

# ePTRI

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

# The ePTRI project and the future of Paediatric Research

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09/07/2020



This project has received funding from the European Union's Horizon 2020 research and innovation programme under Grant Agreement No 777554

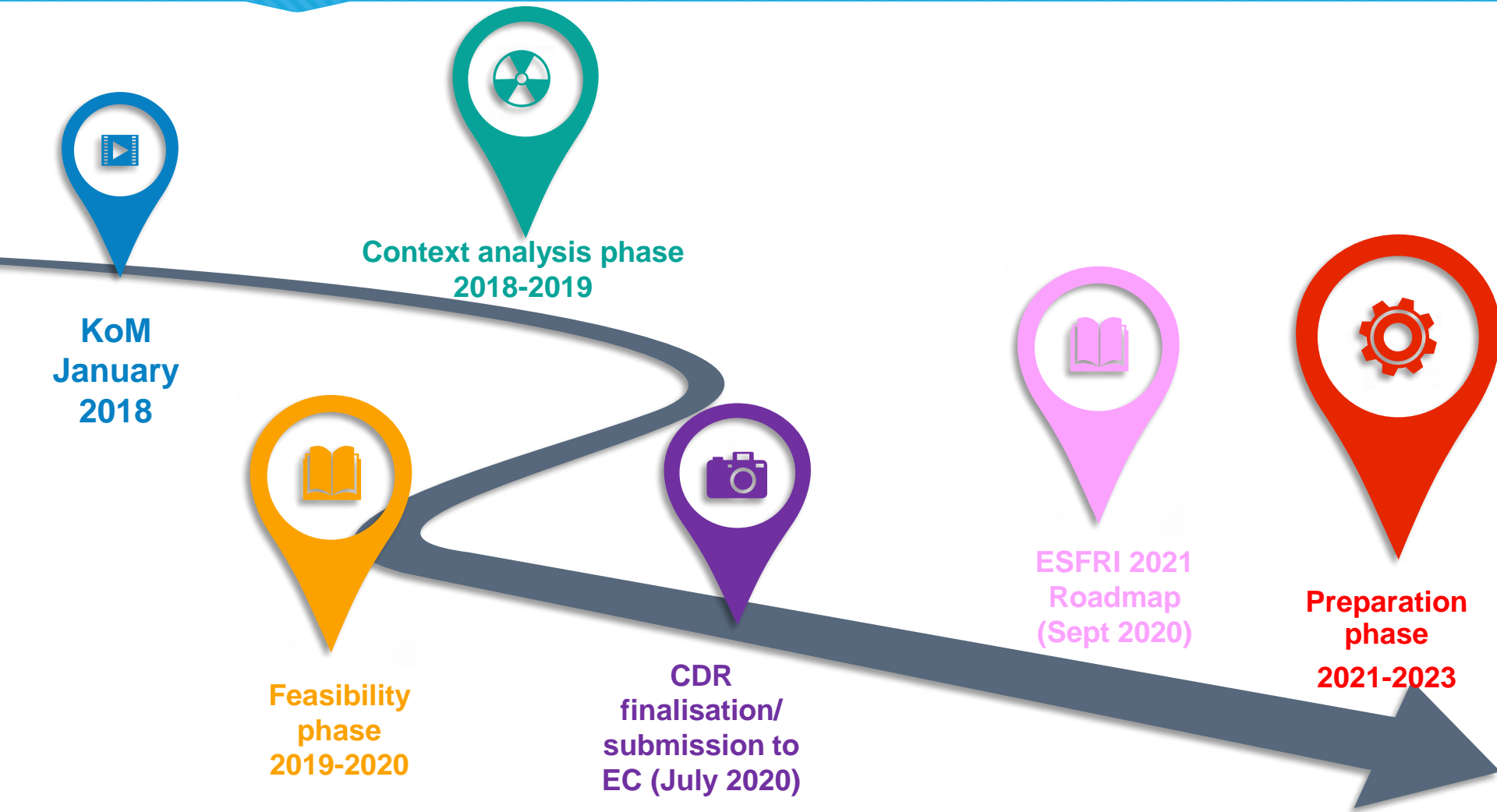
# EPTRI in a nutshell

- ❖ **Funds:** Horizon 2020 EU Research and Innovation programme
- ❖ **Coordinator:** Consorzio per Valutazioni Biologiche e Farmacologiche
- ❖ **Start date of the project:** 1 January 2018
- ❖ **29 partners** from 21 EU/non EU countries
- ❖ **259 research units** candidate as EPTRI providers from 29 EU / non EU countries



