

# Paediatric Medicines Discovery TRP

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# Why a TRP on paediatric medicines discovery?



Children represent more than 20% of the EU population, but more than 70% of marketed drugs have not been **properly tested** for them

Paediatric drug development is usually **driven by adult-based development**

70% of serious life-threatening rare diseases have an exclusively paediatric-onset



Only 20% of medicines approved for a **rare disease** affecting also children has a paediatric indication

13-69% of the prescriptions within a paediatric hospital setting is **off-label**. This percentage increases dramatically in neonatal setting

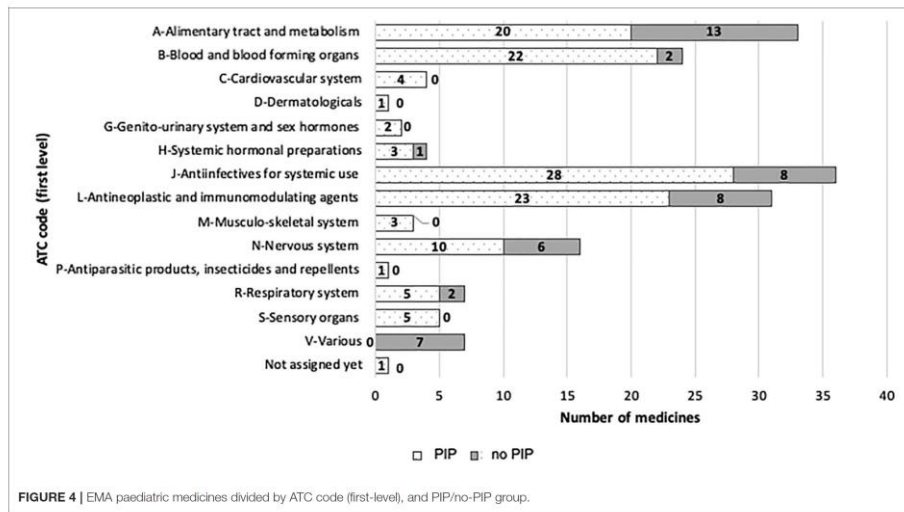
# Paediatric medicines discovery and unmet medical needs



Paediatric drug development is usually driven by adult-based drug development and follows clinical trials in adults



'Paediatric-only' diseases are likely to remain 'orphan' of medicines and therefore 'unmet needs'

