

# Collaborative efforts to promote innovative drug discovery for children

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EPTRI webinar: biotechnology to bring innovation in the paediatric drug development, 2<sup>nd</sup> October 2020

The event is part of the European Biotech Week 2020

PART OF

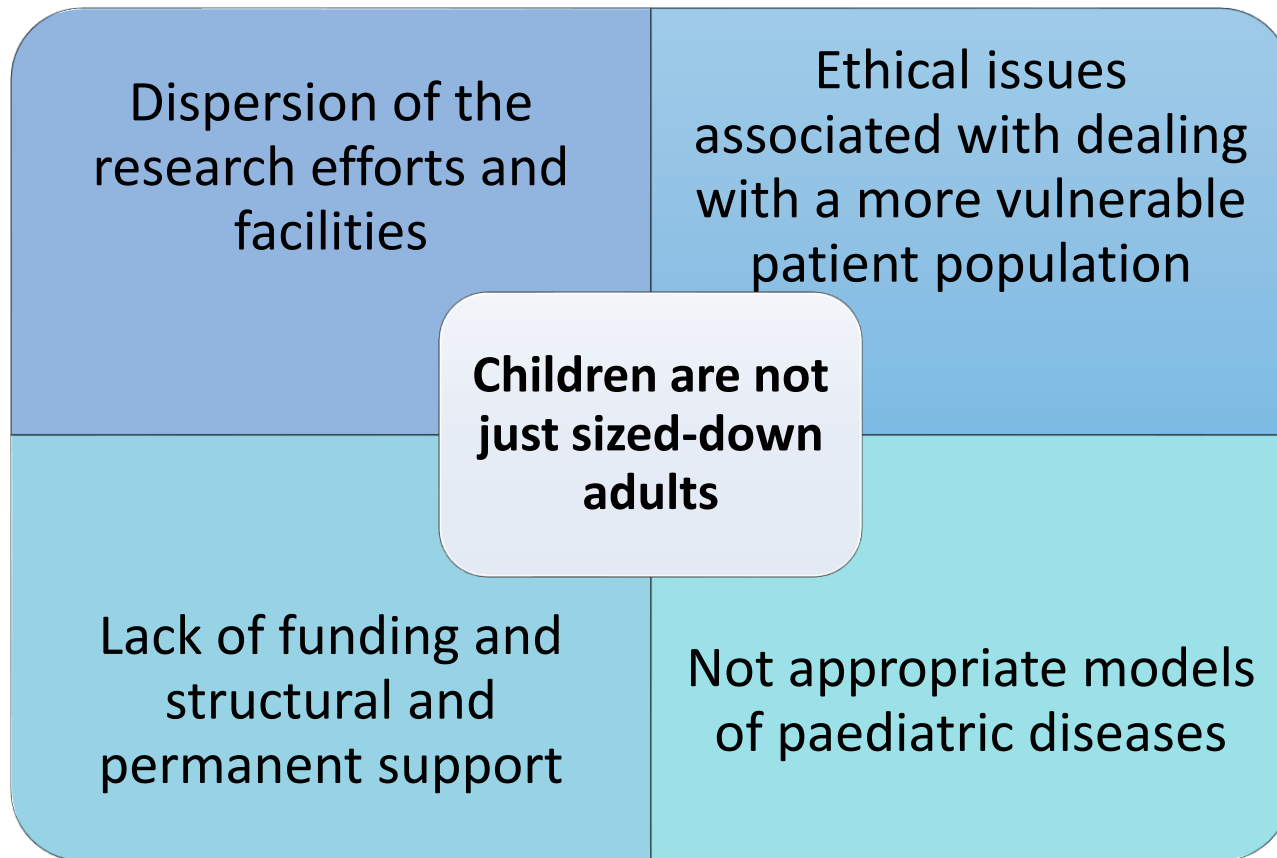
EUROPEAN  
BIOTECH  
WEEK



INNOVATION IS IN OUR GENES

# Lack of medicines for children

*There is a broad medical need for new, innovative medicines for children*



# A structured and specific approach is needed

## Structured approach to research

- overcomes fragmentation of paediatric expertise
- provides a consistent framework for all stages of drug development
- speeds up research by using the same systems and processes for each study
- enhances quality and ensure that the voices of patients and caregivers are heard



## Specific approach for paediatrics is needed

- impact of ontogeny on drug development,
- most child health services are hosted in dedicated facilities and provided by specialised staff