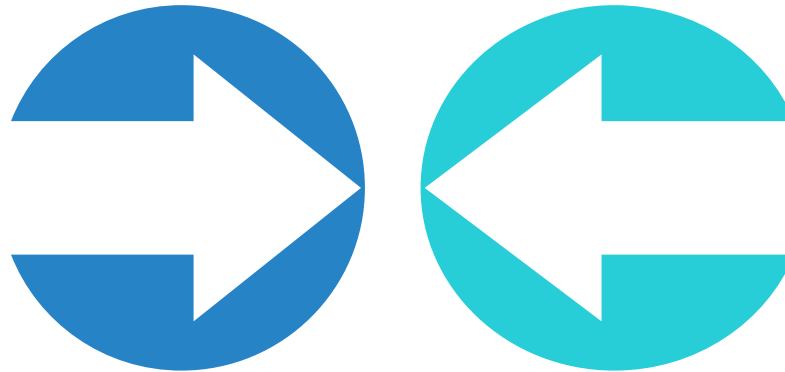
- Accelerating the paediatric drug development processes from medicines discovery and preclinical research to paediatric biomarkers, developmental pharmacology and paediatric formulations
- Use results of basic and translational research to underpin paediatric clinical trials and studies
- Support the development of age tailored medicines

# EPTRI life cycle



Based on more than 250 RUs involved in paediatric research surveys, the main gaps to be covered were identified:

Dispersion of the research efforts (competencies, equipment, facilities exist but do not receive continuous and structural support).

