Patient-oriented research in Newfoundland and Labrador – sharing our examples, challenges and successes

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NEWFOUNDLAND AND LABRADOR
SUPPORT
Support for People and Patient Oriented Research and Trials

Outline

- Two examples from my own work (before my involvement in SPOR and the SUPPORT unit)
- Examples of success at the SUPPORT Unit level
- Challenges we have faced in POR and our attempts to mitigate these
- Myths interwoven throughout

Myth 1 – it can't be done

- Patient oriented research takes too much time, effort, money
- Patients are not 'trained' or 'qualified' to conduct research

My recent experience – it *can* be done (but it takes effort!)

Currently running two projects, each of which has a patient partner

But...their involvement is at different stages and they have different roles

- Breast cancer surgical treatment choices – patient and surgeon opinions (patient partner, Becky)
- Universal tumor testing for Lynch syndrome – perspectives of key stakeholders (patient partner, Doug)

Breast cancer project

An example of recruiting a patient to be a member of the research team before the study protocol was fully designed

Becky was recruited by my co-PI who knew her professionally years ago

Becky came to the first team meeting and from then on, has had a full voice in every decision made about the project – just like every other team member

Becky's expertise comes in being a person who has made a surgical decision for breast cancer – lived experience

Becky's role

To contribute to the initial discussions about the project where methods and measures were decided, then to review the grant application

To advise on the ethical and practical implications of the methods throughout the project

To help collect data (e.g., she attends focus groups)

To help analyse focus group transcripts

To help plan the knowledge translation event at the end of the study

In contrast, tumor testing project

Doug has had colon cancer and was not recruited at the beginning of the study

This study includes consulting with pathologists and genetic counselors across the country (via online surveys), but also a postal patient survey in Newfoundland

Doug was brought into the study at Phase 3 – the patient survey, well over a year from the study start

He was also recruited by word of mouth as I talked about the project with colleagues

Doug's role

Doug's lived experience is as a patient who would be eligible to complete the survey

He advised on ethical and acceptable ways of getting the surveys to patients

He was instrumental in drafting the survey:

- Both determining content areas;
- And in particular the opening paragraph explaining tumor testing. We were too complex, he parsed the text to a manageable reading level

Doug's experience

Doug has refused offers to take part in the whole project, explaining the patient survey is what is important to him and where his lived experience can best be used

However, he has expressed interest in being a part of the knowledge translation event at the end of the study

He would like to see the results of the other two phases and have a chance to help draft end-of-study recommendations

What have these patients taught me?

Talk often and openly about your desire to find a patient partner – they are often recruited through word of mouth

Have informal, initial chats upfront – I met both these partners at local coffee shops to verbally explain what I was looking for

Patients are the best judge of what they can offer once they get a sense of what our projects are about

Having them on the team has really not been all that different than any other team member who brings different expertise - but regular communication assures them research can move slowly and I haven't forgotten them!

Myth 2 - patients have their own agenda

Patients will focus only on their own condition/illness and be unable to advise more broadly or give a perspective of a wider range of patients – i.e., they will somehow 'hijack' the project

Success at the SUPPORT Unit level

Our Patient Advisory Council (busting Myth 2)

Patient Advisory Council

A group of up to 25 members of the general public from all areas of our province, est. in 2015

Currently, we have 15 members, with 8 youth about to join

With them, we created a terms of reference

Two face-to-face meetings per year, two web/teleconference meetings; ad hoc events as they arise (e.g., with them, we hold a lay scientific day each year – this year, at a local farm)

Patient Advisory Council

The PAC advises the SUPPORT Unit on all aspects of governance and operation

Two members sit on the large steering committee for the Unit's activities and have an equal vote

All members of the council sit on *at least* one research project as a full team member

The council regularly advises stakeholders on POR – this can be anything from determining study questions, to reviewing study tools, to reviewing grant applications and student fellowships, and the list goes on

Challenges

Time – finding patient partners and maintaining real relationships with them takes time. No easy fix here, but ***early*** thinking and discussion generally works

1. Recruitment of patient partners (what is the process of recruitment and where do you find them?)
2. Patient partner compensation

Recruiting

Recruiting patient partners is NOT like recruiting patients to be study subjects

- Think of it as a hiring process
- Patients Canada suggests we develop a candidate profile and job description
- Screen candidates and conduct an interview

Alies Maybee et al. (2016) Partnering with citizens in research. What is helpful from our persepctive
http://www.patientscanada.ca/index.cfm?pagepath=Make_an_Impact/Strategy_for_Patient_Oriented_Research/Tools_for_Patient_Partnership_in_Research&id=76578

´Role description´

What do you need and how can patients help?

