# Enpr-EMA International Collaboration Working Group

## Environmental Scan

OCTOBER 14TH 2019

# Objectives of Environmental Scan

- Compare the pediatric regulatory and ethics clinical trial requirements, submission and review processes in Australia, Canada, EU, Japan and the US:

  a. to assist investigators and industry involved in conducting these studies and

  b. to identify regulatory challenges in conducting these trials on an international scale.

- Compare the ethics and other requirements and process for participation as a pediatric clinical trial site among the above jurisdictions.
- Environmental Scan was an open survey with a list of questions to be answered by both the regulators and the networks

# Jurisdictions

- Regulators
  - USA: Food and Drug Administration (FDA)
  - EU: European Medicines Agency (EMA)
  - Australia: Therapeutic Goods Administration (TGA)
  - Japan: Pharmaceuticals and Medical Devices Agency (PMDA)
  - Canada: Health Canada
- Networks
  - USA: Institute for Advanced Clinical Trials for Children (i-ACT) and Pediatric Trials Network (PTN)
  - EU: European Network of Pediatric Research at the European Medicines Agency (Enpr-EMA)
  - Australia: Australian Pediatric Trials Network (PTN)
  - Japan: Pharmaceuticals and Medical Devices Agency (PMDA)
  - Canada: Maternal Infant Child & Youth Research Network (MICYRN)

# Definitions

- **Clinical Trial Application (CTA)**: comprehensive application submitted to the regulatory authority with the purpose to conduct a proposed clinical trial using an investigational medicinal product/medical device. Other terms for this application include *Investigational New Drug Application (IND)*.
- **Investigational Status:** status of a medicinal product/device that is not yet approved for use or is being tested outside of the intended indication/patient population (off-label use) AND is currently under investigation in a clinical trial.
- **Jurisdiction**: a country, state or other area where a particular set of laws or rules governing the conduct of a clinical trial must be obeyed.
- **Sponsor:** An individual, body, or organization that conducts a clinical trial complying with the obligations as set out by the regulations of the jurisdiction.

# Environmental Scan-Clinical Trial Applications and Ethics review

- Environmental Scan is divided into 4 categories
  1. Pediatric Clinical Trial Regulatory Requirements and Incentives
  2. Pediatric Clinical Trial Submission Process
  3. Pediatric Clinical Trial Review Process
  4. Ethics and Other Requirements and Processes for Participation as a Pediatric Clinical Trial Site

# Environmental Scan-Clinical Trial Applications and Ethics review

- Version for completion was dated 31 May 2019
- Regulators to complete categories 1-3 related to the regulatory pathways for clinical trial applications
- Networks to complete category 4 related to the ethics component and intermittent questions highlighted in categories 1-3

# Environmental Scan-Clinical Trial Applications and Ethics review

- Categories 1-3, Regulatory Pathways of CTAs
  - Complete information was provided by 2/5 jurisdictions
- Category 4, Ethics Component
  - Complete information was provided by 4/5 jurisdictions

# Environmental Scan-Clinical Trial Applications and Ethics review

- Challenges with compiling data:
  - Some jurisdictions provided links to where information can be found rather than answering questions directly
  - References were not provided for many of the jurisdictions
  - Clarification and confirmation may be required with individual jurisdictions

# Environmental Scan-Clinical Trial Applications and Ethics review

- Next Steps:
  - Retrieve information from missing jurisdictions
  - Compile outstanding data
  - Explore potential synergies with the work done by the Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard

# Enpr-EMA International Collaboration Working Group

- Jean Temeck, Susan McCune (FDA),
- Collin Hovinga (i-ACT), Christoph Hornick (PTN)
- Michiyo Sakiyama, Junko Sato (PMDA),
- Hidefumi Nakamura (NCCHD),
- Pauline Kerr, Agnes Klein, Barbara Njie (HC),
- Thierry Lacaze, co-Chair, Breanne Stewart (MICYRN)
- Dirk Mentzer, Koen Norga, Irmgard Eichler, Gunter Egger (Co-chair) (EMA)
- Mark Turner (Enpr-EMA)
- Andrew Davidson (Australian PTN)
- Neil Everest, Monique Stone (TGA)