>ROOM: Grand West Ballroom<br>**Addressing Issues of Diversity and Equity in Rare Disease**<br>**Rare Advances at National, Regional, and Global Levels**<br>Facilitator: Lisa Safarty, NORD<br>1. China - Shuyang Zhang, Peking Union Medical Hospital<br>2. Colombia - Gérman Escobar Morales, Director of Health, ProPacífico<br>3. EU Countries - Dorica Dan, Romanian National Alliance for Rare Diseases<br>4. Hong Kong - K.P. Tsang - HK Alliance for Rare Diseases<br>5. WHO Universal Health Coverage Policies and Practices to Insure “no patient is left behind” regardless of social, economic, and political circumstances: Inclusion of Rare Diseases - Yann LeCam |

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| | 5. Reflections from Parent Who Also Happens to be a Genetics Professional - Mazdak Bagherie, University Health Network<br><br>*Discussion* | *Discussion* | *Discussion* |
| **12:00 p.m. – 1:00 p.m.** | **Lunch** | | |
| **1:00 p.m. – 2:30 p.m.** | **STREAM ONE:**<br>ROOM: Grand East Ballroom<br><br>**Designing Pathways to Patient-Centered Care**<br>Facilitator: Sandra Anderson, Innomar Strategies<br><br>1. Disease modeling: Real world patients and Real-world practice of medicine - Michael Liebman, IPQ Analytics<br>2. European Reference Networks: Cross country collaboration and best practices (Galactosaemia) - Eileen Treacy, National Rare Diseases Ireland<br>3. Best Practice Case: Hemophilia - David Page, Canadian Hemophilia Society<br>4. Best Practice Case: CAPS - Ron Laxer, The Hospital for Sick Children<br>5. Bone marrow as a Vehicle for Correction of Rare Disorders: Donna Wall, The Hospital for Sick Children | **STREAM TWO:**<br>ROOM: Grand Centre Ballroom<br><br>**Programs and Strategies for Access to Innovative Therapies**<br>Facilitator: Devidas Menon, University of Alberta<br><br>1. Tania Stafinski University of Alberta<br>2. Suzanne McGurn, Ontario Public Drug Programs<br>3. Vikrant Vats, US Blue Cross/Blue Shield<br>4. Edmund Jessop, former adviser HSTP, NICE | **STREAM THREE:**<br>ROOM: Grand West Ballroom<br><br>**Grassroots Strategies Across the Globe**<br>Facilitator: Fergal Mills, Innomar Strategies<br><br>1. Taiwan Foundation for Rare Disorders - Min-Chieh Tseng<br>2. Agrenska: A Rare Service Model - Anders Olauson<br>3. Community-based RD Networks - Rachel Yang, Chinese Organization for Rare Disorders<br>4. RDD Japan 10th Anniversary - Yukiko Nishimura<br>5. Newborn screening and Comprehensive Care Program for Sickle Cell Disease in Ghana - Isaac Odame, The Hospital for Sick Children<br><br>*Discussion* |

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| | *Discussion* | | |
| **2:30 p.m. – 2:45 p.m.** | **Refreshment Break** | | |
| **2:45 p.m. – 4:15 p.m.** | **STREAM ONE:**<br>ROOM: Grand East Ballroom<br>**Empowering the Whole Person**<br>Facilitator: Maureen Smith, CORD<br>1. Designing Care for and by Families - Isabel Jordan, Rare Disease Foundation<br>2. RareConnect: Connecting and Engaging Rare Disease Patients Globally - Matthew Osmond, CHEO<br>3. Psychological, Behavioural, Social & Ethical Aspects of Rare Neurological Disease: Ataxia - Ramaiah Muthyala, Indian Organization for Rare Disorders<br>4. Engaging Young People with Rare Diseases Around the Globe - Brynne Dalmao, HDYO<br>5. Use of Clinical Patient Registries to Inform Best Practices Including Access to Therapies - Craig Campbell, London Health Sciences Centre<br>*Discussion* | **STREAM TWO:**<br>ROOM: Grand Centre Ballroom<br>**Drivers of Drug Development – Regulatory Collaboration**<br>Facilitator: Oxana Iliach, IQVIA<br>1. Health Canada - Fiona Frappier<br>2. European Medicines Agency/Medicines and Healthcare Products Regulatory Agency - Daniel O'Connor<br>3. US CDR, FDA - Lucas Kempf<br>4. US FDA - Larissa Lapteva | **STREAM THREE:**<br>ROOM: Grand West Ballroom<br>**Ethnic and Diversity Issues**<br>Facilitator: Étienne Richer, CIHR<br>1. Inclusion in Clinical Trials - Jeff Keefer, IQVIA<br>2. Indigenous Populations Silent Genome Project - Laura Arbour, University of Victoria<br>3. Addressing Rare across French Canada - Gail Ouellette, RQMO<br>4. State-of-the-Art Across Key Countries and Pathways to Progress - Eva Maria Ruiz de Castilla, EMOLUVA Partners LLC<br>5. Engaging Medical Students in Rare Disease - Jessie Kulaga-Yoskovitz and Kristin Hunt, McGill University<br>*Discussion* |