Turner MA, Cheng K, de Wildt S, Hildebrand H, Attar S, Rossi P, Bonifazi D, Ceci A, Claverol J, Nafria B, Giaquinto C. European research networks to facilitate drug research in children. Br J Clin Pharmacol. 2020

# A structured and specific approach is needed

## Context

Proportionate regulations  
Sufficient drivers and incentives

## Resources

Rational and efficient deployment  
of resources within organizations  
to where they can yield most  
value

## Coordination

Coordination (avoiding duplication and  
unnecessary gaps)  
  
Promoting collaboration (finding  
researchers, clinicians and experts, and  
supporting joint working)  
  
Ensuring the patients and caregivers  
involvement

## Science

Rigour and stringency;  
  
Good guidelines and pathways;  
  
Good techniques leading reliable,  
repeatable results;  
  
Coherence across each step of the pathway

# Context: Regulatory attempts to overcome challenges

Paediatric Medicine Regulation, which came into effect in the European Union (EU) in 2007

Best Pharmaceuticals for Children Act (BPCA) (2002) and the Paediatric Research Equity Act (PREA) (2003) –made permanent in 2016

Incentive and mandate for drug companies to conduct paediatric clinical trials and expand labelling to include paediatric indications.

Provisions to stimulate research on off-patent drugs used commonly in children

Encouragement to the establishment and consolidation of investigator networks and trial sites for paediatric research.

Limited progresses had been made in diseases that only affect children or where there are significant pathological differences between adults and children with the same disease

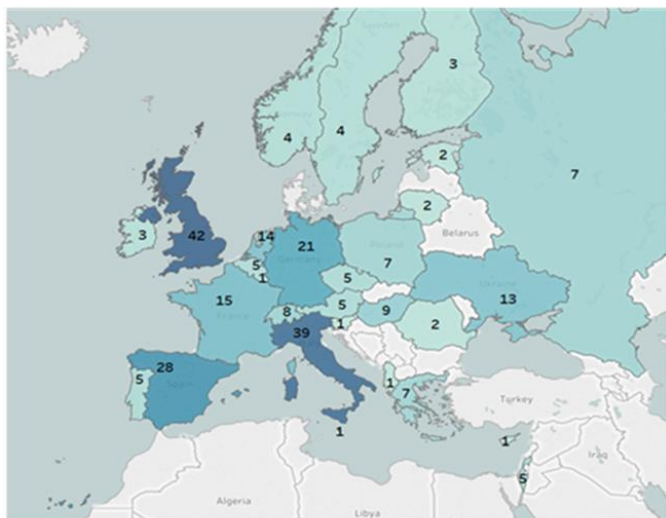
# Resources

*Many relevant organisations, networks and research consortia are involved in paediatric research and demonstrated the ability to provide excellent research capacity in the whole paediatric medicine field including basic research on human development and ontogenesis and many scientific topics related to preclinical and translational paediatric research.*

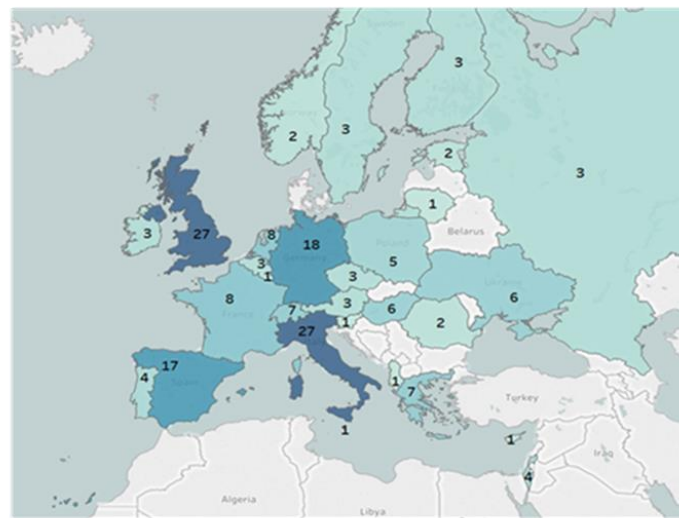
# Paediatric Resources

A large survey has been performed within the Paediatric Scientific Community from April 2018 to November 2019 to identify the perceived needs and the possible gaps to be covered and to enquire the scientific paediatric community with its expertise and research capacity