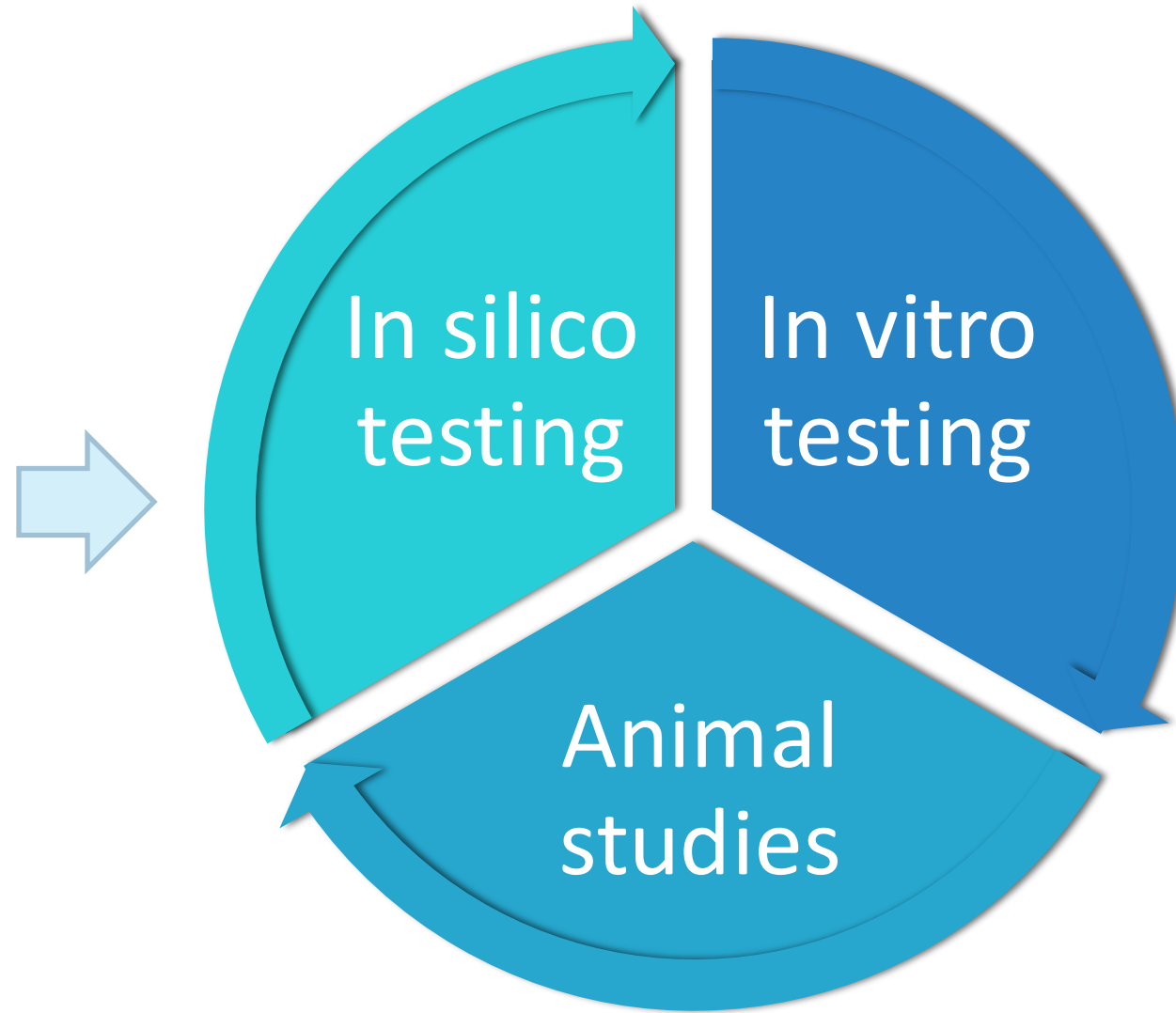


Some therapeutic areas still have limited therapeutic options for children (as well as youngest children)