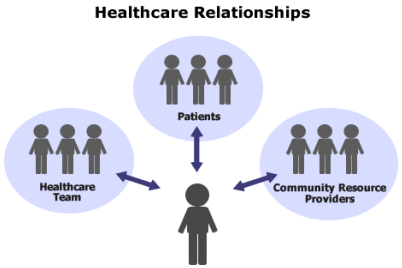
Critical for outlining expectations

- Characteristics of the research project
- Perspectives you are looking for
- Commitment
- Support available
- Recognition and compensation
- Contact information
- Other?

Recruitment strategies

Social marketing recruitment

Community outreach recruitment



Health system recruitment

Partnering recruitment

Vat E, Ryan D, Etchegary H. Recruiting patients as partners in health research, Res Invol Engagement 2017; 3:15
https://researchinvolvement.biomedcentral.com/articles/10.1186/s40900-017-0067-x

To compensate or not?

- Consider and discuss compensation with patients
  - recognition of their time and expertise
  - may take the form of a gift card, money, thank you – **this depends on your institution**
  - reimbursement for fees and expenses related to the research

- **SPOR – CIHR Compensation Guidelines for Engaging Patients as Partners in Research (these should be available any day)**

- **We developed local guidelines and policies after (many!) consultations with our advisory council, finance office, human resource office, legal**

- **Interestingly, our advisory council did not want to use the word compensation, but rather 'appreciation'**

What should you know?

- Determine what kind of Patient Compensation is an Eligible Expense
- Remember that compensation could require the issuing of T4 slips
- Clearly communicate that compensation can impact patients´ tax status
- Give options and discuss what is feasible together
- Budget for Patient Engagement!
- Recognition is as important as compensation

Last myth

- It's too new, we have no evidence on impact, we don't know what we're doing

- I would argue that while formal evaluation of patient engagement in research is indeed lacking, we know quite a bit about best practices and have good libraries of examples

Useful resources

- INVOLVE - http://www.invo.org.uk/
- PCORI - www.pcori.org
- Good databases of examples
- And guidance on numerous issues – e.g., evaluation rubric, briefing notes about ethics, compensation, recruitment, etc.

Some of our work

Recruiting patients as partners in health research: a qualitative descriptive study

Lidewij Eva Vat, Devonne Ryan and Holly Etchegary; *Research Involvement and Engagement*2017**3**:15; https://doi.org/10.1186/s40900-017-0067-x

Engaging patients in health research: identifying research priorities through community town halls. Holly Etchegary, Lisa Bishop, Catherine Street, Kris Aubrey-Bassler, Dale Humphries, Lidewij Eva Vat,Brendan Barrett

