



EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

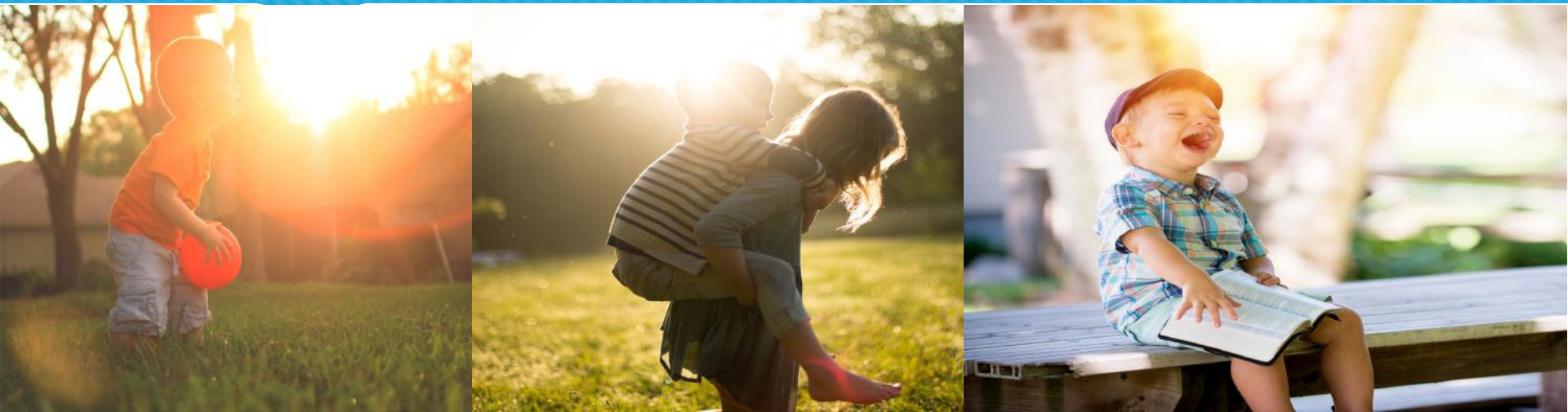
# ID-EPTRI EPTRI OPEN MEETING

April 2020



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# Introductions



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**European Medicines Agency PDCO Member - Eurordis**

**World Duchenne Organization Board Member**

**European Patient Forum Board Member**

# The 3Ds

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# Introductions WDO and EPF



**WORLD  
DUCHENNE  
ORGANIZATION**  
UPPMD



**ePTRI**  
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April 2020

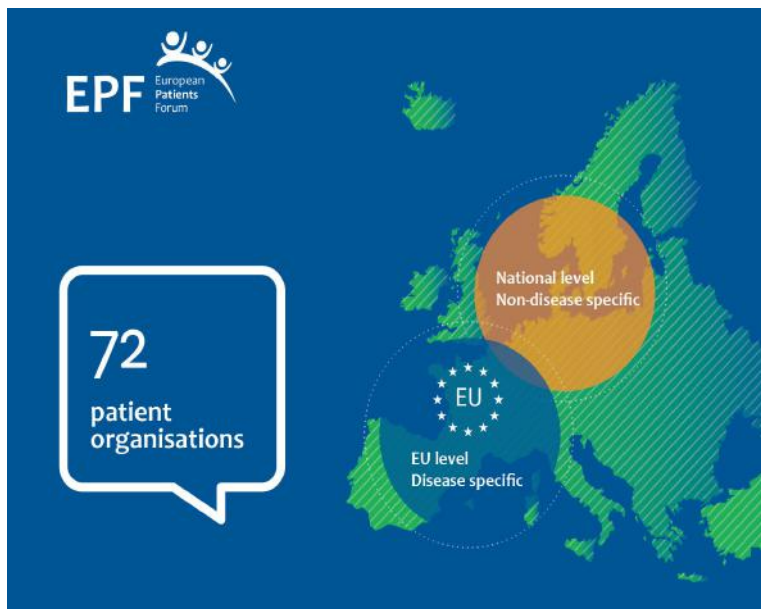
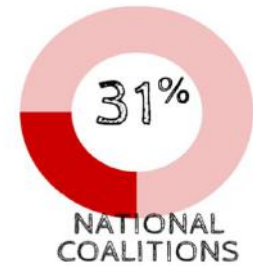


# World Duchenne Organization

- **Global advocacy, Regulatory and Policy work,**
- **High interest from basic research .. Pipeline development, PROMs...**
- **EMA and FDA interactions**
- **Members of EURORDIS and EPF**
- **40 member from 5 continents**
- **Scientific Directors Committee**
- **Duchenne Data Platform**
- **Certified Duchenne Care Centers Program**
- ...



