Expert Patient Advocates & 21st Century Therapies Forum

Hyatt Regency Toronto
Toronto, Ontario
Nov 19 – 20, 2015
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
Day 1: 19 November, 2015

I. Introduction to 21st century drug discovery and development

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.

9:00 am – 10:30 am

Chair: Durhane Wong-Rieger, CORD

Panel: Innovation in Medicines

- Mark Lundie, Director, Medical Affairs, Rare Diseases, Pfizer Canada Inc. (Toronto)
- Frederic Lavoie, Head, Policy and Reimbursement, Oncology Business Unit, Novartis Pharmaceuticals Canada Inc. (Montreal)
- Cate McCready, Vice President External Affairs, BIOTECanada (Ottawa)
- Mehmood Alibhai, Director, National Policy & Market Access, Boehringer-Ingelheim Canada Ltd. (Vancouver) (tbc)
- Michael Reilly, Executive Director, Alliance for Safe Biologic Medicines (Washington DC)
- Suzanne White, Patient Requirements Research (Ottawa)

Questions for Discussion:

- How is “big” pharma engaging with patients to foster innovative drug discovery and development? And what they are bringing to Canadian patients)
• How are innovative biotechnology companies “changing the game” with targeted drug discovery and adaptive clinical trials? (What they are working on and how we can have more clinical trials and therapies in Canada)
• How researchers are partnering across disciplines, borders, and with private industry are repurposing old drugs, developing new therapies and identifying “new” patients for old and new therapies?
• What is emerging evidence about SEBs (biosimilars) as treatment options and what are the implications for inclusion and management in the future?

10:30 am – 10:45 am Break

II. Introduction to Changes in Regulatory Guidelines and Health Technology Assessment (global and Canadian)

Innovation Among the Regulators and Reviewers

Innovative medicines, especially those that address unmet needs or offer breakthroughs in terms of impact on clinical outcomes, quality of life, safety, or usability, are creating opportunities for previously untreated patients to access therapies, for reducing morbidity and mortality in debilitating and life-threatening conditions, and for providing targeted (precision) therapies that are both effective and tolerable. However, these therapies often face huge barriers in getting to patients, post-regulatory approval. The evidence may be based on novel, short, and small clinical trials and therefore considered as highly “uncertain” in predicting outcomes in real world use. They create challenges for traditional health technology assessment processes because they often have no or few comparable or alternative therapies and may be very costly on a per-patient basis. 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?

10:45 am – 12:00 pm

Chair: Wayne Critchley, Global Public Affairs

Panel: Innovation in Review, Assessment and Appraisal

• Barbara Sabourin, Director General, Therapeutic Products Directorate, Health Canada
• Cathy Parker, Director General, Biologics and Genetic Therapies Directorate, Health Canada
• Chander Sehgal, Director, Common Drug Review (CDR) & Optimal Use of Drugs, CADTH
• Jeffrey Biggs, Manager, Policy Development, Policy and Economic Analysis Branch, Patented Medicine Prices Review Board
Questions for Discussion:

• What are innovative processes at Health Canada based on Bill C-17 and Orphan Drug Regulatory Framework?
• What are opportunities for Harmonization/Convergence in Regulatory Standards and Guidelines across regulatory bodies?
• How is CADTH introducing both evolutions and revolutions in the Drug Review Processes to meet needs of innovative therapies, including targeted, orphan, complex, and biosimilars?
• What are options from the Patented Medicines Prices Review Board to address innovative therapies?
• How does the Life Cycle Approach allow for jointly Planned and Coordinated Clinical Trials Design, Regulatory Approval, Health Technology Assessment, Value-Based Pricing, and Real-World Monitoring/Data Collection? What about adaptive pathways?

12:00 pm to 1:00 pm Lunch

III. Patient Access to Therapies

Even though the overall budget impact of most of these innovative therapies is still very small within the overall drug plans, the numbers are increasing (much to patient delight and payers dismay). Payers, both private and public, are increasingly challenged to justify their place in drug plans. The challenges are not limited to Canada, and there are innovations emerging from the pressures experienced by HTA bodies and payers worldwide. What are, can, and should we be doing collectively to assure sustainable, appropriate access to innovative therapies for ALL patients?