## Sunday, May 12, 2019: Skills Training and Capacity Building

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| **8:30 a.m. – 9:00 a.m.** | **Continental Breakfast: Grand East Ballroom** |
| **9:00 a.m. – 9:30 a.m.** | **Overview of Day 1 & 2**<br>Durhane Wong-Rieger, CORD<br><br>**PARTICIPANTS MOVE INTO WORKSHOP SESSIONS** |
| **9:30 am – 12:00 pm** | **Theme One: Diagnosis and Beyond** (ROOM: Grand East Ballroom)<br>**WORKSHOP A: Genetic Counselling: Making Sense of Newborn Screening and Genetic Diagnoses** - Breanne Dale, Andrea Djolovic, Canadian Association of Genetic Counsellors, Alison Elliot, University of British Columbia |
| | **Theme Two: Empowering the Patient Community** (ROOM: Grand Centre Ballroom)<br>**WORKSHOP B: Designing culturally appropriate patient empowerment solutions** - Lynda Brown-Ganzert, CURATIO |
| | **Theme Three: Platforms for Rare Disease Collaboration and Coordination** (ROOM: Grand West Ballroom)<br>**WORKSHOP C: APEC Rare Disease Framework: Designing and Developing National Plans** - Cameron Milliner, Sue Fletcher, Co-Chairs APEC Rare Disease Network |
| | **Theme Four: Patient Registries, Data Sharing, and Targeted Therapies** (ROOM: Sheraton A)<br>**WORKSHOP D: From Genetic Research to Precision Therapy**<br>• CoRDS Program and Targeted Therapy Research - Benjamin Forred, Sanford Research<br>• How NORD Fosters Patient-Driven Registries - Pam Gavin, NORD, Jessica Bohonowych, Foundation for Prader-Willi Research |
| | **Theme Five: Patient-Engagement Skills Development** (ROOM: Sheraton B) |

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| | **WORKSHOP E: NCATS Toolkit for Patient Focused Therapy Development** - Eric Sid, NIH, NCATS Office of Rare Diseases Research |
| | **Theme Six: Rare Cancer Networks and Personalized Care** (ROOM: Sheraton C)<br>Facilitator: Bill Dempster, 3Sixty Public Affairs<br>**WORKSHOP F: Rare Cancer Networks Bridging Common Cancers and Rare Conditions** - Richard Vines, Rare Cancer Australia, Lisa Machado, CORD/Canadian CML Network |
| **12:00 p.m. – 1:00 p.m.** | **LUNCH** |
| **1:00 p.m. – 3:30 p.m.** | **Theme One: Diagnosis and Beyond** (ROOM: Grand East Ballroom)<br>**WORKSHOP G: Cell and Gene Therapy from Laboratory to Market** - Facilitators: Saffiya Gassman, Pfizer, Durhane Wong-Rieger, CORD |
| | **Theme Two: Empowering the Patient Community** (ROOM: Grand Centre Ballroom)<br>**WORKSHOP H: Empowering and Supporting Caregivers**<br>• Supporting the Patient Ecosystem - Cheryl Petruk, Canadian MPN Network<br>• Move On: Dealing with Anger and Anxiety - Karen Tompkins, Westview Freedom Academy |
| | **Theme Three: Platforms for Rare Disease Collaboration and Coordination** (ROOM: Grand West Ballroom)<br>**WORKSHOP I:** Unleashing the Power of Real-World Evidence - Efficiently Delivering Regulatory Grade Data at Last - Femida Gwadry-Sridhar, Pulse Infoframe |
| | **Theme Four: Access to Rare Disease Medicines** (ROOM: Sheraton A)<br>**WORKSHOP J: Alternative Access Schemes: Managed Access (Entry) Programs with Risk Sharing** - Tania Stafinski, University of Alberta, Judith Glennie, PRISM |

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| | **Theme Five: Patient-Engagement Skills Development** (ROOM: Sheraton B)<br>**WORKSHOP K: Organizational Skills Building**<br>• Essential and Best Practices for Small Organizations - Devon Pfeil, Global Genes<br>• Social Media 1.0 and 2.0 - Christian Rubio, Rose Mary Moegling, Global Genes |
| | **Theme Six: Orphan Drug Pricing for Innovation and Access** (ROOM: Sheraton C)<br>**WORKSHOP L: Project Hercules: A UK Duchenne Global Collaboration** - Josie Godfrey, JG Zebra Consulting |
| **3:30 p.m.** | **Conference concludes** |

**The Canadian Organization for Rare Disorders and Rare Diseases International acknowledges the contribution of all our Corporate Partners to improving the lives of patients and families with rare disorders.**

**We are especially grateful to the following that have supported this conference.**

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