

# Creation of a pan-European Paediatric Clinical Trials Network

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# Need for public-private collaboration

- Due to European Regulation, the number of children to be involved in clinical trials is constantly growing
  - Paediatric research infrastructure not developing at the same pace
  - Increased competition between studies for shared resources (Investigators, sites, patients)
  - Delay in availability of innovative medicines to children
  - Negative impact on academic research
- The success of many paediatric clinical trials depends on a public private partnership between multiple stakeholders including private sponsors and publicly funded organisations
- Proposed solution:
  - European initiative that promotes the rapid delivery of paediatric drug trials through improved uniform processes in a coordinated, sustainable network that is widely accessible

# Scope of the proposal

- The overall vision of this proposal is to create a large collaborative paediatric network that will facilitate the development and availability of new drugs and other therapies, and the expansion of knowledge about drugs currently used in the paediatric population.
- This will be accomplished by not only advising on how best to do the necessary research, but by actually building sufficient infrastructure and best practices to support planning, running, and completion of all types of clinical studies (phase I-IV) by all kinds of sponsors (industry and non-industry)

# Objectives of the full project (1/2)

- Building a network with a lean central coordinating organisation, arranged around 'national hub coordinating centres' cooperating with multiple sites within each EU member state
- Installing scientific advice and trial readiness groups to consult with sponsors on the scientific soundness and feasibility of their proposals and to drive innovation.
- Develop and implement standardised processes, procedures, and performance metrics for initiation and execution of studies and maintenance of high-level performance across the network



# Objectives of the full project (2/2)

- Testing viability of the network by measuring performance metrics during the execution and completion of a number of different clinical studies (phase I-IV) from different sponsors (industry, non-industry) and different therapeutic areas, across all age groups
- Develop and implement a business model that provides sustainability of the network after the period of IMI2 funding
- Preparing the network to become a member of the European Network for Paediatric Research at the European Medicines Agency (Enpr-EMA)

# Expected impact

- Access for paediatric patients to new experimental therapies in well-designed clinical trials
- The improved efficiency in executing trials (reduced timelines and reduced cost)
- Improved data quality and faster development of next generation medicines for children
- Enhancing the role of clinicians and patient/parent advocacy groups in planning and designing studies
- Broadening the access of academic medical centres and clinical faculty across Europe to new experimental therapies for multiple clinical indications
- Contribution to regulatory policy around feasibility, innovative study design, meaningful endpoints, and risk-benefit assessment

# Industry consortium, duration & budget

- Janssen (lead)
  - Bayer (co-lead)
  - Novartis
  - Pfizer
  - Lilly
  - GlaxoSmithKline
  - Roche
  - Servier
  - Sanofi/Genzyme
  - UCB
- Indicative duration: 72 months
  - The indicative EFPIA in-kind contribution will be in the range of EUR 67MM
  - Financial contribution for IMI2 will be also in the range of 67 M EUR

# Suggested architecture of the project

- The suggested work packages for this project would be:
  1. Project Management and Oversight of IMI project
  2. Organisation and Governance of the pan European Paediatric Clinical Trials Network
  3. Business Plan Development, Expansion of the Network, and Sustainability of the Network Sources of Funding post IMI2 support
  4. Scientific Advice, Feasibility and Innovation
  5. Data Coordinating Centre and Data Quality Standards
  6. Network Research Personnel Education and Training
  7. Planning and Execution of Clinical Trials

# Expected contributions of the applicants (1/2)

- Experience and know-how in conducting paediatric clinical trials (incl. clinical operations and clinical program management)
- Expertise in paediatric drug development (incl. clinical pharmacology, modelling and simulation, extrapolation, regulatory science, statistical methods, epidemiology and use of clinical databases, study design and ethical considerations)
- Access to a large paediatric population
- Physicians and other health care providers covering a wide array of clinical paediatric subspecialties
- Patient/parent organisations
- Experience in working with the use of standardised procedures and processes in all clinical trials
- Information technology / data management

# Expected contributions of the applicants (2/2)

- Expertise in legal and clinical compliance / ICH GCP (International Council for Harmonization - Good Clinical Practice) aspects
- Strong project management and communication expertise
- Office administration and website management
  
- Organisations in scope:
  - Existing regional or national networks;
  - Pan-EU disease-specialty networks;
  - Large children's hospitals and medical centres;
  - Small-medium sized entities
  - Patient/parent organisations

# Expected (in kind) contributions of industry consortium (1/2)

- Program management
- Clinical trial design including adaptive design and the use of modelling/simulation,
- Clinicians, clinical pharmacologists, and clinical scientists
- Clinicians for communication, on-site visits, and other interactions with academic medical centres, investigators, and advisory boards
- Information technology/ data management expertise to co-lead the central network data coordinating centre, co-maintain the central organization website
- Regulatory expertise
- Clinical operations

# Expected (in kind) contributions of industry consortium (2/2)

- Business planning and development; contractual agreements
- Financial planning and implementation
- Legal counselling
- Industry-sponsored clinical trials to test the viability of the network

# Key deliverables of the full project

- Establish the structure and governance of the project
- Set up and maintain groups of scientific experts to trigger innovation in the development of paediatric therapies
- Implement standing disease or condition-focused network clinical advisory groups
- Develop and implement standardised processes, procedures, and performance metrics
- Test the readiness of the network for multiple diverse clinical trials
- Build and expand the clinical trials infrastructure across the EU
- Develop a business model and funding mechanism viable after the initial period of IMI2 support
- Build a process to open the network for submission of studies from all kinds of sponsors

# Questions?

Contact the IMI Programme Office  
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