European Federation of Pharmaceutical Industries and Associations

Update on the IMI2 Proposal to Build a Sustainable pan-European Paediatric Clinical Trials Network

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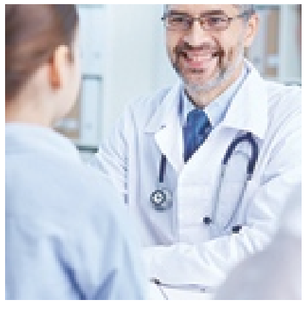

* On behalf of the EFPIA Consortium

What's the Solution?: Build a Sustainable Pediatric Clinical Trials Network

- Use the IMI2 PPP process to build a pan-EU network
- 10 companies from EFPIA contributing in-kind expertise and studies: (**Janssen, Novartis, Bayer, GSK, Roche, Pfizer, Lilly, Sanofi/Genzyme, Servier, UCB)**
- Multi-specialty capable, pediatric (neonates to adolescents) phase 1-4 clinical trials network devoted to planning and executing both industry-sponsored and non-industry sponsored studies
- Work with existing national and disease-specific networks in the EU, and apply for Enpr-EMA membership
- Central organization, plus national hubs and clinical sites throughout the member states of the EU
- Sustainable business model after initial 6 years of public/private funding

Multi-stakeholder attendance/support for the IMI2 Project at an April 5, 2016 Workshop Sponsored by EU Commission

Companies

Janssen, Bayer, Novartis, Lilly, GSK, Pfizer, Roche, Sanofi/Genzyme, Servier, UCB

EFPIA Consortium

(Heidrun Hildebrand, Bayer)

Research and Clinical National and Disease-specialty Networks

GRiP, ECRIN, EnprEMA, PENTA, PRINTO, FINPEDMED, Canada, PTC/Critical Path

Barcelona, Warsaw Childrens Hospitals, Hungarian CRO

EU CROs, SMEs, large children's hospitals

Enpr-EMA PDCO

Health Authority

European Genetic Alliance
European Rare Diseases Alliance

Patient advocacy groups

Published Minutes from Workshop

- The EUPCTN initiative was welcomed as a solution to overcome formidable planning and operational challenges inherent in conducting paediatric clinical trials; and as a means to make Europe a competitive region for delivering high quality trials.
- Recommended that such a network:
  - Be open and serve both public and private interests
  - Integrate with current good models of national and disease-specific networks
  - Include components which help to create new innovative ways of designing / conducting pediatric clinical trials
  - Provide education, uniform training, and enhance national and site-specific capacity building
  - Immediately start work on a business model for sustainability beyond the period of IMI2 support including collaboration with national health systems
  - Ensure interoperability with other ex-EU networks to foster the ability to conduct global clinical trials

pan-European Paediatric Clinical Trials Network

Overview Key Deliverables and proposed Work Packages

- Central network organization
- Liaison with member state governments, national hub sites, other EU initiatives
- Clinical Advisory Groups

- Development of a transparent process to select/accept studies to be run by the network, incl. initial studies aiming to test the validity of the net-work
- Development of operational implementation plan to execute the study of a new drug
- Development and implementation of performance metrics to measure site and network performance

- CDAs, master contracts, budget templates
- Ethics Committee (ICF, Assent) Liaisons and Procedures
- Requirements for Sites and Accreditation, Performance Metrics, and Quality Control
- Define and utilize available tools for Robust Feasibility Assessment

Industry Contribution

- **!3 FTE/yr equivalents with multiple categories of expertise:**

  clinical, clinical operations, clinical pharmacology, program management, legal-contracts, regulatory, IT, business development

- **3-5 Industry-sponsored studies for regulatory drug approval**

Further Insights: What Can the EC Support?

- All tasks of the Consortium that are directed to setting up, organizing (including sites recruitment), governing, managing, training, and evaluating the network
- Tasks directed toward forming collaborations with regulators, existing national, disease-specific and other networks, SMEs, and patient advocacy organizations
- Tasks at the National Hub sites to organize the network within member countries
- Tasks at sites within the member countries to bring their training and operational performance up to regulatory grade standards for the execution of trials and certification of sites
- Activities directed toward building sustainability including relationships with member-state governments.
- Activities to develop innovative approaches to planning and executing paediatric clinical trials
- What the EUC **cannot** support: Paying salaries of people at sites who are “on standby” waiting for the throughput of clinical trials

Comparative Cost/Benefit Analysis for this Public-Private Partnership to Foster Better Medicines for Children

EFPIA Industry Consortium

- In-kind cost; 67.5 M Euros
- Benefits:
  - Pan-EU pediatric network (50-100 sites in 15-25 countries)
  - Multi-specialty, multi-phase, neonate-adolescent capable
  - Cost savings from faster, cheaper studies
  - Increased success at completing studies (PIPs)
  - Greater collaboration with academia, patient groups, regulators

IMI2/EU Commission

- Cost=67.5 M Euros
- Benefits:
  - Create infrastructure for pan-EU self-sustained network (enhance EU competitiveness)
  - Multi-specialty, multi-phase, neonate-adolescent capable
  - Non-industry sponsored clinical trials (IIS, SMEs, off-patent)
  - Early consultation of academia, regulators with industry on prioritization of studies, feasibility, innovative trial design

Timeline for Call Topic 10

	Call 10
	2016
Consultation period with SRG, SC, EC	01/09 - 21/09/16
Topic finalisation following consultation	
Feedback to SRG,SC, EC	
Updated indicative text published on IMI website	
IMI GB written procedure	03/10-18/10/16
Launch of Call	**19/10/2016**
Submission deadline SP	24/01/2017
Board approval SPs	1-16/03/17
Submission FPP	13/06/2017
Board approval FPP	19/07-3/08/16
GA signed	Sept-Oct 17

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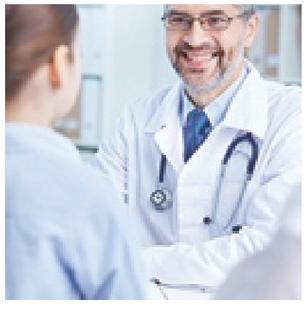
- Thank you for your attention





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