# European Patients Forum



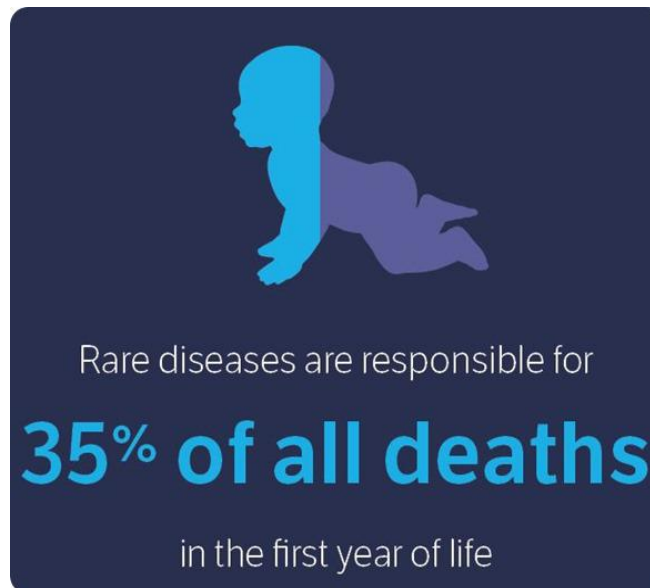
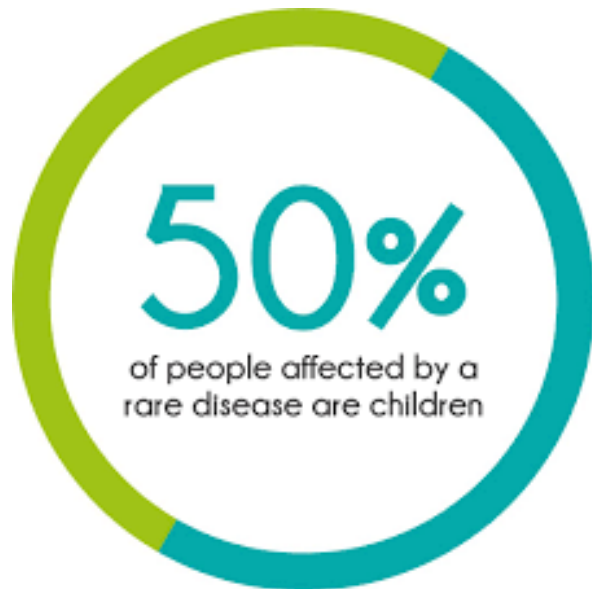
# Common Objective

**Meet the needs of the EU paediatric patients**



# Paediatric Unmet Need

**30 million European citizens are affected by a RD**



EURORDIS Therapeutic Report



# Paediatric Unmet Need

**Since  
2000**



**2067**  
Orphan  
designations



**171**  
Orphan designations  
included in authorised  
indication



**153**  
Authorised  
OMPs



**61**  
To be used in  
children



**4**

Removed from  
the market

**43**

Marketed, but no  
longer "orphans"

**To date**

**110**

Products with a marketing  
authorisation and an orphan status in  
the European Union

EURORDIS Therapeutic Report

**ePTRI**

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# Solution?

The **European Paediatric Translational Research Infrastructure, EPTRI**, “ is a project that arises from the need to find answers to the serious lack of medicines for children in EU and worldwide. It aims to **propose developmental models for a future research infrastructure** focused on paediatric medicines, integrating technology-driven aspects with clinical trials.”

