

Breakout Session 1

How should Canadian RD research networks/consortia should be defined and categorized/grouped (for example: cluster of conditions, number of sites, research expertise, patients' engagement, others)?

Breakout Session 1: Notes

- Hybrid approach generally endorsed
- Interest in working together re: common challenges
 - all rare diseases: e.g., innovative methods
 - grouping re: disease areas, themes, sites where shared infrastructure would be beneficial
- Primary concern: equity
 - Access to research & care for patients who live outside large centres
 - Core principle: all diseases in a cluster supported
- Need to think about clinical care vs research (need to discuss re: Canada) – “knowledge to follow patient”

Breakout Session 2

Does Canada need a national research Infrastructure to support RD research networks/consortia? If yes, what supports to foster collaborative research should the infrastructure provide?

- For clinical trials:
 - Capacity building for sites interested in participating in trials
 - Specific capacity-building to support innovative trial designs that are suitable for rare diseases – e.g., n of 1 trials
 - Methods but also submissions to regulator
- For research that will support reimbursement decision-making:
 - Support for research that may lead to submissions to CADTH – e.g., support with study designs, evidence standards, etc.
 - Real-world evidence – e.g., to support post-market surveillance & follow-up post-trial
- Leverage existing data and infrastructure – scaling up to better serve current needs and to stimulate research - be more nimble in connecting research to practice
 - e.g., registries, electronic health record data, linkage of research data to health care data
 - Recognize innovations, facilitate evaluation & implementation
- Bringing patients/caregivers, providers, researchers together
 - Capacity building for the advocacy community

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- Horizon Scan needed
 - Help identify gaps
 - Grass roots approach
- Inclusivity (non-human research, pre-clinical research)
- Industry funding (industry funded and IIT lead projects)
- Categorizing by conditions?

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- Training
- Local point of contact at each centre to facilitate research
- Funding mechanism to support the discovery phase into patients
- Registry-need funds to support data entry
- Online platforms to be efficient
- Streamlined processes and using repository of knowledge so as not to re-invent the wheel
- Grant writing support to help with the sustainability model

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FORM following FUNCTION

- Site Based
 - Not ideal
 - Look for areas of alignment
 - Research but also alignment but also patient care –
 - Watch building too unique structure – siloed
- Cluster Model
 - Has advantages
- Hybrid
 - Between European – but also need local sites
 - Centralized
 - RFA process
 - “Paving the path – ie technical network
 - “Scientific / investigation” network – ie design and manage protocols
 - Local
 - Expensive as it requires local capacity building
 - Consortia (how broad is each one? – some advocacy for some higher level grouping,
 - Core resources (ie generic capacity for research)
 - Integration of clinical care and research
 - Responsive to top down (industry or federal research funding)
 - Bottom up – ie locally developed trials, RWE, longitudinal,
- General notes
 - Joining / partnerships at the level of projects or consortia or even sites
 - Leverage our expertise but also be honest regarding our size – so connections will be crucial

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- National support
 - Tension between jurisdictions (HOW!)
 - Priorities for research
 - Adults to children
 - Standard of care – addressing inequities and including lived experience(s) – all patients
 - Registries
- ERNs..... Face similar issues – initially focused on clinical care leverage capacity and expertise in large programs (ie cross border care), then secondarily why not start longitudinal outcomes and clinical trials)
 - Start easy...
 - Transfer of funding would come with cross “border” or “within border” care
- Centralized
 - RFA process
 - “Paving the path – ie technical network
 - “Scientific / investigation” network – ie design and manage protocols
 - DCC.... Likely no single mode
 - They key is interoperability locally, nationally, internationally...
 - “All for one” central registry and genomics library
 - Study coordination

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Facilitator – Dr Craig Campbell

Reporter – Bonnie Wooten

7 Participants

What does a Rare Disease Network look like:

- Canada is a large country and small population spread out
- Do not have a large number of hospitals that can be centres of excellence
- Have main hospitals ignite the system and community hospitals monitor
- Hub model was highlighted as a way to go by one person
- Consider personality of Canada – do we want more collaborative facing centres and less individual facing? What is good for Canada
- Consider what we would focus or what the model would be
- Is a centre of excellence focused on clinical care or the site
- Can not separate the clinical need from research – one drives the other – Marry the two and enhance each other

Session 1 Cont'd

- Knowledge dissemination – knowledge is being held in institutions and not much trickles out - need more networks disseminating the information – like a centralized network (CT/Biometrics/rare diseases, all stakeholders)

4 Needs

1)Centres of excellence with CT capacity

2)Real world data sources

3)Natural history studies

4)Knowledge of dissemination

- Keep in mind to narrow focus of accomplishments – 1st year and 2nd year – can not do everything or be all to everyone. Focus on a pathway or network
- Remember it is a competition
- Be ready and prepared – less risk
- Clustering Rare Diseases is important – work with NM network

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Question – does it Need a Research Infrastructure?

- Lets not reinvent the wheel – build on Micyrn and CHEERS
- CT's are poorly resourced – a struggle to mobilize
- Build capacity
- Build efficiencies and focus on patient
- Different rules and policies with each hospital for patient registries
- Centres of Excellence will build capacity
- Lack of connection to everything – all issues /common problems
- Leverage it all – connect the dots
- Anticipated funding – unknown
- Think Big – beyond partnerships -we are a big country