*Research Units by country*



*Research Institutions by country*

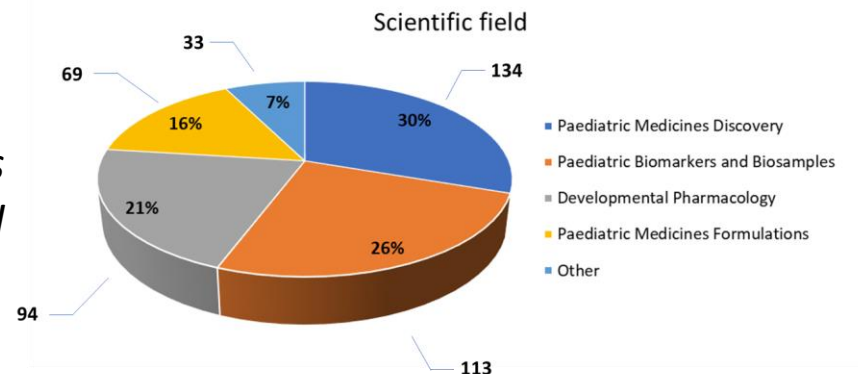


*Notes: Update November 2019, RUs answering to more than one WP have been considered once in the maps, RUs referring to Italian CNR have been counted once.*

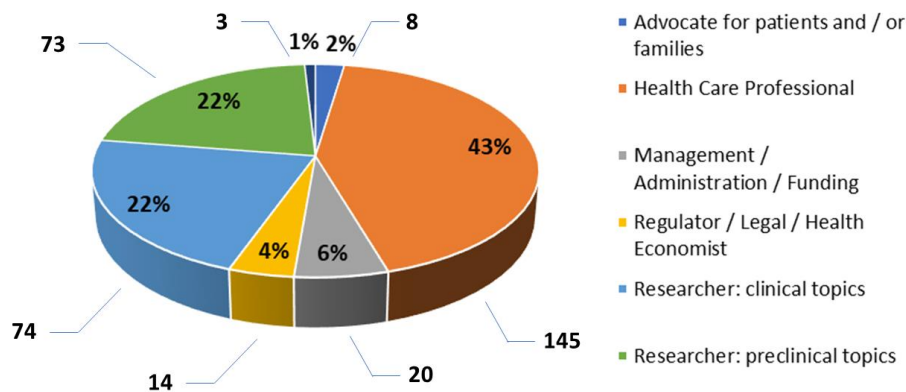
**259 Institutions, 330 Research Units**

# Targeted user community

*Distribution of the targeted users divided by scientific field*



**Sort**



*Distribution of the targeted users divided by sort*



# Science

- Physiologically based drug targets and assays methods,
- 3D Cellular models
- Genetic/genomic derived innovation and omics technologies
- In silico pharmacology methods
- Advanced therapy
- E-resource based technologies
- Data sharing and interoperability



*We need to establish more effective system to recognise promising basic research at an early stage as well as to safeguard and develop the results effectively*

# Coordination

## *Structured approach to research*



European Strategy Forum  
on Research Infrastructures

Some transnational research infrastructures (RIs) that aim to support specific activities have been established but have not worked in paediatrics as they lack of specific expertise and facilities

## *Structured and specific approach for paediatric research*



# EPTRI aims

To accelerate and expand the paediatric drug discovery and development processes by focusing on basic and preclinical research methods and taking into account:

1. the continuous ontogeny induced variability in drug disposition, disease targets and diseases biomarkers and
2. the natural condition of the child growth requiring size adaptation of medicine forms and tests (small volumes, microtracing, microdosing, etc.)
3. the advancement in life science, technologies and data science to be implemented in the paediatric setting.

# EPTRI collaborative approach

Liaise with the many paediatric initiatives conducted at national and international level as well as with the international research programmes and projects



Increase the impact of EPTRI services and initiatives and its global partnership

Reduce the barriers between basic science and citizens' wellbeing providing evidence of the direct benefits to society

# Outreach and networking strategy



Building liaisons with all relevant stakeholders including Scientific Community, Policy and Regulatory decision makers, Pharma and Biotech Industries and SMEs

# EPTRI in the context of the existing Paediatric Initiatives



# Conclusion

***Paediatric research should have a single identity that represents and integrates all the competences and initiatives of medicines development, from bench to bedside, having in mind the final goal of children's health.***

- ✓ *EPTRI will facilitate the translation of the acquired new knowledge and scientific innovation into paediatric clinical studies phases and medical use.*
- ✓ *EPTRI will enhance innovative technologies useful to accelerate paediatric drug development processes, using an original scientific approach that is paediatric patients centred.*



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**EPTRI**

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

European Biotech Week 2020 - EPTRI webinar, 2<sup>nd</sup> October 2020





# *Thank you for your attention*