BMC Health Services Research; 2017**17**:192

https://doi.org/10.1186/s12913-017-2138-y

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Can-SOLVE CKD Network

Strategy for Patient-Oriented Research SPOR Putting Patients First

Canadians Seeking Solutions and Innovations to Overcome Chronic Kidney Disease

October 24, 2017

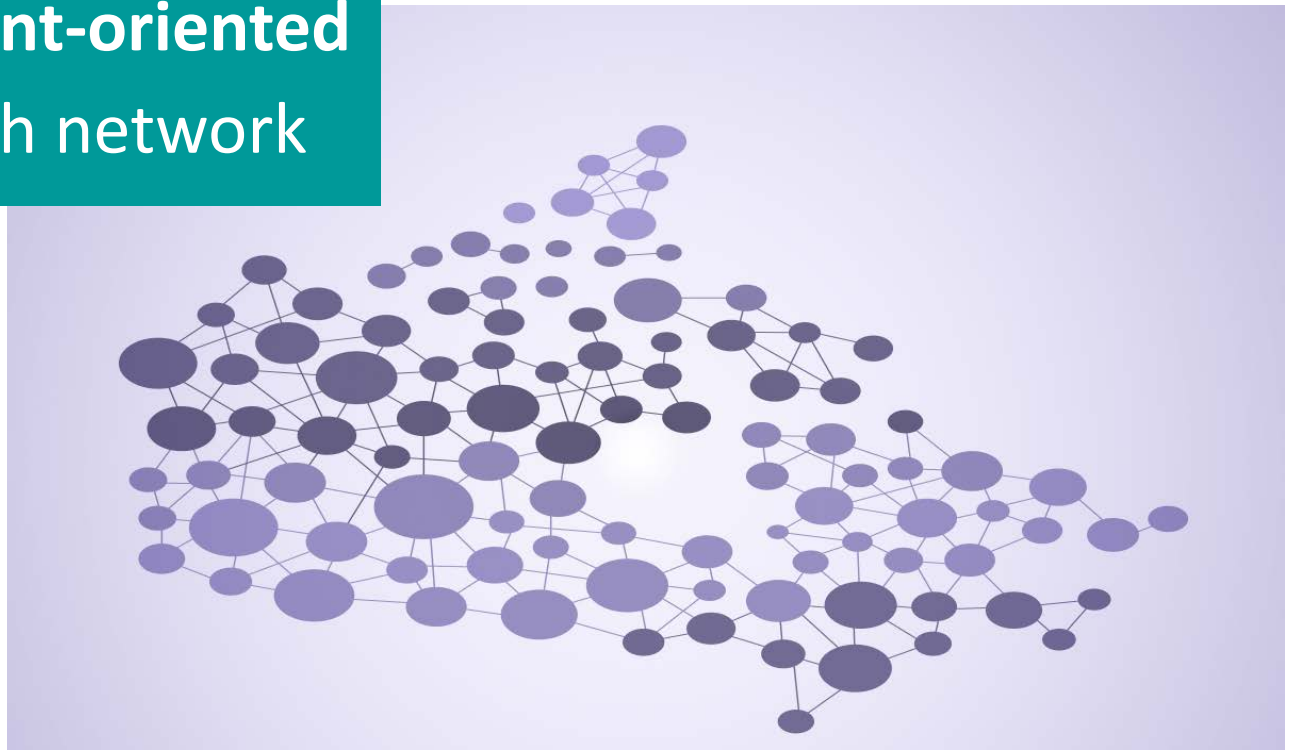
Michael McCormick
Co-Chair, Can-SOLVE CKD Patient Council

Biography

- Diagnosed with CKD and started hemodialysis in 1987
- Have had two failed transplants due to recurring disease
- Have dialyzed in-centre in Toronto, Waterloo, and Calgary
- Currently on Home Hemodialysis
- Senior Manager Operations, Toronto Stock Exchange and Montreal Exchange
- Got involved with Ontario Renal Network in 2012, with the creation of Ontario Renal Plan II
- Chair the ORN provincial Patient and Family Advisory Council
- Ontario Renal Network is funding partner for CanSOLVE CKD, SPOR initiative for renal research
- Chair the CanSOLVE national patient council

Can-SOLVE CKD Network

A national **patient-oriented** kidney research network



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VANCOUVER SUN

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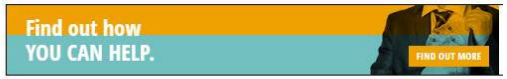

Vancouver doctor co-leads national kidney disease project

ERIN ELLIS, VANCOUVER SUN 03.31.2016 |

A $59-million, five-year national research program into kidney disease announced Thursday will be led from B.C. and Alberta. Dr. Adeera Levin, head of nephrology at the University of B.C., joins with University of Calgary's Dr. Braden Manns, a nephrologist and health economist, in overseeing studies that aim to reduce Canada's reliance on kidney dialysis and transplants. VANCOUVER SUN / UBC HANDOUT

SHARE

VANCOUVER - A $59-million, five-year national research program into kidney disease announced Thursday will be led from B.C. and Alberta.

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B.C. team co-leads $59M kidney disease initiative

Research into improving care across Canada will involve patients and indigenous communities

CBC News Posted: Mar 31, 2016 2:22 PM PT | Last Updated: Mar 31, 2016 3:32 PM PT

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Trying to 'change the culture of medicine' by letting patients decide what gets researched

Patients can bring attention to issues they face that may not previously have been studied

By Daryl Hol, CBC News Posted: Mar 12, 2017 5:00 AM ET | Last Updated: Mar 12, 2017 5:00 AM ET

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Our vision

By 2020, every Canadian with or at high risk for chronic kidney disease will:

Receive the **best recommended care**

Experience **optimal outcomes**

Have the chance to participate in studies with **new treatments**

Listening, learning, leading

To patients, caregivers, policy-makers, researchers & clinicians

From patients, caregivers, policy-makers, researchers & clinicians

With patients, care givers, policy-makers, researchers & clinicians

Patient engagement

Patients are partners in:

- Study design
- Study execution
- Study interpretation
- Study result communication…and more!

