

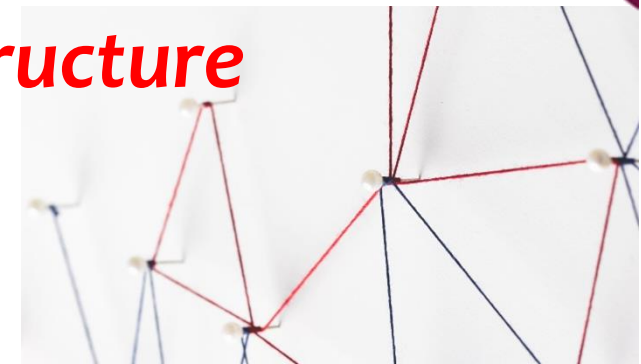
## Question:

We know that networks are giving an important contribution to clinical research.

Which is instead the role of a research infrastructure and what RIs can provide to favour the application of innovative research methodologies in the translational research? Any specificities in paediatrics?

**Donato Bonifazi - Coordinator**

***European Paediatric Translational Research Infrastructure***

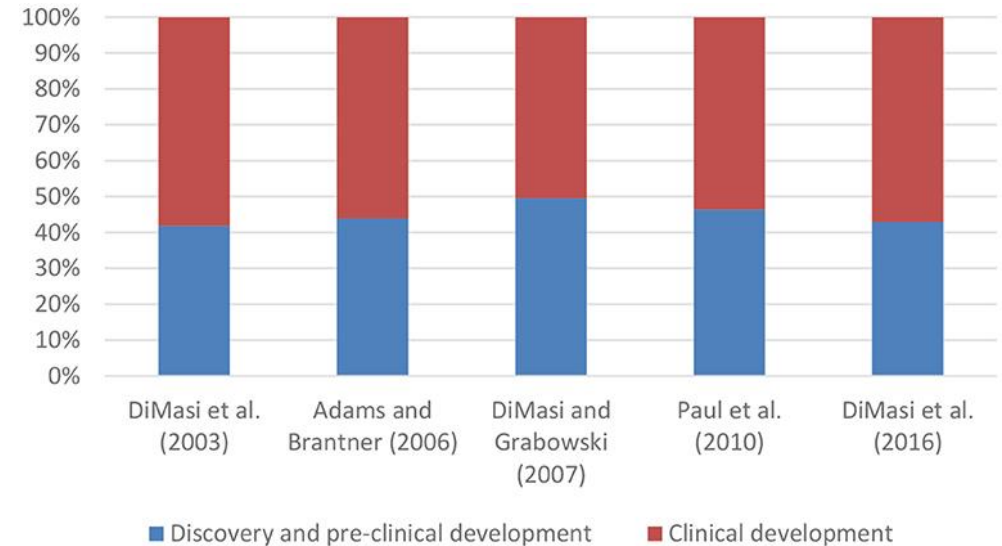




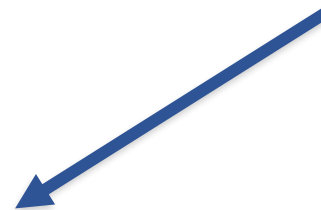
# Efforts and costs for drug development

❖ *Drug development is a long and costly process including different steps: a) discovery, basic and pre-clinical b) translational and clinical research*

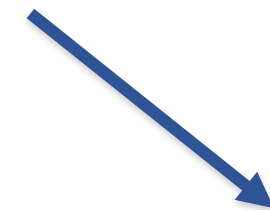
❖ *Ad hoc research instrument recognized at EU level, mainly adopted to support academy and non profit research are based on:*



## TWO PILLARS



**Clinical Trial Networks**



**Research Infrastructures**



# Clinical Trial Network *versus* Research Infrastructure

## What is a Clinical Trial Network?

Clinical Trial Networks are composed of several organizations within a country or a wider context having specialty competencies in a specific disease or therapeutic area or having methodological expertise in designing and conducting clinical trials

- *Paediatric Specialty Networks*
- *Paediatric Methodological Networks*
- *Rare Disease (disease-specific) Networks*  
... are the most represented in EU



## What is a Research Infrastructure?

Research Infrastructures are structured organizations that include equipment facilities, and multidisciplinary expertise providing resources and services to the research communities

- *Technology-based*
- *No disease-specific*
- *EPTRI is the only one focused on paediatrics*