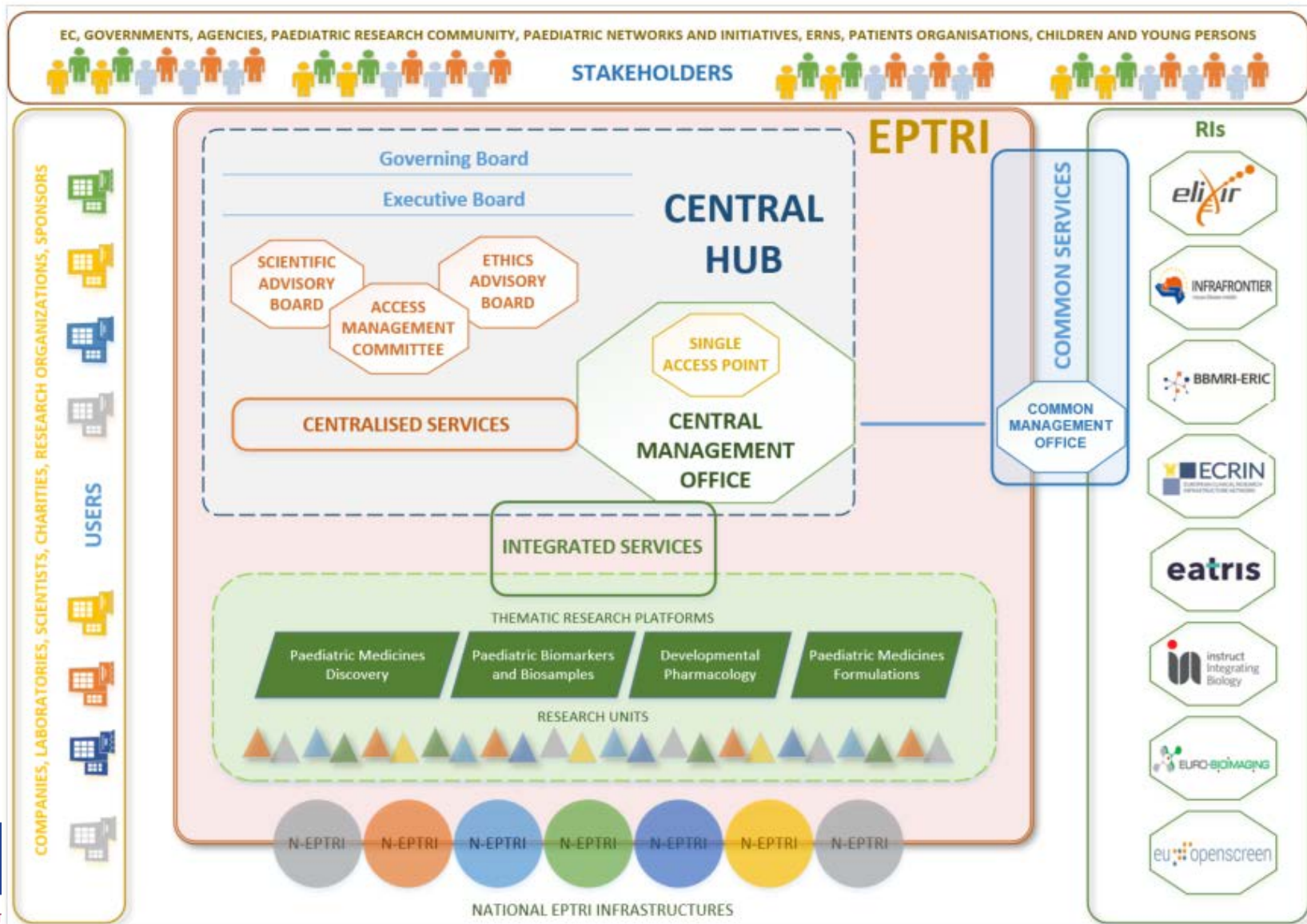


Innovative technologies (new diseases models, biomarkers, innovative study design) are few applied in the paediatric setting.

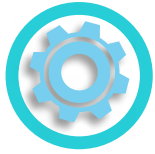
The following four research areas were acknowledged by participants as the more relevant gaps in paediatric drug development:

**Paediatric Medicines Discovery, Paediatric Biomarkers and Biosamples, Developmental Pharmacology, and Paediatric Medicines Formulations.**

# EPTRI Governance



# EPTRI-IA



## Networking activities

To strengthen the coordination and integration within the national and regional research infrastructures that will provide services relevant to paediatric basic, preclinical and translational research



## Trans-national access services

- To improve capacity and quality, and provide efficient trans-national access to advanced research services provided by the research organizations participating in EPTRI



## Joint research activities

To develop services enabling basic, preclinical and translational paediatric research, selected according to criteria of feasibility, costs, excellence and expected impact



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# The EU Pharmaceutical Strategy



The global pharmaceutical market is estimated to grow in the next years, offering a growth potential for EU industry. Innovative technologies and access and analysis of data collected from clinical experience are changing the way products are developed and have the potential to transform therapeutic approaches and business models