Toma et al. Front Med. Volume 8, 2021

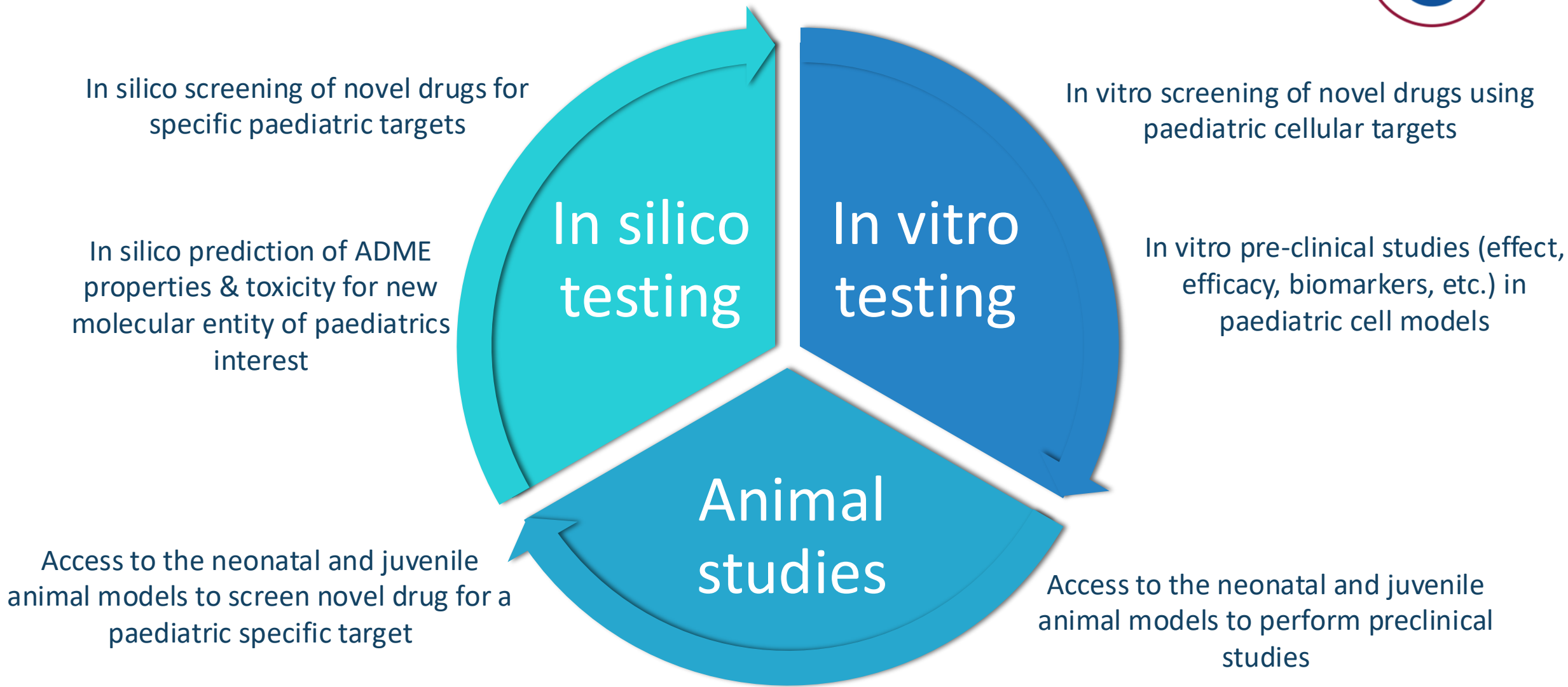
[European Commission, Public Health, last access August 2023](#)

# Paediatric medicines discovery TRP



Research units performing activities and delivering services in three main areas

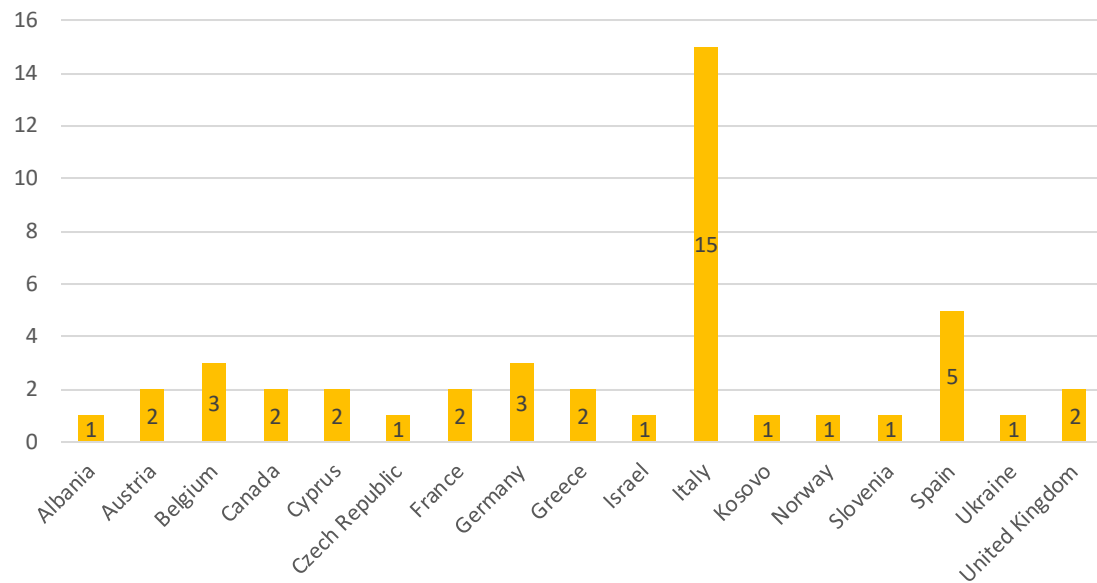
# Paediatric medicines discovery services



