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**Canada’s Clinical Innovation in 50 Years: The Future May Be Rare**

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Speakers: Kym Boycott, Leader, Care4Rare Canada and Rare Diseases Models and Mechanisms networks; Susan Marlin, CEO, Clinical Trials Ontario; William McKellin, Medical Anthropologist, University of British Columbia; Durhane Wong-Rieger, CEO, Canadian Organization for Rare Disorders

Moderator: Lawrence Korngut, Associate Professor, Department of Clinical Neurosciences, Hotchkiss Brain Institute, University of Calgary

**Takeaways and recommendations**

Policy considerations

* Canada needs a national data infrastructure for rare disease research that is cloud based and uses internationally recognized data standards such as those developed by theGlobal Alliance for Genomics and Health. Such an infrastructure should include standard operating procedures, harmonized agreements between provinces (i.e. for sharing patient data) and a governance framework which includes terms for data access.
* Proposed changes to Patented Medicines Regulations may delay new drugs and hinder life science investments.
* Canada needs an effective policy for moving drugs from off-label to on-label; this would also allow for reimbursement of these drugs.
* Canada needs a fully developed regulatory framework for orphan drugs that provides incentives for R&D, similar to U.S. (Orphan Drug Act of 1983) and Europe. This would create a harmonized system that allows companies to apply simultaneously to Canada, U.S. and Europe for an orphan drug designation.
* Canada needs to take a life cycle approach to drug development, access, monitoring and feedback—one that involves patients and families.
* Canada needs a policy that would provide patients with early access to drugs not yet approved here.
* Canada would benefit from uniformed newborn screening of diseases.

Research considerations

* The clinical community has come together with a common vision: to enable all people living with a rare disease to receive accurate diagnosis within a year; next generation sequencing reduces the time and cost of diagnosis by simultaneously examining many to all genes at once.
* Achieving that vision will require fundamental changes to the way science is conducted, shared and applied to the care of patients with rare diseases (e.g. FORGE Canada and Care4Rare Canada research consortia)
* Canada has a strong collaborative model for rare disease research and a strong clinical trials environment.
* Opportunities include: increase efficiencies of clinical trials (e.g. reduce study start up); improve patient/public engagement with trials; collaborate across Canada to improve performance
* Micro-grants (about $3500 each), peer-reviewed by scientists and parents, can help establish the relevance of a particular research project.
* Fund more clinician-driven research. This would allow for off-label testing, and the development of innovative treatment protocols based on a particular patient.
* There is a need for a national centre of research excellence to bring all the stakeholders together to address research gaps.

Patient/family considerations

* Consider the importance of patient- and family-reported outcomes (“Personally Meaningful Outcomes”).
* Breakdown silos (e.g. research, health, education) that segregate families by the disease; instead, respond to shared experiences that cut across disease types and research disciplines.
* Almost every rare disease policy was the result of patient group advocacy and rare disease groups are increasingly taking the lead in funding and designing research programs.