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150+ network members

- Patients living with kidney disease
- Researchers
- Health care providers
- Administrators and policy-makers

Patient Council

30+ members:

- People living with CKD
- Kidney donors
- Indigenous peoples
- Women and men of all ages
- Different medical conditions
- Diverse ethnic backgrounds
- Urban, rural, & remote areas

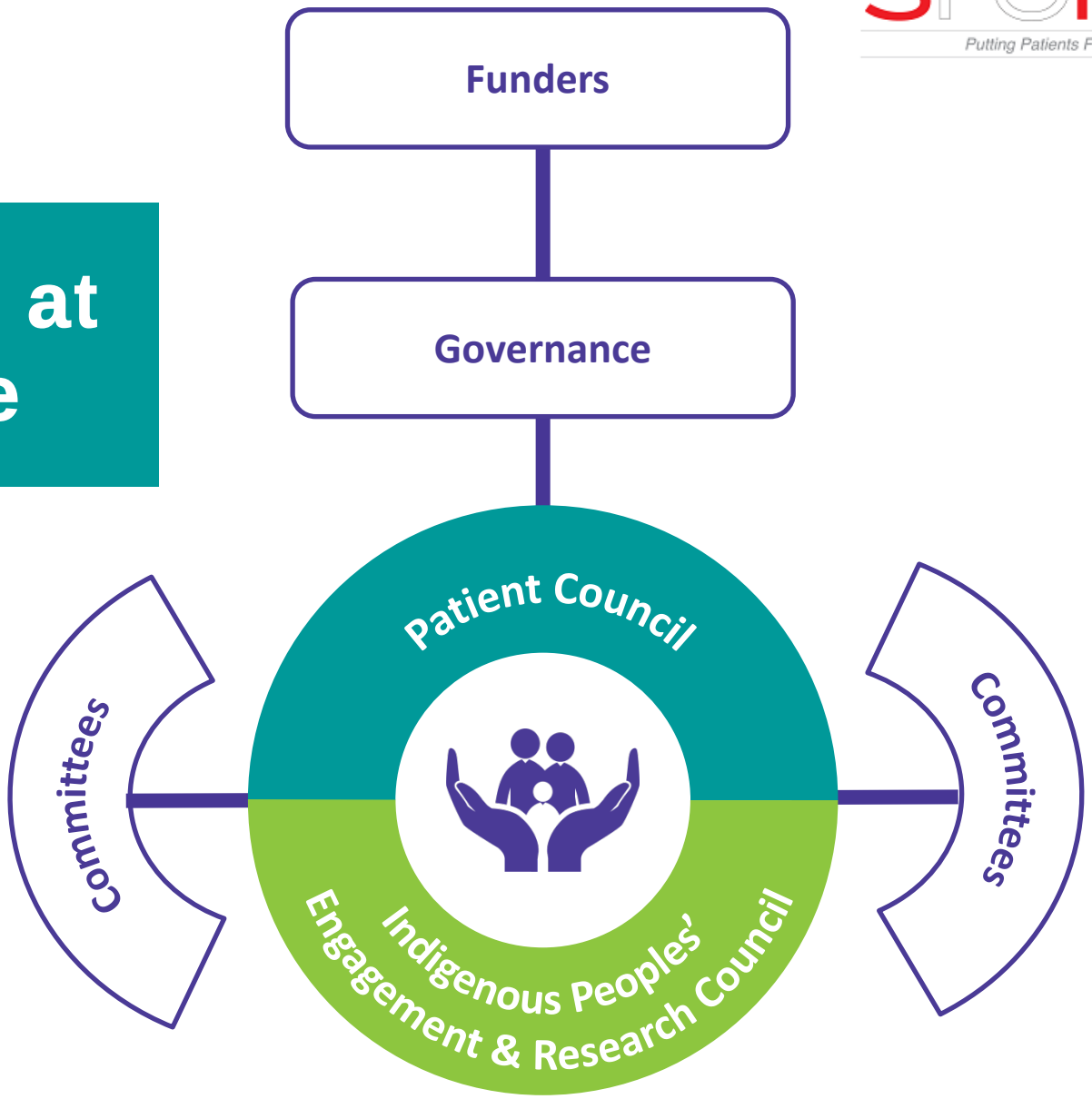
Patient Council

- Led by 3 patient co-chairs
- 2 working groups ensure actions are guided by patient input:

Research Projects & Recruitment

Knowledge Translation, Communications & Outreach

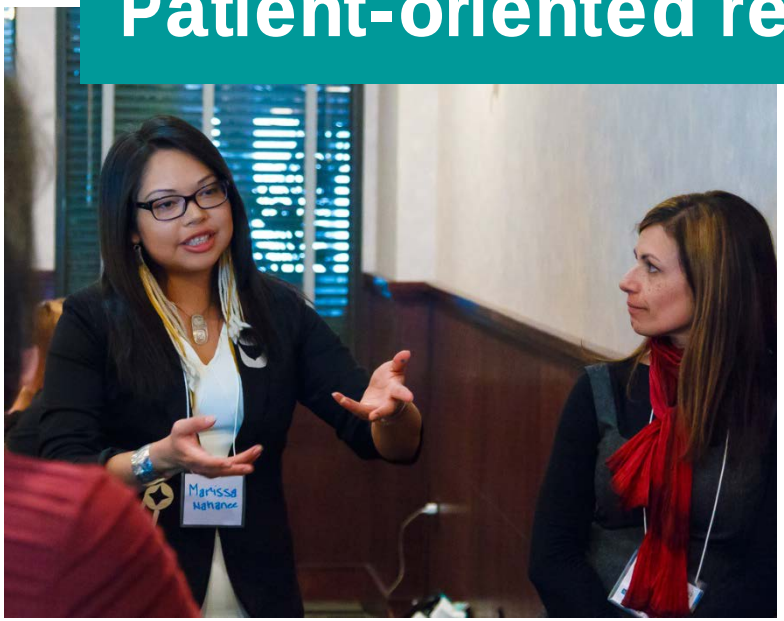
Patients are at the centre



Indigenous Peoples' Engagement & Research Council

- Supports collaboration grounded in traditional values and partnerships with Indigenous communities
- **~15 members:** Indigenous patients, caregivers, researchers and community leaders

Patient-oriented research



3 years of priority-setting discussions led to a research program based on **patient questions.**

3 research themes

Identify kidney disease earlier and support those at highest risk

Define the best treatments to improve health and quality of life

Deliver innovative, patient-centred treatment and care

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18 research projects

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Theme 1: Earlier diagnosis

Why wasn't my kidney disease identified earlier?

- Defining CKD risk in youth with diabetes (2 projects)
- Identifying diabetes & CKD in Indigenous communities

How can we identify and treat those at highest risk for kidney failure?

- Defining risk & personalizing treatment for GN & ADPKD
- GN translational research program

How can we identify those at highest risk for adverse outcomes?

- Integrating kidney failure risk equations into clinical care
- Predicting risk of heart complications in patients with CKD

Theme 2: Better treatments

What are the best treatments to improve the health of patients with CKD?

- Cell therapy for advanced diabetic kidney disease
- Clinical trials of promising re-purposed drugs for ADPKD
- Aldosterone inhibition and enhanced toxin removal in hemodialysis patients

What are the best ways to manage symptoms?

- Dialysis symptom control
- Etiology of pruritus during dialysis
- Patient-reported outcomes

Theme 3: Optimal care

What model will best deliver evidence-based personalized care?

- Restructuring kidney care to meet the needs of 21st century patients

How can we better enable self-management, where appropriate?

- Targeted de-prescribing in patients with CKD
- Strategies to enhance patient self-management of CKD

How can we help patients access the best treatment for kidney failure?

- Improving patient knowledge about treatment options
- Increasing the use of living donor kidney transplantation

