"A unique approach to ..."

PATIENT PARTNERS LEADERSHIP TRAINING

Canadian Organization for Rare Disorders

WHO WILL BENEFIT?

THIS PROGRAM WILL ENHANCE THE CAPABILITIES OF:

- Patients and patient advocates to partner effectively throughout all phases of the drug lifecycle from drug development to real-world usage
- Patient relations personnel from various stakeholder groups involved in drug development and access, including primary research, development and testing, regulatory approval and monitoring, value assessment and reimbursement, and real-world management

WHY BECOME A PATIENT PARTNER LEADER?

Partnering with patients has transformed how drugs are discovered, developed, and made available. However, the pathway to effective patient partnership has been more serendipitous than strategic and more incrementally iterative than continuously progressive. Indeed, from the perspective of the patient as well the researcher, developer, regulatory, and funding stakeholders, the journey has been besieged with misunderstandings, missteps, mislearning, and missed opportunities. Nevertheless, we persevered and as each partner experienced the collective benefits of elevating the patient role, we have transitioned from a nexus of "patient focus" to "patient centricity" to "patient participation" to "patient engagement" to "patient involvement" and to where we feel we are now, that is, "patient partnership".

This program is not a blueprint for effective patient partnership. It is not a "step-by-step" guide to becoming a patient partner leader. It is not a compilation of learning or best practices

WHAT IT DOES OFFER

A series of structured opportunities, comprised of expert presentations, panel discussions, case studies, simulations, and **deliberate dialogues**, for patient advocates and other stakeholders to engage as equal partners in exploration, discovery, and creation of roles as leaders in a variety of patient partner situations.

WHAT WILL YOU GAIN FROM THIS PROGRAM?

PATIENTS AND PATIENT ADVOCATES WILL:

- Gain understanding of the drug lifecycle (discovery to real-world use) and how patients can partner throughout
- Practice skills for partnering within different levels and domains
  - how to share individual experience and needs to have a meaningful impact on other stakeholders
  - how to contribute to values (health technology) assessments
  - how to contribute to designing patient-centred provisional (managed) access programs
  - how to help develop useful and useable data collection mechanisms to monitor real-world drug use
- Experience (through simulations) the nuances of partnering in various settings and at different levels of decision-making and accountability

INDUSTRY REPRESENTATIVES WILL:

- Gain understanding how partnering with patients can help
  - Target drug development (needs and acceptability of products)
  - Improve clinical trials implementation (recruitment, design, retention, reporting)
  - Improve regulatory review process (patient risks-benefits trade-offs, unmet needs, urgency, and impact, patient-relevant outcomes)
  - Support input to values assessments processes that incorporate patient needs and impact
  - Support awareness and understanding of value of appropriate products to publics and decision makers
  - Educate patients and others to assure appropriate use and monitoring of products
- Practice skills, learn techniques, and discover tools and templates for “patient-centred“ partnering to
  - Gain insights from (inside) the perspectives of patients and patient advocates
  - Bring patient voice into regulatory approval process
  - Develop access programs that position organizational interests around patient interests, from conception to implementation to review
  - Appropriately support patient organizations in their awareness and advocacy activities to ensure patient independence and integrity
  - Identify opportunities for patient-partnered initiatives that will, for example, address gaps in services, promote integration, and improve family well-being

COURSE SYLLABUS

MODULE A
PARTNERING WITH PATIENTS FOR DRUG DISCOVERY AND DEVELOPMENT

Session 1 – Patient-industry partnerships in new drug discovery and development

- Roles of patients in each stage of drug lifecycle
- Contribution of patients to discovery of rare disease drugs
- Designing patient-industry partnerships "fit for purpose"

Session 2 – Patient partnerships for breakthrough and incremental therapies (Case of Hemophilia)

- Patient roles supporting breakthrough and incremental drug development
- Challenges/opportunities for disease-specific vs cross-disease partnerships
- Supporting patient-clinician informed decision-making on therapeutic choice

Session 3 – Designing Clinical Trials "Fit for Purpose" (Case of "Soft Bones")

- Innovative clinical trial design for innovative (rare disease) therapies
- Implications of distinct clinical trials for paediatric and adult populations
- Patient groups defining relevant "patient-relevant outcome measures"
- Patient advocacy as "art" and "science"

MODULE B
PATIENT PARTNERSHIP IN REVIEW PROCESSES

Session 4 – Patient engagement in regulatory processes

- Health Canada's review of drugs for rare diseases (relative to FDA and EMA)
- Patient partnership in regulatory reviews in other jurisdictions and opportunities for Canada
- Skills training for patient participation with regulatory agencies

Session 5 – Patient engagement in drug pricing and reimbursement (Case of CF)

- Cost of drug development
- Relationship between drug prices and access
- New Drug reimbursement pathway in Canada
- Role of PMPRB in setting "non-excessive" drug prices
- Patient advocacy and access

Session 6 – Patient Engagement in Health Technology Assessment (Case of SMA)

- Challenges and adaptations of HTA for innovative therapies
- Relevance of QALY's and ICER's to rare disease drug
- Patient input to HTA reviews: Quantitative and qualitative data
- Alternative approaches to "values-based" assessment for rare disease drugs

COURSE SYLLABUS

MODULE C
PATHWAYS TO APPROPRIATE ACCESS FOR RARE DISEASE DRUGS

Session 7 – Drug pricing and reimbursement challenges and solutions

- Pipeline of innovative therapies for rare diseases and common conditions
- Roles of multiple agencies in Canadian pathway to patient access
- International approaches to drug pricing and reimbursement
- Patient registries, real-world evidence, and post-market re-assessments

Session 8 – Managed access programs (Case of Fabry's Disease)

- Criteria for access (symptomatic, age-related)
- Research plan: registry, access criteria, dosage assessment, subgroup and long-term outcomes, expanded access criteria
- Patient compassionate access
- Patient group awareness and advocacy

Session 9 – Access to durable therapies: Case of gene therapy

- Characteristics of durable therapies (long-lasting, preventive)
- Types and applications across diseases and conditions (cell, gene replacement, gene editing)
- Issues: Outcomes, safety, permanence, ethics, cost, societal value
- Funding and financing
- Patient engagement, awareness, informed choice

MODULE D
REVIEW OF ACCESS TO NOVEL THERAPIES

Session 10 – Structured plans for managed access programs, comparison of types of durable therapies and potential

- Managed access programs for durable therapies
- Rare Disease Strategy for managed access: diagnosis, centres of expertise, therapeutic funding, community support, and research/monitoring
- Evaluation of impact, cost-effectiveness, and sustainability

HOW WILL LEARNING BE CARRIED OUT?

LOGISTICS

Platform: Virtual platform with interactive small group capabilities and storage of materials

Course Dates: From September 18, 2020 (includes a year-end holiday break) to February 5, 2021

Time and Frequency:

- Bi-weekly (every two weeks)
- 2-hour sessions live (on-line)
- 11:00 AM (EDT)/8:00 AM (PDT)

Course Format:

- Pre-work
- 2-hour live sessions
- Post-session discussions and written assignments

Leaders:

- Invited experts from multiple sectors
- Patient experts
- Facilitators

Materials:

- Videos
- Articles

FEES
(INCLUDING ALL ACCESS AND MATERIALS)

Regular: $3750 per participant

Government and academic affiliates: $750 per participant

Patients and patient groups: $250 per participant

CERTIFICATE OF COMPLETION

REGISTRATION

Spots are limited and applications will accepted to ensure a variety of affiliations and perspectives. To register, please submit an application through this **link.**