EURORDIS Therapeutic Report

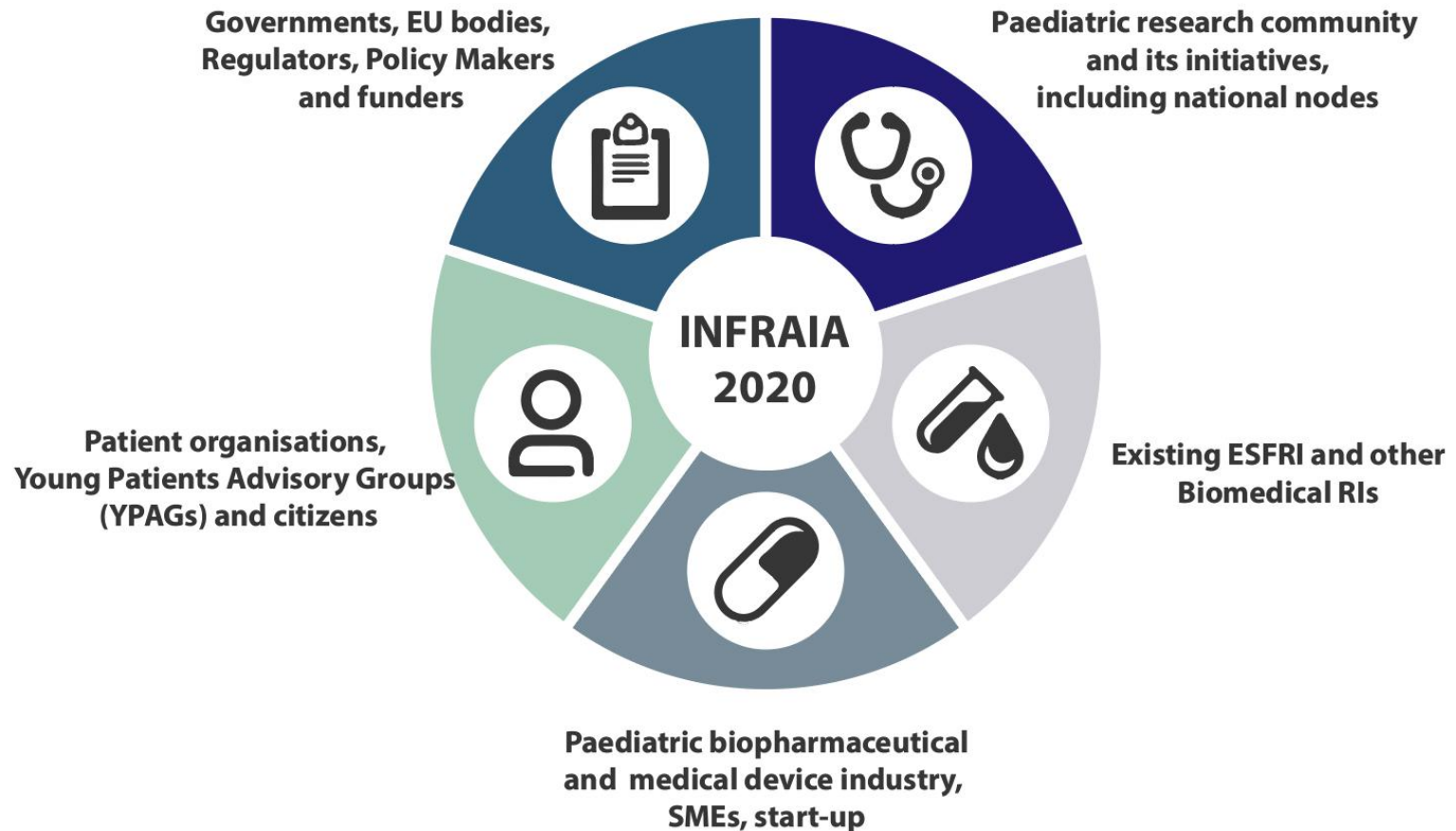


# Outreach and Networking Goal

## Establish a wider network

- Liaise with paediatric research initiatives, programmes and projects conducted at national and international level
- Assure the critical involvement of the patient voice
- Buy in from Policy and Regulatory decision makers and Funders
- Reach out to the real world of medicines development including the Pharma and Biotech Industries and SMEs.

# Outreach and Networking





# Task Breakdown

**Task 4.1 Development and Implementation of an Outreach Plan**

**Task 4.2 Outreach to the paediatric research community.**

**Task 4.3 Networking with European Strategy Forum on Research Infrastructures ESFRI**

**Task 4.4 Outreach to the paediatric biopharmaceutical and medical devices industry and CROs, including SMEs and start-up**

**Task 4.5 Outreach to patients' organisations, Young Patients Advisory Groups and representatives, families and citizens**

**Task 4.6 Outreach to Governments, EU bodies and Funders**

**Task 4.7 Stakeholder forum**

# Three Work Streams

- Create and maintain aggregation and collaboration among the paediatric medicines research groups involved in the project and willing to participate in the new RI.
- Bridge these groups with the already existing paediatric research initiatives in order to globally produce a huge acceleration/qualification of procedures for the whole development process of new medicines.
- Build and maintain an active multi-stakeholder forum:
  - a. to promote the much-needed research in the paediatric population,
  - b. leverage the existing initiatives, projects and knowledge,
  - c. to deal with the research fragmentation but also
  - d. to keep the paediatric medicines development high in the EU agenda.

# Outreach and Networking

## **Task 4.1 Development and Implementation of an Outreach Plan**

**A Dedicated Internal Outreach Taskforce will be created.**

**The members of the Task Force will be appointed by each partner that would be responsible to reach out to its dedicated stakeholder groups with a common and efficient message.**

## **Task 4.7 Stakeholder forum**

**To promote the project' outreach and impact an External Stakeholder Platform (ESP) will be created**

**ESP will be an independent taskforce of EU and Global key opinion and thought leaders that will be supporting the Outreach Taskforce.**

**ESP will provide the High Impact Individuals that are needed both for Policy and Regulatory changes towards a more effective policy for Paediatric Research.**

# Lets make EPRTI the Solution!

## THANK YOU!



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