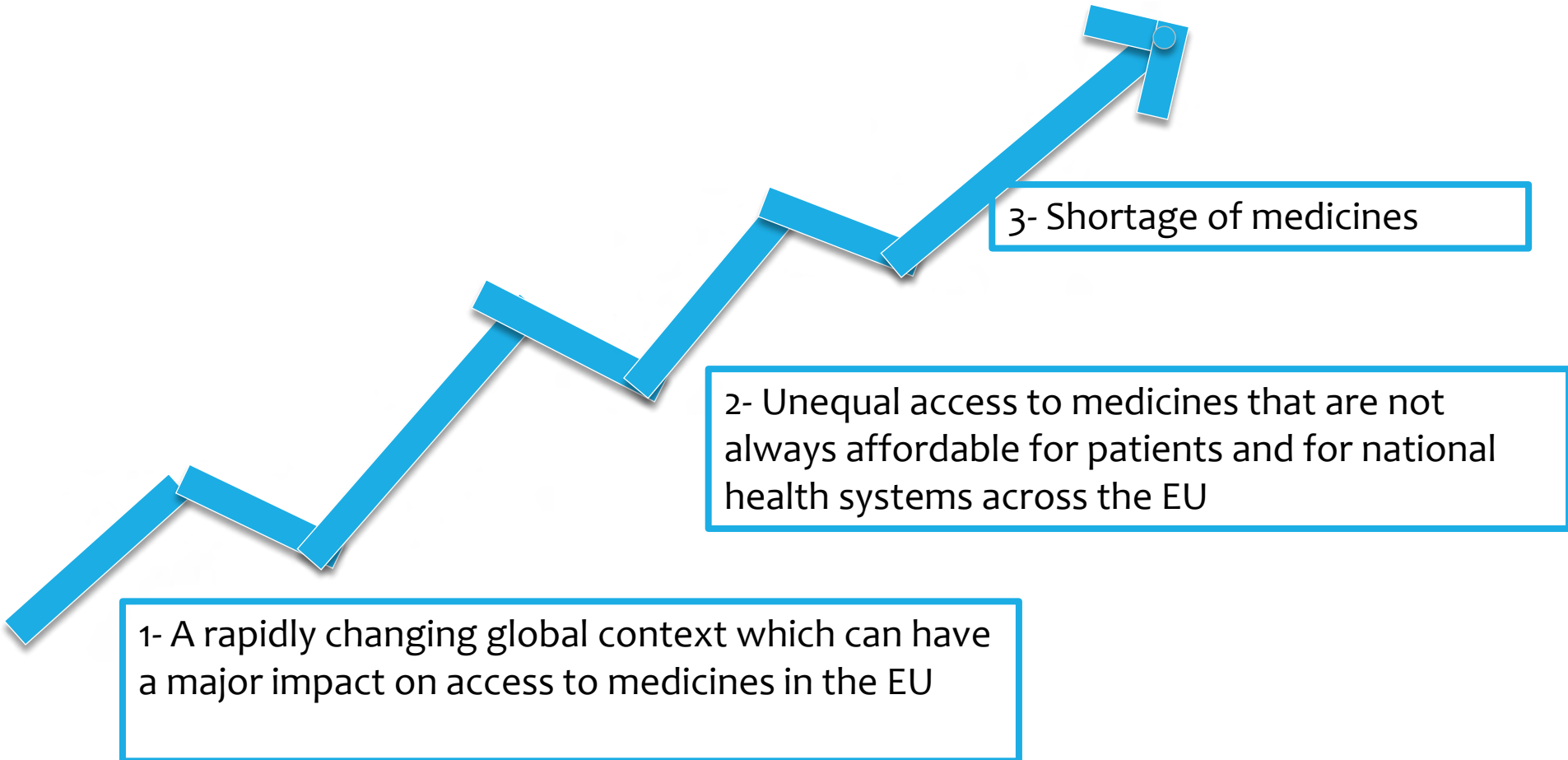


The EC will launch a Pharmaceutical Strategy for Europe to continue ensuring the quality, safety and efficacy of medicines and reinforcing the sector's global competitiveness.

