Proposed

Global Pediatric Clinical Research Network

Overview

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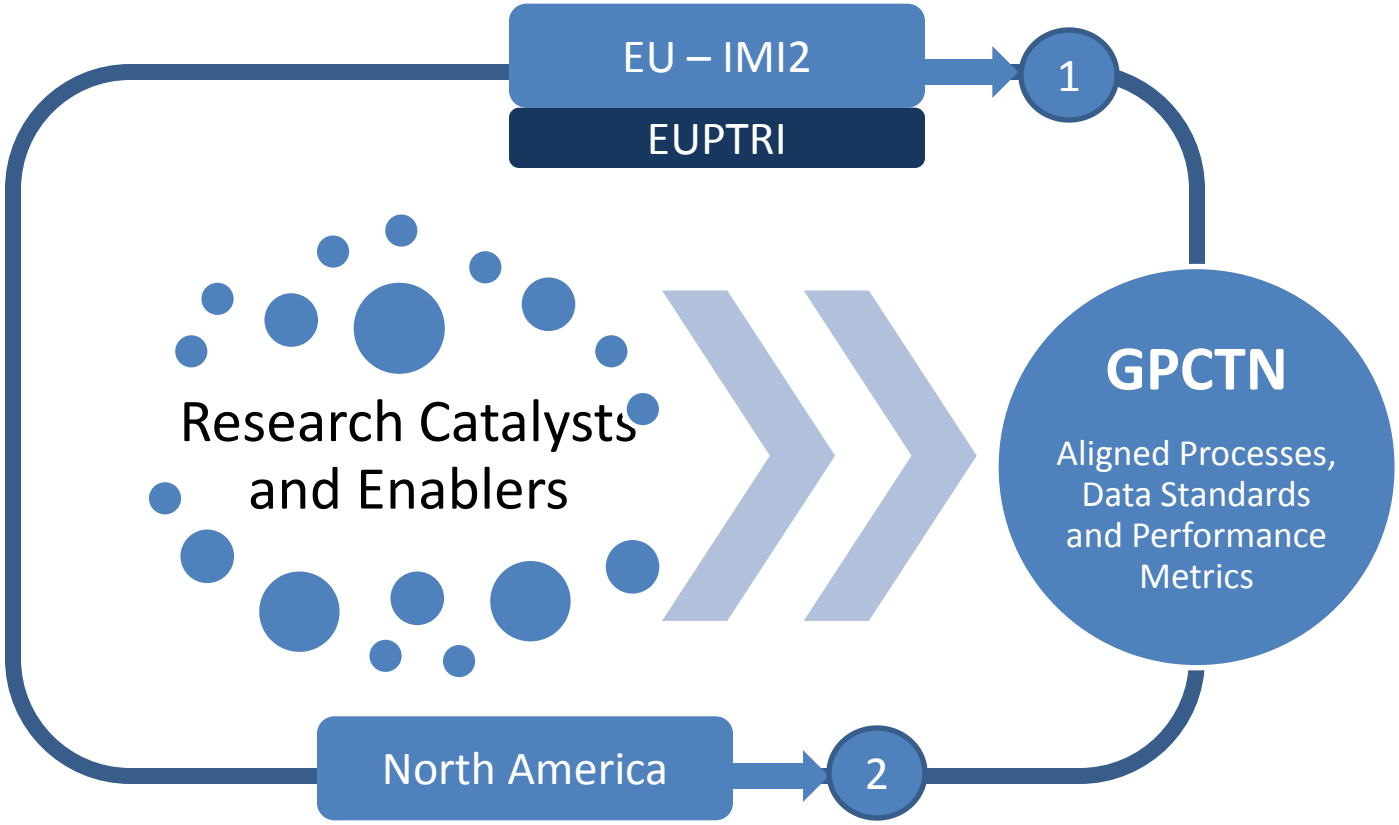
Concept of a Global Pediatric Clinical Trials Network

- Initial funding from a public-private partnership (industry, public/ private research institutions, patient groups)
- Disease-agnostic; phase I-IV; neonatal-adolescence
- Global: US, Canada, EU and beyond
- 50-100 of the best children's medical centers in the world
- Heterogeneous study sponsors including industry, NIH (government research), private foundations, patient advocacy groups, investigator initiated
- Cooperate and partner with existing networks, patient groups, medical societies, foundations
- Global resource for advancing science of pediatric drug development and advocating for sound regulatory policy

What is Different?