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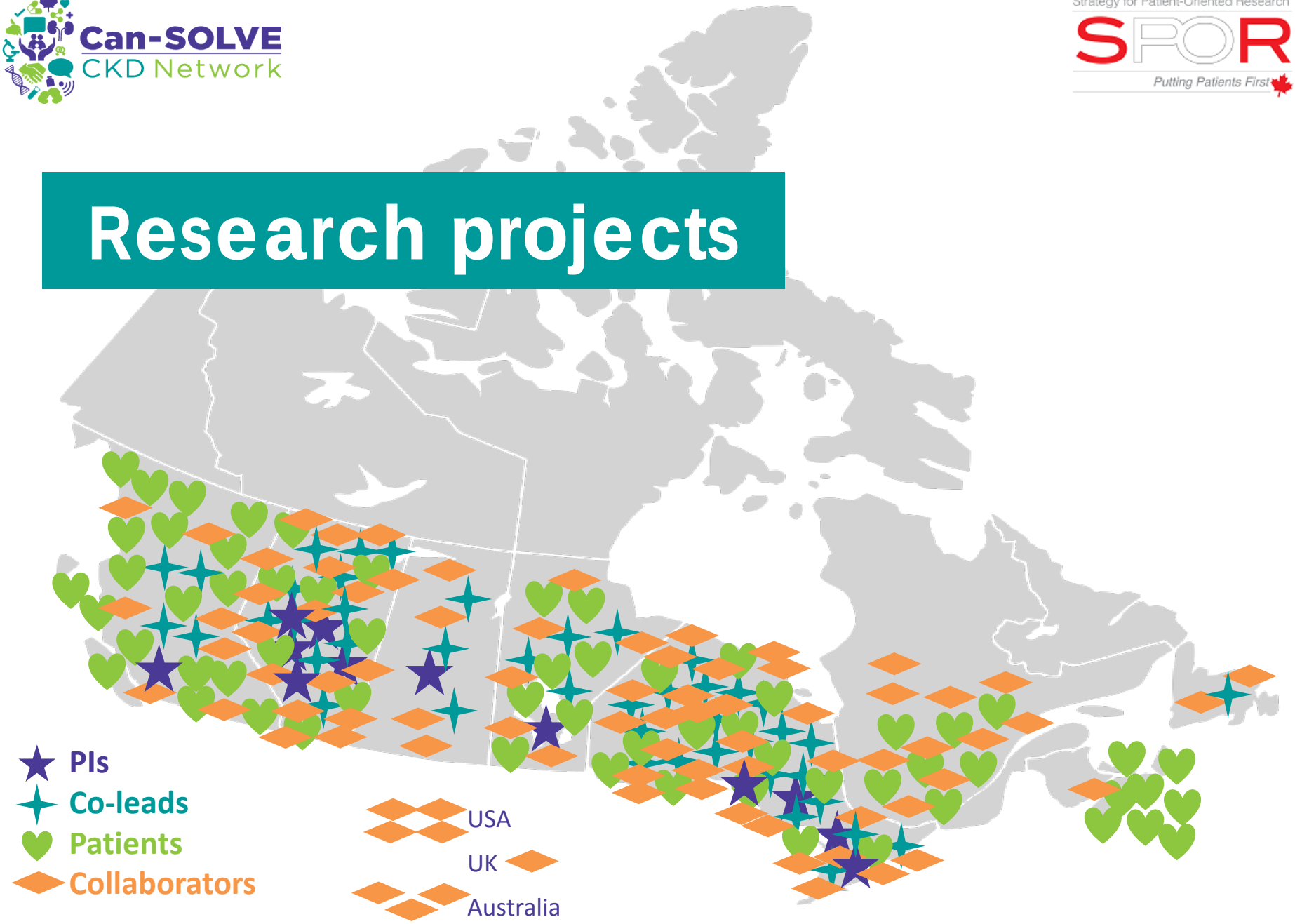
Research projects



Project 3.1A: Restructuring kidney care to meet the needs of 21st century patients

- Using surveys and patient-led focus groups to identify aspects of the patient experience that are highest priority to change
- Results of initial studies to drive further study of most promising and relevant tools, strategies and models to transform kidney care

Myths

- Patients and researchers can't / don't speak the same language
- Patients will only represent their own interests

Successes

- Empowered patients on all aspects of the research project
- Regular check in meetings / calls
- Training curriculum
- Clear and agreed upon understanding of why patients are being engaged
- Mutual Respect: Researchers, practitioners and patients acknowledge and value each other's expertise and experiential knowledge

Challanges

- Researchers are out of their comfort zone
- Knowing when and how to engage patients
- Patients understanding of research process and admin issues ie. ethics board submissions, institution contracts, funding agreements
- Varying skillsets among patients

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Research projects transforming care

- Improving symptoms for those on dialysis
- Improving living donor rates and experience
- Delaying progression with new treatments
- Identifying and treating early CKD