**EPTRI can play a crucial role in addressing the paediatric challenges.**

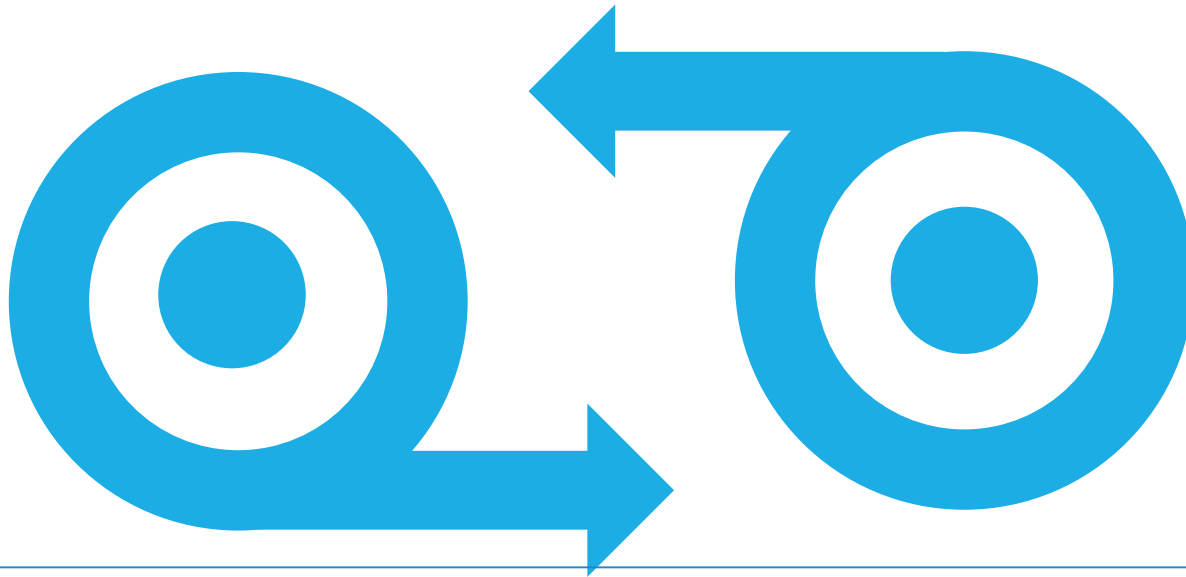


# Challenges



# Challenges

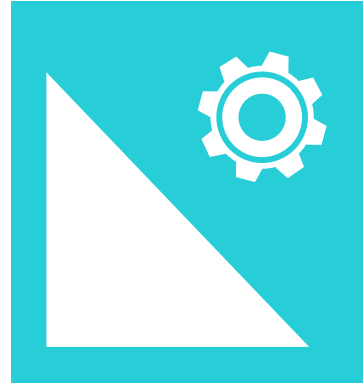
Innovation efforts are not always aligned to public health and health systems' needs



Technological and scientific developments may challenge the regulatory framework and consequently lead to unintended barriers to needs-driven innovation

# Challenges

The European Union has the tools, infrastructure and researchers needed to take the lead in medicines research and innovation for the development of treatments and medical devices.

