Expert Patient Advocates & 21st Century Therapies Forum

Hyatt Regency Toronto
Toronto, Ontario
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Presented by:
Canadian Organization for Rare Disorders and Consumer Advocare Network
Statement of Purpose

There are increasing opportunities for patient engagement and input, thanks in part to strident patient advocacy and multiple stakeholder support as well as demonstrated benefits. But are patient advocates aware and capable of taking full advantage of these, from partnering in informed decision-making to shaping health policy. The purpose of the Canadian Expert Patients in Health Technology is to provide the knowledge, skills, and practical tools to empower patients to take an informed and effective role in assuring that there is sustained, equitable, and affordable access to the most appropriate health technologies for all Canadians, and that patients are central to defining, monitoring and evaluating appropriate use for individuals and the patient population collectively.

Impact

Patients accessing healthcare optimal to individual needs and values: medicines and other therapies, clinical trials and research programs, timely screening and diagnosis, and support to live as well as possible.

Ultimate Outcomes

1. Patient advocates better prepared to take advantage of opportunities for patient engagement and input from level of individual access to clinical trials research and health policy.
   a. Timely information about opportunities
   b. Skills, knowledge, and portals to engage
   c. Decision making capabilities at individual to policy levels
2. Patient advocates collectively engaged as partners in healthcare (to meet needs of patients)
   a. Expert Patients working collectively to propose strategic directions, influence health policy, and direct healthcare provision; strengthen health policy at national, provincial
   b. Expert Patients creating and participating in health boards, advisories, committees, decision making bodies, working groups, task forces
3. Expert Patients providing training and development to all stakeholders, including policy and decision makers, healthcare professionals, drug and devices industry, patient organizations, and other sectors (education, employment, housing, community services) to better serve patient needs
Activities

- Patient training workshops
- Patient tools and resources
I. Introduction to 21st century drug discovery and development

A. Topics: What are the innovations and breakthroughs in drugs being developed now and in the near future?
Hot topics beyond biologics: orphan drugs (for rare diseases), genetically targeted therapies (aka personalized medicines), combination therapies (sequential and simultaneous therapeutic protocols), innovative drug devices (inhaled, embedded, oral, continuous infusion), gene-modifying and replacement therapies (ostensibly a cure), cell modification and replacement therapies (next generation stem cell therapy), biosimilars (aka subsequent-entry biologics), repurposing (old) therapies to serve additional (unmet) needs (beyond expanded indications), and genome/exome sequencing (fast, comprehensive, affordable) available for screening, diagnosis, and prescribing.

B. Presentations on and by:
- Rx&D and “big” pharmaceutical companies (what they are bringing to Canadian patients)
- BIOTEC Canada and smaller biotechnology companies (what they are working on and how we can have more clinical trials and therapies in Canada)
- Genome Canada and CIHR (how partnerships across disciplines, borders, and with private industry are repurposing old drugs, developing new therapies and identifying “new” patients for old and new therapies)

C. Workshop: Dialogue with professional experts to improve “early access” to new therapies
- How can Canadian patients get involved in development and clinical trials for new therapies?
- What are advantages of a single clinical trial protocol and ethics review across multiple sites?
- How can Canadian patient registries facilitate patient engagement? How are electronic health records critical to access to clinical trials and new therapies?
- What are advantages of clinical trial experience in promoting early access?
- What can we do (now) to improve Canada as a preferred site for drug research, clinical trials, and early access?
II. Introduction to Changes in Regulatory Guidelines and Health Technology Assessment (global and Canadian)

A. Presentations:
How are regulatory guidelines and health technology assessment methods changing to meet the challenges of reviewing, monitoring, and assessing the safety, efficacy, and value of these new therapies? Or, are they?

- Health Canada: Bill C-17 and Orphan Drug Regulatory Framework; Harmonization/Convergence in Regulatory Standards and Guidelines; Adaptive Pathways

What are opportunities for Canadian patient organizations and patients engaged in the review of these innovative therapies at all levels and with all agencies (Health Canada, CADTH, panCanadian Pharmaceutical Alliance, provincial drug programs, private insurance programs)? How do patient submissions impact decision making on access to therapies?

- Health Canada patient input (pilot and beyond)
- CADTH (CDR/pCODR) written submissions and beyond
- Provincial Drug Review Programs

B. Workshop: Simulating Health Technology (Drug) Assessment Process
By simulating the process of a HTA committee, participants will experience how these committees decide whether to recommend a drug for funding, and what conditions to impose on drug access. Using scenarios based on real drug applications, participants are provided with data on safety, efficacy, cost-effectiveness, and value to patients. They work in small groups to discuss, deliberate, and subsequently arrive at a group decision on funding.

C. Workshop: Preparing Patient Input (with Impact)
Participants will be guided through a process for preparing patient input to a drug review process. Patient/public representatives from Drug Assessment committees will discuss how the committees function, how data are discussed, and how patient submissions fit into the process. These insights will be used to guide participants through an exercise on the steps of preparing a patient submission. Steps include: how to decide what information is relevant (and what is not), how to collect the information from the “right” patients and caregivers, what to do if there is no Canadian experience, how to synthesize the information to represent the breadth of the community, how to present information on issues relevant to reviewers, and how to (effectively) convey what is important to patients.
III. Challenges in Access to Innovative Therapies: Patient Registries and Managed Access Programs

A. Presentation and Panel Discussions (Policy makers, clinicians, industry, Drug Program Managers, patients)

Challenges: How are Canadian public and private healthcare providers assuring appropriate access to these innovative but often costly and resource-intensive therapies? Some therapies are approved and available for use with considerable “uncertainty” as to their long-term safety and effectiveness across patient populations beyond the clinical trials. Some are approved with requirements for patient enrollments in monitoring programs and registries.

Managed Access Programs: How do MAPs (aka Coverage with Evidence Development, “Start-Stop” Programs, and/or “risk-sharing” programs) provide access to drugs with “high uncertainty” for appropriate patients in real-world settings while collecting more data for re-analysis and guideline modifications? What are different types of MAPs and what has been the experience with these (in Canada and other jurisdictions)? What will be required to ensure healthcare providers and electronic health records are ready for these therapies? While many therapies may have long-term cost advantages in improved patient outcomes, many are costly and require investment of additional resources (before cost benefits can be achieved).

B. Workshop: Providing Input to MAPs

1. Case Study 1: Understanding MAPs
2. Case Study 2: Designing an MAP

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