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Strategy for Patient-Oriented Research SPOR Putting Patients First

Funded by the Canadian Institutes of Health Research and more than **30 partners**

Can-SOLVE CKD Network

Strategy for Patient-Oriented Research
SPOR
Putting Patients First

One of five chronic disease networks established through the national **Strategy for Patient-Oriented Research**

Can-SOLVE CKD Network

CPN
CHRONIC PAIN NETWORK
RDC
RÉSEAU DE DOULEUR CHRONIQUE

CHILD-BRIGHT Network

Diabetes Action Canada

IMAGINE
Inflammation, Microbiome & Alimentation Gastro-Intestinal & Neuropsychiatric Effects

Can-SOLVE CKD Network

Strategy for Patient-Oriented Research SPOR Putting Patients First

Get involved!

info@cansolveckd.ca
(604) 806-9376

www.cansolveckd.ca

Thanks

- Patient partners
- Researchers, policy makers and other partners
- Core operations team
- Funding sources
  - CIHR—SPOR Networks in Chronic Disease
  - The Kidney Foundation of Canada
  - Juvenile Diabetes Research Foundation
  - Provincial renal agencies
  - Private donors
  - Industry sponsors
  - Many others

Health Canada Santé Canada

Canada

CURRENT STATE OF PATIENT INVOLVEMENT IN THE REGULATORY PROCESS IN CANADA

Agnes V. Klein,MD; Stephanie Hardy,MSc; Robyn Lim,PhD
Health Canada,
Deborah Marshall, PhD
University of Calgary

October, 2017

YOUR HEALTH AND SAFETY... OUR PRIORITY.

Points for Consideration

- Background and Regulatory Review
- Health Canada's Plan for Regulatory Transformation
- Principles for Transformation
- Openness and Transparency
- Vanessa's Law (Bill C-17)
- Examples of patient involvement
- Questions for the future

Background to Regulatory Review

- Health Canada's Health Products and Food Branch (HPFB) is the national regulatory authority responsible for evaluating and monitoring the quality, safety, and efficacy of therapeutic products in Canada.
- Regulatory benefit-risk assessments underpin Health Canada’s decisions across the life-cycle of a therapeutic product.
- Canada has an established practice, albeit implicit and often *ad hoc*, for including patient perspectives in both operational and policy-based regulatory decision-making.

Health Canada's Plan for Transformation

- **Objective: An agile regulatory system that supports better access to therapeutic products based on healthcare system needs**

Expanded collaboration with health partners

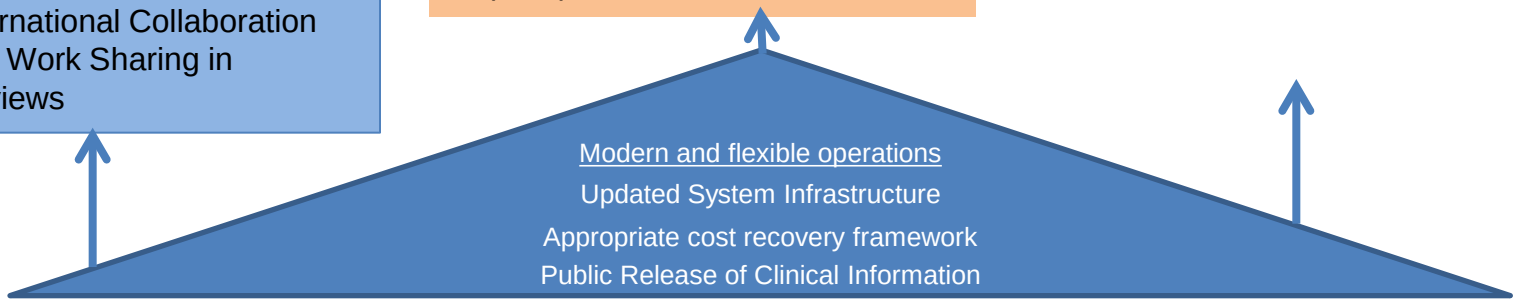
- Alignment of the Health Technology Assessment (CADTH) Review with Health Canada Review
- Implementing a Mechanism for Early Parallel Scientific Advice
- Use of Foreign Reviews/Decisions
- International Collaboration and Work Sharing in Reviews