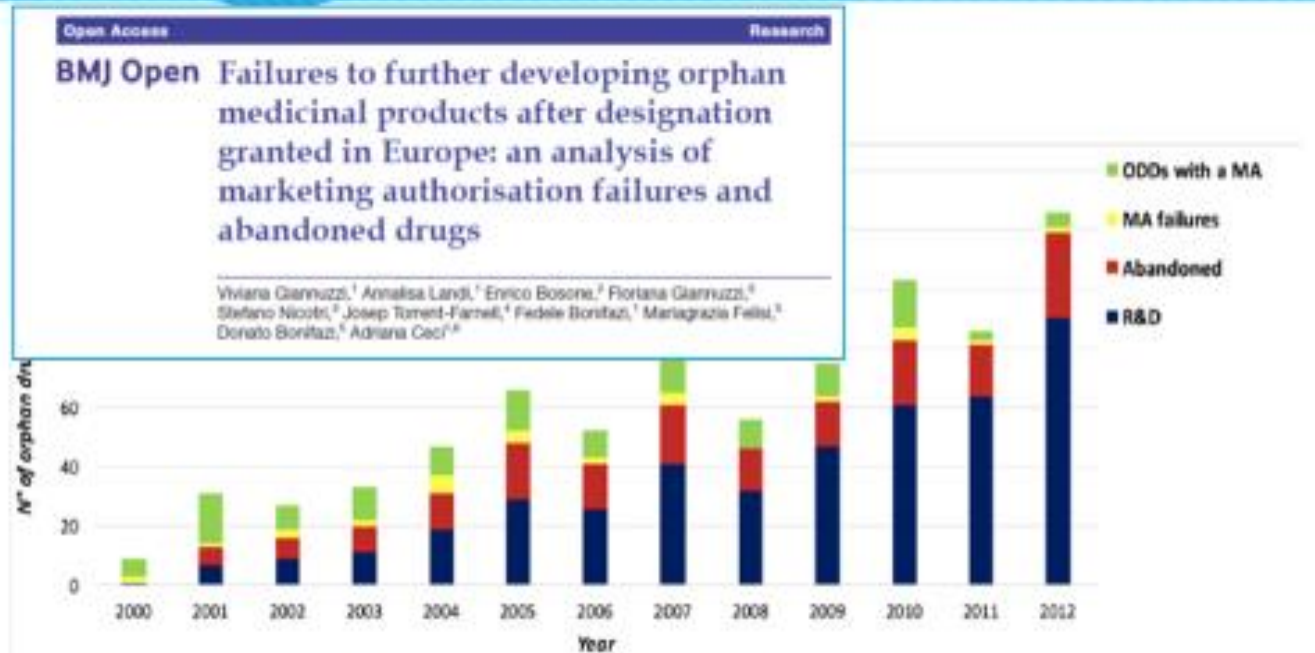


The huge potential paediatric and rare disease research' efforts remain particularly dispersed and disaggregated, and are often excluded from the stronger supportive actions.

# Children are 'orphan' twice...

## Rare diseases often affect children:

- Many rare diseases are genetic
- Start early in life
- Affect growth, sexual and CNS maturation during the developmental process



Distribution of orphan designations by year. MA, marketing authorisation; ODD, orphan drug designation; R&D, research and development.

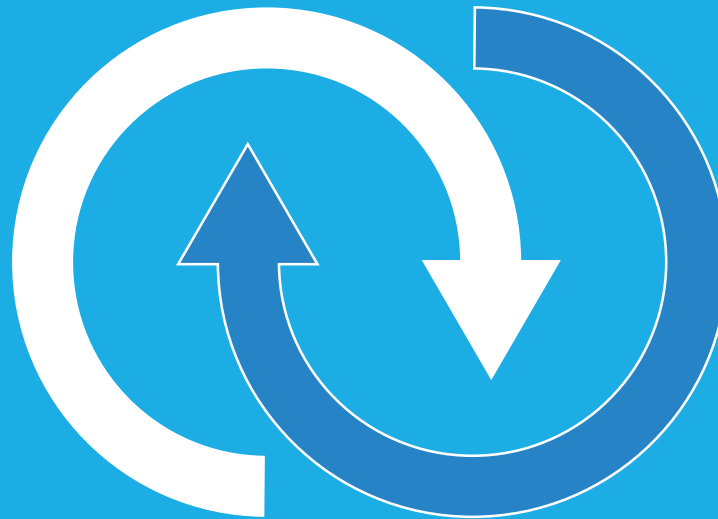
## Few specific information are available because:

- Small number of patients affected by each condition
- Few resources invested
- Use of medicines not specifically tested (off-label, unlicensed)
- Evidence only available from published non registrative studies



