PATIENT PARTNERS LEADERSHIP TRAINING

- COURSE OVERVIEW-

MODULE A
PARTNERING WITH PATIENTS FOR DRUG DISCOVERY AND DEVELOPMENT

October 2, 2020

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”

Presenter:

Emil D. Kakkis, M.D, Ph.D., CEO, and President, Ultragenyx

October 16, 2020

Session 2 – Multiple Patient Roles in Transforming Treatment: 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

Presenters:

David Page, National Director of Health Policy, Canadian Hemophilia Society
Dr. Jerry Teitel, Medical Director, St. Michael's Hospital Hemophilia Treatment Centre

MODULE B
PATIENT PARTNERSHIP IN REVIEW PROCESSES

October 30, 2020

Session 3 – 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 "patient-relevant outcome measures"
- Patient advocacy as "art" and "science"

Presenters:

Dr. Leanne Ward, Children's Hospital of Eastern Ontario

Dr. Cheryl Rockman-Greenberg, Max Rady College of Medicine, University of Manitoba

November 13, 2020

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

Presenters:

Celia Lourenco, Director General, Health Canada

Alysha Croker, Manager, Office of Paediatrics and Patient Involvement, Health Canada

Daniel O'Connor, MHRA UK

Frauke Naumann-Winter, Bfarm

November 27, 2020

Session 5 – Drug Pricing and Reimbursement: Case of "Cystic Fibrosis"

- 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

Presenters:

Neil Palmer, PDCI Market Access

Andrea Souchen, SOBI

Lindy Forte, Patient Access Solutions

December 11, 2020

Session 6 – Patient Engagement in Health Technology Assessment for Rare Disease Therapies

- 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

Presenters:

Brent Fraser, CADTH
Sylvie Bouchard, INESSS
Josie Godfrey, JG Zebra Consulting (Former Associate Director, NICE)
Yvette Venable, ICER

MODULE C
PATHWAYS TO APPROPRIATE ACCESS FOR RARE DISEASE DRUGS

January 8, 2021

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

January 22, 2021

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

February 5, 2021

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

February 19, 2021
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

FEES
(INCLUDING ALL ACCESS AND MATERIALS)

Regular: $3750 per participant
Government and academic affiliates: $750 per participant
Patients and patient groups: $250 per participant

REGISTRATION INFORMATION

Spots are limited and applications will accepted to ensure a variety of affiliations and perspectives. To register, please submit an application through this **link.**
Registration deadline: September 25, 2020