# Paediatric Medicines Discovery TRP at a glance

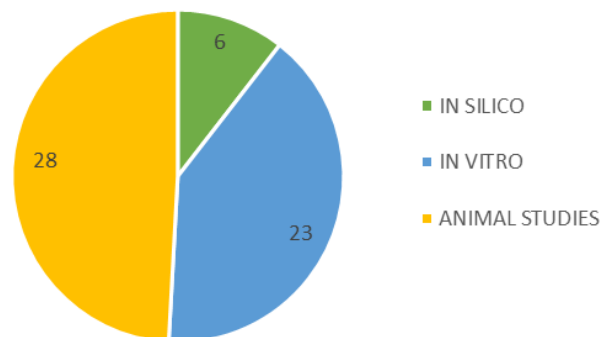


Research units by country



**45 Research Units from 17 Eu/non-Eu countries**

Research units by area(s) of expertise



SERVICES	RESEARCH UNITS NUMBER
In silico screening of novel drugs for specific paediatric targets	6
In vitro screening of novel drugs using paediatric cellular targets	14
Access to the neonatal and juvenile animal models to screen novel drug for a paediatric specific target	16
In silico prediction of ADME properties & toxicity for new molecular entity of paediatrics interest	4
In vitro pre-clinical studies (effect, efficacy, biomarkers, etc.) in paediatric cell models	19
Access to the neonatal and juvenile animal models to perform preclinical studies	20

# Examples of application of Paediatric Medicines Discovery research services



Repurposing of already approved molecules for a new and different paediatric indication offers a **fundamental opportunity** to cover the existing paediatric medicines gaps.



In silico screening and testing could streamline this process

EPTRI animal models application in paediatrics



Animal Model	Examples of application
<i>Zebrafish models</i>	<ul style="list-style-type: none"> <li>• Phenotypic characterization of novel genes identified in paediatric disease.</li> <li>• Generation of zebrafish mutant lines for modelling paediatric diseases.</li> <li>• Generation of zebrafish xenogeneic tumour models.</li> <li>• Chemical screening in Zebrafish models.</li> </ul>
<i>Rodents model of paediatric disease</i>	<ul style="list-style-type: none"> <li>• Metabolic profile and Behavioural phenotyping. Preclinical studies of new compounds.</li> </ul>
<i>Rodent models of paediatric neurodevelopmental disorders</i>	<ul style="list-style-type: none"> <li>• Analysis of the effects of acute or chronic drug administration on molecular, biochemical and morphological features.</li> </ul>
<i>Fly model for cancer and genetic diseases</i>	<ul style="list-style-type: none"> <li>• Generation of fly disease models.</li> <li>• Functional analysis of neuromuscular alterations.</li> <li>• Analysis of macro- and microscopic phenotypes.</li> <li>• Drug discovery and genetic screenings.</li> </ul>
<i>Mouse and pig models focusing on neuroprotection</i>	<ul style="list-style-type: none"> <li>• Resuscitation and neuroprotection studies with different levels of oxygen and drugs</li> </ul>



In the forthcoming session, you will hear four case studies of concrete applications of Paediatric Medicines Discovery research services...

TOPIC	PRESENTER
<i>Bridging Pediatric Disease Research and Developmental Toxicology with Artificial Intelligence-based Public Web Platforms-</i>	<i>Nicola Gambacorta</i>
<i>AI-assisted drug repurposing studies for the identification of new promising vasopressin V2 receptor ligands for pharmacotherapy in pediatric nephrology</i>	<i>Grazia Tamma</i>
<i>Gottingen minipig model to study drug milk excretion and breastfed infant drug exposure</i>	<i>Domenico Ventrella</i>
<i>What animal tests can tell us about safety of drugs for developing brain?</i>	<i>Hana Kubova</i>