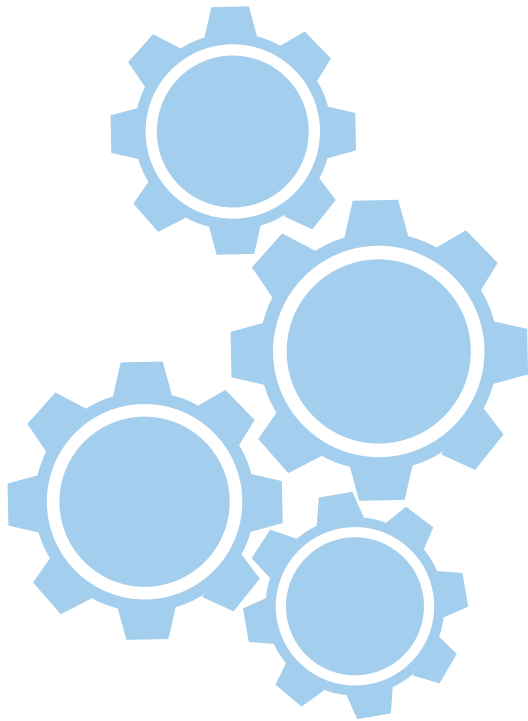
# New approach is needed

1- Focus on researchers-driven research to allow good ideas born in academic and non-profit laboratories to be implemented and therefore become attractive also for commercial sponsors

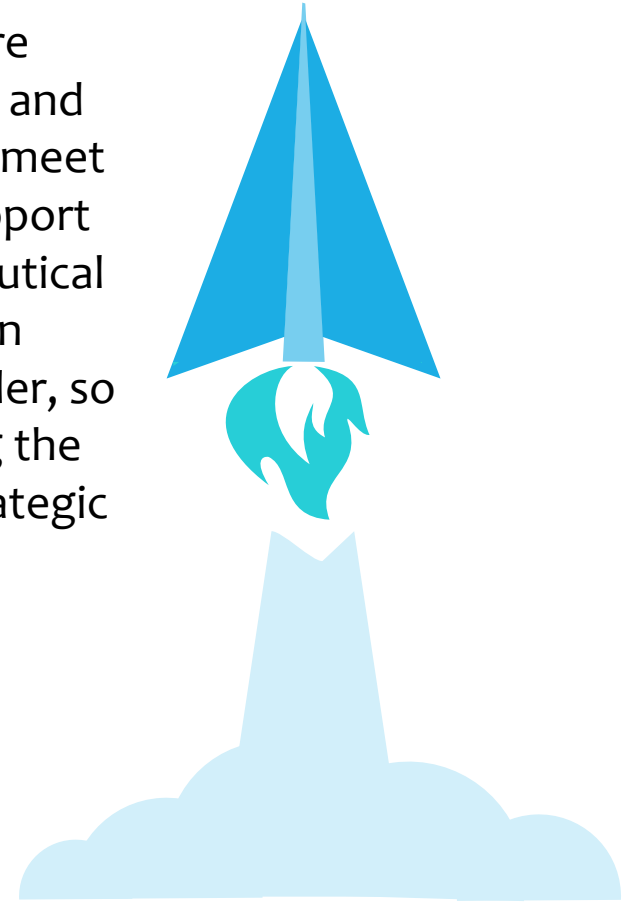


2-Create public / private opportunities to avoid the block of the implementation and market phases

# EPTRI contribution



EPTRI will help ensure Europe's supply of safe and affordable medicines to meet patients' needs and support the European pharmaceutical industry to remain an innovator and world leader, so contributing in building the new Pharmaceutical Strategic framework.



# EPTRI contribution

EPTRI as a new establishing permanent, reliable European wide infrastructure will modify the current landscape helping the development of excellent science and related institutions, and the improvement of the economic and industrial capacity of the EU, positively impacting on the patient populations and their caregivers.

EPTRI will transform the current panorama

It will implement in the paediatric field, innovative methods and technologies

It will close the gap between innovative technologies and paediatric drug development processes

# EPTRI Impact



- ❖ EPTRI will contribute to create an “integrated paediatric system” that will be also interconnected to existing landmark RIs and relevant clinical research networks
- ❖ The adoption of paediatric oriented tools and competences will have a multiple impact on the whole research chain from drug discovery to preclinical and clinical phases
- ❖ EPTRI is also expected to positively impact on the social and ethical aspects





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