# The Paediatric Research networks framework in EU



European Network of Paediatric Research  
at the European Medicines Agency

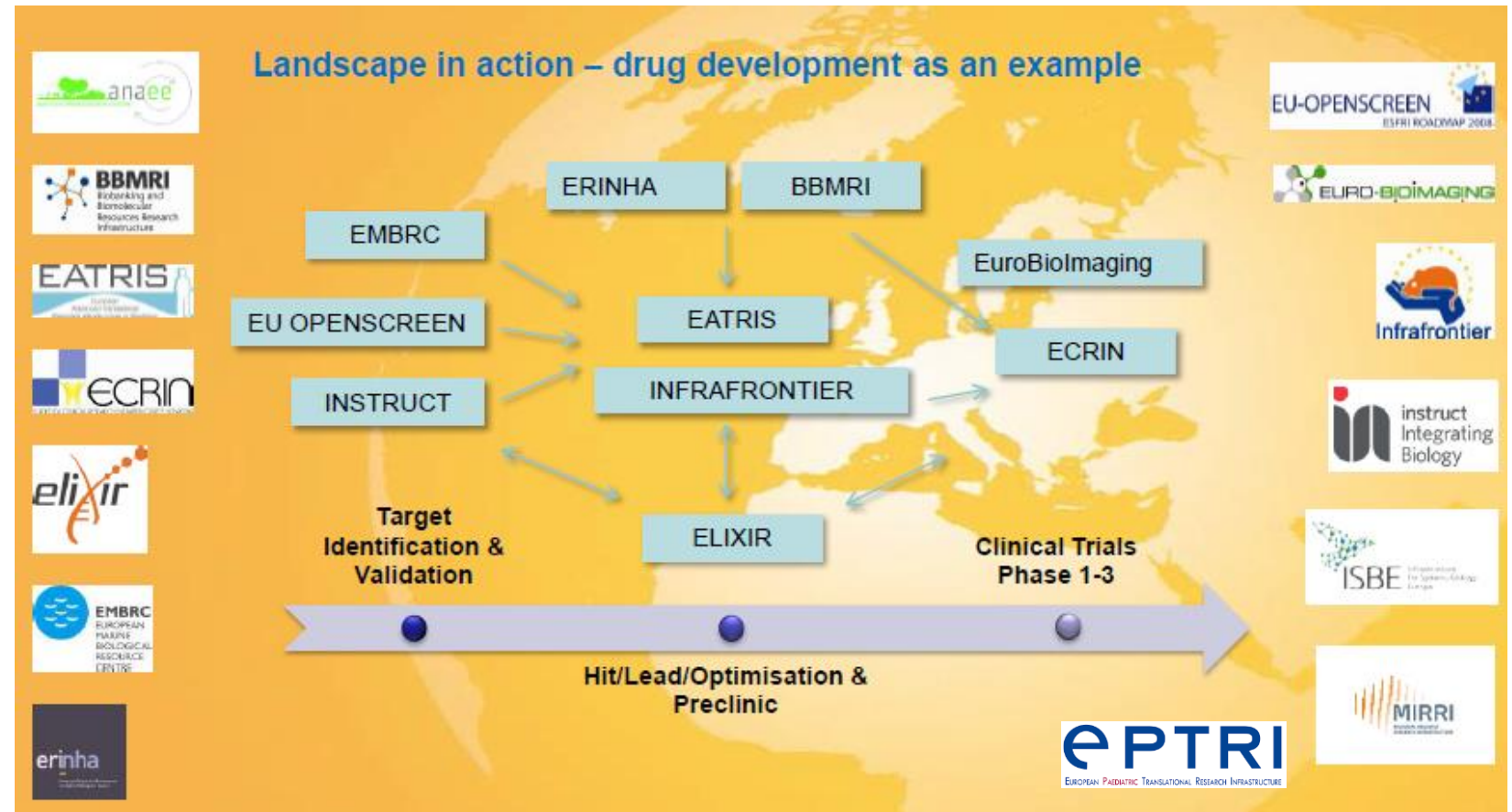


- The European Network of **Paediatric Research Network** (EnprEMA) gathers together 53 networks
- **2 Networks of Excellence** dedicated to paediatric clinical research methodology and support – TEDDY and GRiP
- **20 research consortia** funded by the EC to develop off-patent drugs for children
- **1 IMI2 project:** c4c Global Network to run paediatric trials

# RIs in biomedical field: paediatric specificity was lacking so far...

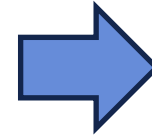
Several RIs are involved in biomedical research and can provide support to drug development in all phases

***EPTRI is the only one focused on paediatrics***



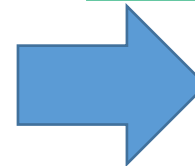
# Why a Paediatric Research Infrastructure?

- Children cannot be addressed with adults' instruments
- To relying on information from studies conducted in adults and generalizing them to children is too risky
- Need to develop new paediatric drugs, biomarkers, therapies, formulations and medical devices



- Differences in terms of aetiology, progression, comorbidities and prognosis
- Differences may affect benefits and/or risks of therapies
- Growth and maturation progress by age changing the drug effects
- Ontogeny influences drugs response (ADME)

Need to incorporate recent advancements from **Life Science** and **Digital Science** that **require equipments and expertise paediatric specific**



**Molecular Targets selection and validation**

**Biomarkers identification/validation on cellular and animal model**

**Bioinformatic application for biological, genetic and imaging data analysis**



# How EPTRI could support Paediatric Research?

## Research needs recognition (data from EPTRI users' survey)

- **Biobank:** few available and accessible biobanks dedicated to children
- **Alternative** non-clinical (non-animal) studies
- Ad hoc paediatric **Formulations and Devices**
- **Genetic factors** may influence pharmacokinetics: ad hoc studies are needed
- **Polymorphisms:** Among the 'omics' only a small number have been studied to identify or validate paediatric biomarkers
- **Personalized medicines** in children
- **IT technologies:** to provide integration and synthesis of evidence reducing trials impact
- **ATMP (gene and cell):** as for other products paediatric approach is needed

## EPTRI Research Platforms and services

### Paediatric services



Medicines  
Discovery TRP



Biomarkers &  
Biosamples TRP



Development  
Pharmacology TRP



Medicines  
Formulations TRP



Medical  
Devices TRP



Centralised  
Services