Gianni Benzi Pharmacological Research Foundation

VII Foresight Training Course

In the context of

NEX-T-WORK

European Projects Forum

The European network for paediatric research

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Types of Paediatric networks (examples)

• **Clinical Trials networks:**
  - National networks (MCRN, FinPedMed, CICPed, PaedNet etc)
  - Disease specific networks (PENTA, PRINTO, Oncology, CF etc)
  - Age specific networks (TINN, Euroneonet etc)
  - Trial/drug/project specific networks (Neomero, DEEP, TINN, GAPP, NeoVanc etc)
  - *(Pregnant women)*

• **General paediatric networks:**
  - TEDDY
  - GRIP
  - Scientific societies

• **Observational study networks (pharmacoepidemiology)**
  - Research database (pedianet, GPRD, IMCI, etc)
  - Antibiotics (ARPEC)
  - Cohort studies (disease specific)

• **EMA network of the networks:**
  - Enpr-EMA
MCRN

MCRN is one of many national networks in the UK that cover all of the country and all areas of clinical medicine

• Coordinating Centre
  – Performance manage local networks and trials at risk

• Clinical Studies Groups
  – 13 clinical areas
  – Proactive approach to portfolio (including PIP development)

• Local Research Networks
  – Support trials and investigators
  – Research costs are met from grants / industry and not from the network
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Paediatric European Network Treatment AIDS (PENTA)

85 sites from 21 countries

MRC CTU, London:
Austria, Finland, Germany, Ireland, Italy, Netherlands, Sweden, UK
Brazil, Thailand
Uganda, South Africa

INSERM SC10, Paris:
Belgium, Denmark, France, Portugal, Poland, Romania, Spain, Switzerland
Argentina
HIV related activities

PENT/ECs network

Clinical Trial Network
- PENTA trials
- NEAT

Cohorts:
- ECS
- Eurocord
- Cohort coll.
- Postmark surv.

Pregnancy
- ECS
- PANNA/PK
- Toxicity
- etc

Training:
- tr@inforPedHIV
- RLS training
- ACTIVATE

Biobank
Pharmacology Ctee
Vir\-immuno Ctee
Clinical Trial Centres
IT support team
Statistical team
Training evaluation team
“...to foster, facilitate, and conduct high quality research in the field of paediatric rheumatology...”

Italy, May 1996

PRINTO bylaws
PRINTO bottom up approach

- Standardized criteria to evaluate response to therapy in JIA, JSLE and JDM
  - ACR pediatric criteria in JIA (FDA, EMEA, ACR)
- Non for profit clinical trials (JIA, JDM, JSLE)
- Standardised information to families
- Training to young researchers
- Service facility for PRINTO members

- Main source of funding European Union, AIFA
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TEDDY is a Network of Excellence funded under the 6th Framework Program, encompassing 19 Research Centres and 10 European Countries and Israel and cooperating with more than 200 researchers and experts.

Aimed at increasing the availability of paediatric safe and efficacious drugs.
General objectives

To facilitate the development and promote the availability of medicines for children by reducing the fragmentation of ongoing efforts in relevant fields of research. In addition, GRIP aims to create consensus on international standards, methodologies and tools for paediatric research.

The day-to-day work of the consortium will primarily focus on:

> The development of a Paediatric Clinical Pharmacology Training Program;
> Filling important “gaps” in paediatric medicines research by validation and harmonisation of research tools specific for paediatrics;
> Sharing of strategies and plans;
The "Global Research in Paediatrics – Network of Excellence (GRiP)" is funded by the European Union under the **Seventh Framework Programme**. The funding started on 1 January 2011 and is expected to last until 31 December 2015.
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ARPEC

Prof. Mike Sharland, St George’s University of London
Possible paediatric networks Interactions

Complementary and support activities

- Enpr-EMA
- Teddy/GRIP
- PMS studies
Paediatric European Network Treatment Infection (PENTi): Proposed governance and management structure

TEDDY/GRIP:
- Training
- Pharmacoepidemiology
- CT methodology
- Ethics
- Regulatory
- Health technology assess

PENTA network/Foundation:
- Trial management
- Administration/PM
- Statistical support
- PID, Viro/Immuno expertise
- Project management
- Funding application
- Legal entity
- etc

ESPID:
- Scientific endorsement
- Scientific input
- PID expertise

TINN:
- Neonatal network

EMA:
- Enpre-EMA
- PDCO

PENTi /PENTAid
- Definition research priorities
- Coordination research activities
- Liaison with EMA/PDCO
- Liaison with ESPID and PENTA
- Budget definition
- Networking
- Dissemination of the results
- etc

Neomero SC²
ARPEC SC
TINN
Trial/study B SC
European Network of Paediatric Research at the EMA (Enpr-EMA)

Recital 33 and Article 44 of the European Paediatric Regulation (EC) No 1901/2006², as amended, states: “Clinical trials in the paediatric population may require specific expertise, specific methodology and, in some cases, specific facilities and should be carried out by appropriately trained investigators. A network, which links existing national and Community initiatives and study centres ......, would help facilitate cooperation and avoid unnecessary duplication of studies. This network should contribute to the work of strengthening the foundations of the European Research Area in the context of Community Framework Programmes ...., benefit the paediatric population and provide a source of information and expertise for industry” and “The Agency (i.e., the European Medicines Agency, EMA) shall, with the scientific support of the Paediatric Committee, develop a European network of existing national and European networks, investigators and centres with specific expertise in the performance of studies in the paediatric population”.
Key operational goals

- To link together existing networks
- To provide expertise and access to infrastructure for industry to conduct studies in children
- To define consistent and transparent quality standards
- To harmonise clinical trial procedures
- To define strategies for resolving major challenges
- To communicate with external stakeholders
EMPREMA is important for EU paediatric NETWORKS

EMPREMA

DEMAND

OFFERING

Chosen by the Companies

Driven by PDCO

NETWORKS

deriving from new EU Regulation

PIP – paediatric studies
Workshop on PIPs for medicines to treat Type 2 Diabetes Mellitus

- Participants: regulators, experts, industry
- To discuss feasibility and better alignment with clinical practice and medical needs:
  - Few available paediatric T2DM patients
  - Many simultaneous, competing paediatric drug developments in T2DM
  - Few consortia/networks of specialised centres to facilitate recruitment of patients
Workshop Type 2 Diabetes Mellitus

Potential solutions discussed

- Staggered development of products from the same class, by means of different lengths of deferrals

- Multi-company, multi-agent study for products being developed concurrently: need for only one control group

- Single-company, multiple-agent study: need for only one control group.

- Simplified PK studies (peak and trough levels, dried blood spots)

- Potential broadened role for extrapolation (efficacy)

- Establishment of an Enpr-EMA diabetes/endocrinology network