- Primary focus development and approval of new therapies; comparative evaluation of existing therapies
- Real (not virtual), global (not local or national) network
- Disease-agnostic, not disease-specific
- All age groups (including neonates)
- All phases (I-IV, registries)
- **Sustainable infrastructure**
  - Governing board (industry;academia; government research institutions)
  - Independent corporate non-profit entity with full time FTEs to operationalize and manage the network
  - Financial support of on-site employees in clinical trials units; and of site PI champions to foster long-term community of investigators
- **Sustainable Funding**
  - Founding partner initial capitalization
  - Subsequent additional partner's buy-in
  - Partner yearly dues (including non-industry partners and partnering sites)
  - Ad-hoc fees to non-partner companies utilizing services for specific studies
  - Ad-hoc fees to patient advocacy groups/foundations utilizing network
  - Grants from NIH, other government, foundation sponsors

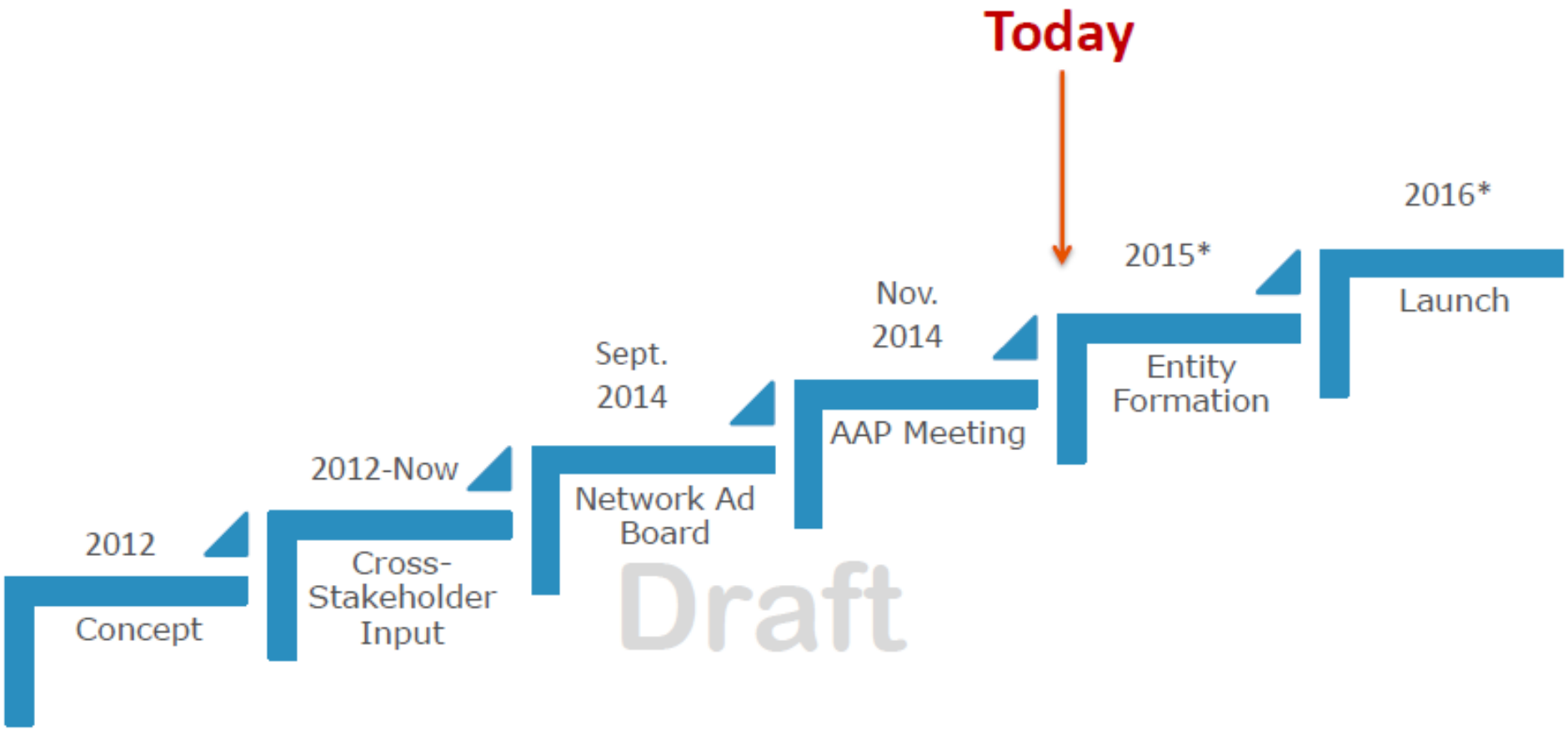
Parallel Initiatives Toward the Creation of a Global Pediatric Clinical Trials Network



Research Catalysts and Enablers include:

- International Neonatal Consortium
- GRiP
- Specialty Networks

Path Forward



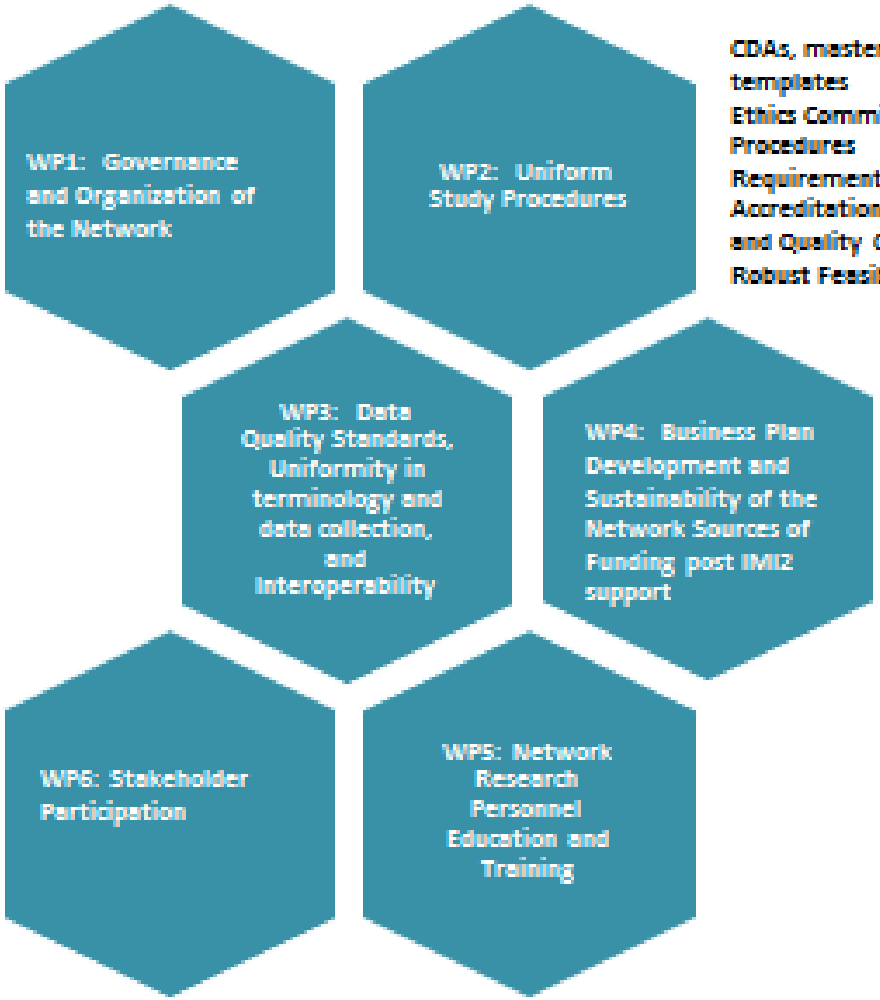
*goals

Companies and Organizations Represented at the IMI2 Pediatric Clinical Trials Network Meeting on March 5, 2015

- Lilly
- Pfizer
- Roche
- Janssen (J&J)
- Glaxo-Smith-Kline
- Astra-Zeneca
- Boehringer-Ingelheim
- Lundbeck
- Bayer
- Grunenthal
- Novartis
- Bristol-Myers-Squibb
- UCB
- Novonordisk
- Servier
- EFPIA
- EnprEMA
- IMI2-Europe

Proposed Work Packages

Central network organization
Procedure for access to the EUPCTN
Clinical Advisory Groups
Liaison with member state governments, national hub sites
Three proof of concept clinical trials



CDAs, master contracts, budget templates
Ethics Committee Liaisons and Procedures
Requirements for Sites and Accreditation, Performance Metrics, and Quality Control
Robust Feasibility Assessment

Patient/Parent Group Participation
Liaison with EnprEMA
National pediatric networks, and disease specific EU networks
Clinical trial infrastructure networks (ECRIN, TEDDY, GRiP)

Scorecard of EU "Interest" in EUPCTRI (EUPCTN) (14/28 Member States)

- 4 countries pledged national network money (England, Switzerland, Austria, Spain)
- 4 Ministries of Health very interested (Italy, Poland, Albania, Netherlands)
- 2 countries with strong networks interested in alignment (Ireland, Finland)
- 4 countries with developing networks interested in alignment (Norway, Iceland, Estonia, Sweden)
- 4 multinational subspecialty networks (Rheumatology, HIV/ID, Oncology, CF)

EU Process for GPCTN: IMI 2 project life cycle