More timely access to drugs and devices

- Expansion of Priority Review Pathways
- Improving Access to Biosimilars and Biologics
- Improving Access to Generic Drugs
- Building Better Access to Digital Health Technologies
- Pre-Submission Scientific Advice for Medical Devices
- Special Access Programme (SAP) Renewal

Enhanced Use of real-world evidence

- Leveraging Data for Assessing Drug Safety and Effectiveness
- Strengthening Post-market Surveillance of Medical Devices



Principles

- Mandate of our Minister allows for innovative approaches to drug development
- Can consider newer and diverse trial designs
- Considering priorities for products/uses that need to fulfill health care system needs
- Reducing lag between regulatory approval and funding decisions in the health care system
- Increased use of post-market data (pragmatic studies and other methods)
- Other practical additions to the regulatory process

Recent Changes (1)
Transparency and Openness

- Recent legislative amendments and Health Canada's Regulatory Transparency and Openness Framework aim to:
  - enhance the transparency of the regulatory review processes, and
  - provide public information about review decisions
- Opportunities to advance in the area of seeking and considering patient perspectives throughout the lifecycle of therapeutic products.

2) Protecting Canadians from Unsafe Drugs Act Vanessa's Law (Bill C-17) Nov 2014

- Amendments to *Food and Drugs Act* to improve Health Canada's ability to collect post-market safety information, and take appropriate action when a serious risk to health is identified.
- Key amendments include:
  - Power to require information, tests or studies
  - Power to require a label change/package modification
  - Power to recall unsafe therapeutic products
  - Ability to disclose information in certain circumstances
  - Tougher measures for those that do not comply
  - Mandatory reporting of serious adverse drug reactions and medical device incidents by healthcare institutions

Canadian Examples of Patient Involvement

Scientific/Expert Advisory Committees

- Patient advocates serve as members of Health Canada's standing Scientific and Expert Advisory Committees to provide medical, technical, and/or scientific advice, practical and contextual perspectives, to help resolve issues
- Patient advocates on ad hoc Expert Advisory Panels as-needed to provide advice on specific drug submissions or on emerging and/or controversial issues post-market.
- Examples include:

1) panel on use of insulin of animal origin and its place in the treatment of Type 1 diabetes mellitus;

2) public forum on selective Cox-2 inhibitor NSAIDS;

3) focused consultation with patient safety groups to discuss risk minimization options regarding acetaminophen overdose and liver injury.

Canadian Examples of Patient Involvement

Patient Involvement Pilot Project (2014)

- Explored the value and feasibility of patient involvement in the orphan drug context as starting point for systematic, structured opportunities to inform benefit-risk assessment and management
- Simulated how input from patients, their caregivers, healthcare professionals and patient groups could be collected and incorporated in the drug submission review process.
- Online questionnaires were designed to gather qualitative information on the following (examples of one biologic and one pharmaceutical):
  - the impact on individual patient's quality of life;
  - experience with currently available therapies;
  - unmet medical need; and
  - the patient's level of risk tolerance

Results from the Pilot Project:

- Patient education on regulatory review and decision-making processes and reviewer training on when and how to best consider patient input in these processes is needed;
- Timing of when reviewers receive patient input is important;
- Additional experience needed.

Opportunities and Future Prospects

- Determining the best ways to elicit and consider patient input in a systematic manner and exploring the scope and nature of patient input of highest value.
- Assessing the overall suitability and feasibility of adopting, modifying or collaborating with other existing models such as those used by the FDA and EMA, and HTA bodies

Patient Involvement - Further Exploration

a) Who is best situated to provide input?
b) At what stage(s) in the regulatory process is it most feasible, or valuable, for patient input to be collected?
c) Is there information to enhance the regulator's understanding of patient drug experiences that could be gleaned from within data collected during clinical trials and submitted as part of the traditional data package?
d) What are the most appropriate and effective formats for patient input?
e) How should patient input be considered and captured in the regulatory assessment and decision-making processes?

And going forward…

- Following patient pilot HC contracted an expert in the field to analyse when and how to involve patients in drug review
- Several recommendations were put forward and are undergoing internal review
- Recommendations included the following:
  - Use phased approach to implement patient involvement
  - Larger pilot project to use publicly available information
    - Case by case approach in the pilot with based on clear criteria that are consistent with other regulators' approaches
  - Continue to involve patient members on advisory committees/expert advisory panels
  - Develop and establish principles, processes guidelines and training for affected HC personnel and for patient groups
  - Develop a communication framework and evaluation strategy

Thank you

???