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Enpr-EMA Meeting
Disclaimer

• The opinions contained are those of the authors and are not intended to represent the position of Brigham and Women's Hospital or Harvard University.

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• I have no personal conflicts of interest with this presentation.
Agenda

• Introduction to MRCT Center
• Conception of the “Pediatrics” Project
• Scope, to be refined
• Opportunities for collaboration
The MRCT Center is a research and policy center focused on addressing the conduct, oversight, ethics and regulatory environment for clinical trials.

Vision

Improve the integrity, safety, and rigor of global clinical trials.

- Academic credibility
- Trusted collaborator
- Independent convener
Engage diverse stakeholders to define emerging issues in global clinical trials and to create and implement ethical, actionable, and practical solutions.

MRCT Center is affiliated with BWH and Harvard University
MRCT Center Team

• **Leadership:**
  – Barbara Bierer, Faculty Director
  – Mark Barnes, Faculty Co-Director; Partner, Ropes & Gray, LLP
  – Sarah White, Executive Director

• **Senior Advisors:**
  – Elizabeth Cahn, Program Coordinator, Cancer Connection
  – Luke Gelinas, Chairperson, Advarra
  – Rebecca Li, Executive Director, Vivli
  – David Peloquin, Associate, Ropes & Gray LLC
  – Stephen Sonstein, Professor Emeritus, Eastern Michigan University
  – David Strauss, Columbia University

• **Faculty:**
  – Deborah Zarin, Program Director, MRCT Center; Harvard Medical School

• **Staff:**
  – Carmen Aldinger, Administrative and Training Manager
  – Hayat Ahmed, Project Manager
  – Sylvia Baedorf Kassis, Program Manager
  – Jen Ewing, Communications Specialist
  – Elisa Koppelman, Program Manager
  – Linda McMaster, Administrative Assistant
  – Laura Meloney, Program Manager
  – Walker Morrell, Project Manager

• **Student Researchers:**
  – Joshua Smith-Sreen
Projects:

- Prioritized by EC/SC committee and EAB
- All projects are international
- All lead by 2 or more representatives of different stakeholder groups
- Recommendations apply to all stakeholders (academic, industry, etc.)
- Must have practical deliverables
- Not a formal consensus methodology but to date, no dissent or minority opinion
Promoting Global Clinical Research in Children:
Aligning ethical, pragmatic and regulatory approaches

Project Leadership:
Enrica Alteri, MD
Barbara E. Bierer, MD
Steven Joffe, MD, MPH
Robert “Skip” Nelson, MD, PhD

Project manager:
Lisa Koppelman, MSW, MPH
For this audience, I will not review background, nature of the challenges, data to support approach, need, or theory.
Organizations & individuals engaged to date

• Currently 63 members on the workgroup.
• Expert individual and group calls conducted to date representing:
  – Regulatory
  – Academic Medical Center
  – Industry
  – Trade and Professional Associations
  – Research Institutes & Not-for-Profits
  – Parents, Patients (mainly adolescents) and Patient Advocacy Groups

• Essence of success is collaboration, leveraging and building upon the work of others, joining forces for the common good
Contextual Issues for Consideration and need for focus

• Nature of intervention:
  – Product development: drugs, biologics, vaccines, devices, behavioral interventions, technology initiatives
  – Post-approval studies: comparative effectiveness, observational/RWE studies

• Age span:
  – Neonates, infants, young children, adolescents
  – Different physiology, disease type, and disease burden

• Rare vs common diseases

• Location
  – Community vs academic sites
  – LMIC vs HIC
Current and Ongoing Analyses

- **Legal Landscape Analysis**: to understand and assess the current global landscape of pediatric research regulations, with a focus on illuminating legislative and regulatory gaps and inconsistencies

- **Ethical Guidance Analysis**: to identify and characterize existing ethical considerations broadly and in a geographic/culturally specific manner

- **Connecting with organizations with overlapping remit or activities**: iACT for Children, Empr-EMA, iCAN, others

- **Background review of legislative “incentives” for pediatric studies** (not as focus or direction, although)

- **Now refining topics and scope**
Preliminary Project Objectives

1. To understand and assess the current global landscape of pediatric research regulations, with a focus on illuminating legislative and regulatory gaps and inconsistencies.

2. To describe existing initiatives related to pediatric clinical research and drug development, including efforts to harmonize ethical and pragmatic approaches in pediatric clinical research.

3. To identify and characterize the relevant ethical considerations in designing, recruiting, conducting, and reporting multi-regional pediatric trials and to distinguish between widely shared ethical considerations & those that are culturally and/or geographically specific.

4. To define and address pragmatic barriers to conducting pediatric trials.

5. To recommend harmonized approaches to regulating pediatric research around the globe.
Project Deliverables to Consider

- Descriptive document incorporating the legal landscape analysis of regulations and guidance governing pediatric research
- Descriptive document detailing existing ethical foundations and guidance related to pediatric research
- Roadmap for harmonizing regulatory frameworks for pediatric research that allows for context-specific implementation
- Recommendations for new or revised guidance or regulations on research in children
- Resources, tools, and best practices to address common challenges in the conduct of pediatric trials.
- Educational resources for children and families on participation in clinical research
- Other

Collaboration is key to progress.
Thank you 
and
Discussion