1:00 pm – 2:45 pm

Chair: Bill Dempster, CEO of 3Sixty Public Affairs

Panel A: Challenges and Innovation in Canadian Access

• Updates in Public Payer Process: George Wyatt/Ferg Mills, Wyatt Health Management
• Overview of Private payer landscape, types of plans, trends in plan design and impact on access to medicine, how to advocate with private payers: Suzanne Lepage, Suzanne Lepage Consulting
• Private Payers and Access to Specialized Therapies: Stephen Frank, Vice President - Policy Development and Health, Canadian Life and Health Insurance Association (Toronto)
• Lisa Callaghan, AVP Product Group Benefits, Manulife (Toronto)

Panel B: Challenges and Innovation (broader context)

• Update on international access to innovative therapies: Neil Palmer, President & Principal Consultant, PDCI Market Access Inc.
• Patient support programs for public and private access patients: Sandra Anderson, VP, Consulting and Business Development Innomar Strategies
Questions for Discussion:

- What are the challenges in access and how are Canadian payers creating opportunities and innovations to address these concerns?
- What are innovations and adaptations taking place in international jurisdictions to address the challenges of providing sustainable access to innovative therapies?
- How can managed access programs, including adaptive pathways, help to promote appropriate access and to sustain affordable access and use?
- Are there programmes and processes for early access, such as Special Access Programme, Early Access Program, risk-sharing that are and should be part of innovative solutions?
- What are opportunities, strategies, and tools for patient engagement in development and clinical trials for new therapies?

2:45 pm – 3:00 pm Break

3:00 pm – 4:00 pm

Workshop: Evolving Best Practices for Canadian Access

4:00 pm Adjourn—Day 1

Day 2: 20 November, 2015

III. Challenges in Access to Innovative Therapies: Innovative Access Solutions

9:00 am – 9:45 am

A. HTA and Patient Input Solutions

This session provides a brief introduction to the basic principles and procedures of Health Technology Assessment, especially as it is practiced at CADTH, including the opportunities for patient input (submissions) to drugs under review.

- Brief introduction to HTA (CADTH)
- Provincial Drug Review Programs
- Patient Input Opportunities

9:45 am – 10:45 am

B. Workshop: Preparing Patient Submissions 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.

• **Inside Review Committees** (Maureen Smith, Patient Member, Ontario Committee to Evaluate Drugs)
• **Experiences from engagement with pCODR** (Deb Maskens, Kidney Cancer Canada)
• **Reality for “large” patient groups** (Seema Nagpal, Canadian Diabetes Association)
• **Challenges for Small Patient Groups** (Cathy Evanochko, Tuberous Sclerosis Canada)

10:45 am – 11:00 am Break

11:00 am – 12:00 pm

**C. Exercise in Preparing Patient Submissions**
This session takes patients through the process of preparing a patient submission, building upon the guide prepared by pCODR on how to collect and analyze patient feedback and a supplement guide prepared by the Consumer Advocare Network on the information needed to prepare a patient survey or interview as well as the information to be included in the submission.

12:00 pm – 1:00 pm Lunch

**D. Making Managed Access Accessible in Canada**
Everyone needs to be involved if Canada is to assure sustainable appropriate access to innovative medicines that are often life saving or life-altering but also costly and resource-intensive. We push for therapies to be approved and available for use as soon as possible but that often comes with considerable “uncertainty” in terms of the evidence as to their long-term safety and effectiveness across patient populations beyond the clinical trials. Some are approved with requirements for post-market surveillance and re-assessment after a specified period of time.

Managed Access Programs (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).

1:00 pm – 2:00 pm

Panel: Experiences with Managed Access in Canada (and How to Make MAPs Patient-Centric)

This panel will address the experience with Managed Access Programs in Canada. What are the types of MAPs that have been implemented in Canada? How have these MAPs been developed? What criteria are used and who is consulted? What are barriers and supportive factors? What have been the results?

- Frederic Lavoie, Head, Policy and Reimbursement, Oncology Business Unit, Novartis Pharmaceuticals Canada Inc.
- John Haslam, Country Manager, Canada, Alexion Pharmaceuticals Toronto, Canada Area Pharmaceuticals
- David Fortier, Pfizer Canada
- Deb Maskens, Kidney Cancer Canada
- Michael Eygenraam Vice-Chair, aHUS Canada
- John Adams, President and CEO, Canadian PKU and Allied Disorders
- Cathy Evanochko, Co-Chair, Tuberous Sclerosis Canada
- Sandra Anderson, Innomar Strategies

2:00 pm – 4:00 pm

D. Workshop: Providing Input to MAPs

A series of case studies based on MAPs that have been implemented in Canada will be presented, and participants will work in small groups to critique, especially in terms of patient impact. In addition, participants will be provided with information about a number of hypothetical drugs and the patient populations for which they are intended, with the goal of developing patient-appropriate MAPs, with requirements for implementation.

Click on link below to register.

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