

BIOMARKERS AND BIOSAMPLES TRP & Belgian National JRU

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EPTRI SCIENTIFIC AND GENERAL MEETING – BARI – 18-19/07/2024

Paediatric biomarkers in drug development

Biomarkers play a pivotal role in drug development

- understanding how drugs interact with children's bodies,
- guiding dosing,
- safety monitoring
- efficacy assessments
- stratification of paediatric patient populations

Informing dose selection and adjustments

Rapid growth and development → lead to differences in drug metabolism compared to adults

Serum creatinine and cystatin C help assess kidney function in children can guide appropriate drug dosing



DOI:[10.3390/jpm1010005](https://doi.org/10.3390/jpm1010005)

https://www.google.com/search?q=paediatric%20drug%20doses&udm=2&bs=rimg:CTEDZwTrHwDnYX4Dc3uX0y7YsglAwAIA2AIA4AIA&rlz=1C1GCEA_enCY1026CY1026&hl=en&sa=X&ved=0CBoQuIlBahcKEwjl8MWhr6aHAxUAAA AAHQAAAAQBw&biw=1536&bih=729&dpr=1.25#imgrc=misn6huLBMMyjhM&imgdi=SkJdg5w8oQgr9M

Assessing drug safety

Children may experience different side effects or reactions to drugs compared to adults due to their unique physiology.

Cardiac troponins / liver enzymes help monitor potential organ toxicity



<https://www.google.com/url?sa=i&url=https%3A%2F%2Fwww.kindercare.com%2Fcontent-hub%2Farticles%2F2017%2Fapril%2F6-simple-home-practices-to-keep-curious-kids-safe-from-the-medicines-you-use-ever&psig=AOvVaw3T593j3Bu2AGyih710n0BX&ust=172.104.140.680.4000&source=images&cd=vfe&opi=89978449&ved=0CA8QjRxqFwoTCOCito mypocDFQAAAAAdAAAABAK>

Drug efficacy in children

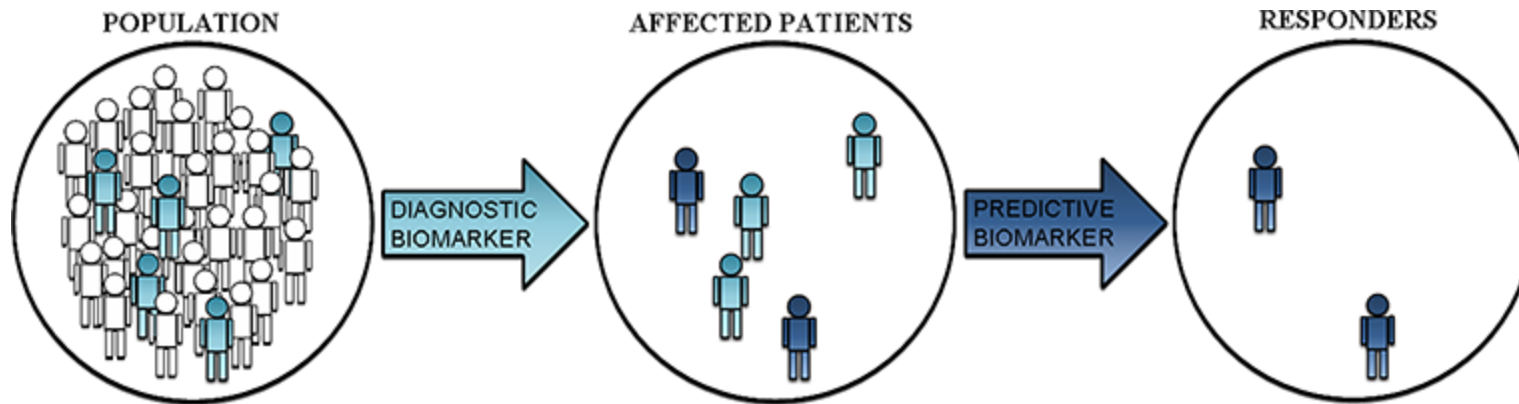
Children may respond differently to medications than adults due to factors such as body size, metabolism, and developmental stages
Biomarkers like cytokine levels or genetic markers can help assess the effectiveness of drugs in pediatric populations



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Stratification of paediatric patient populations

Biomarkers can help **stratify paediatric patient populations** based on their molecular profiles, enabling researchers to design clinical trials with more targeted enrolment criteria.



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Biomarkers in drug development

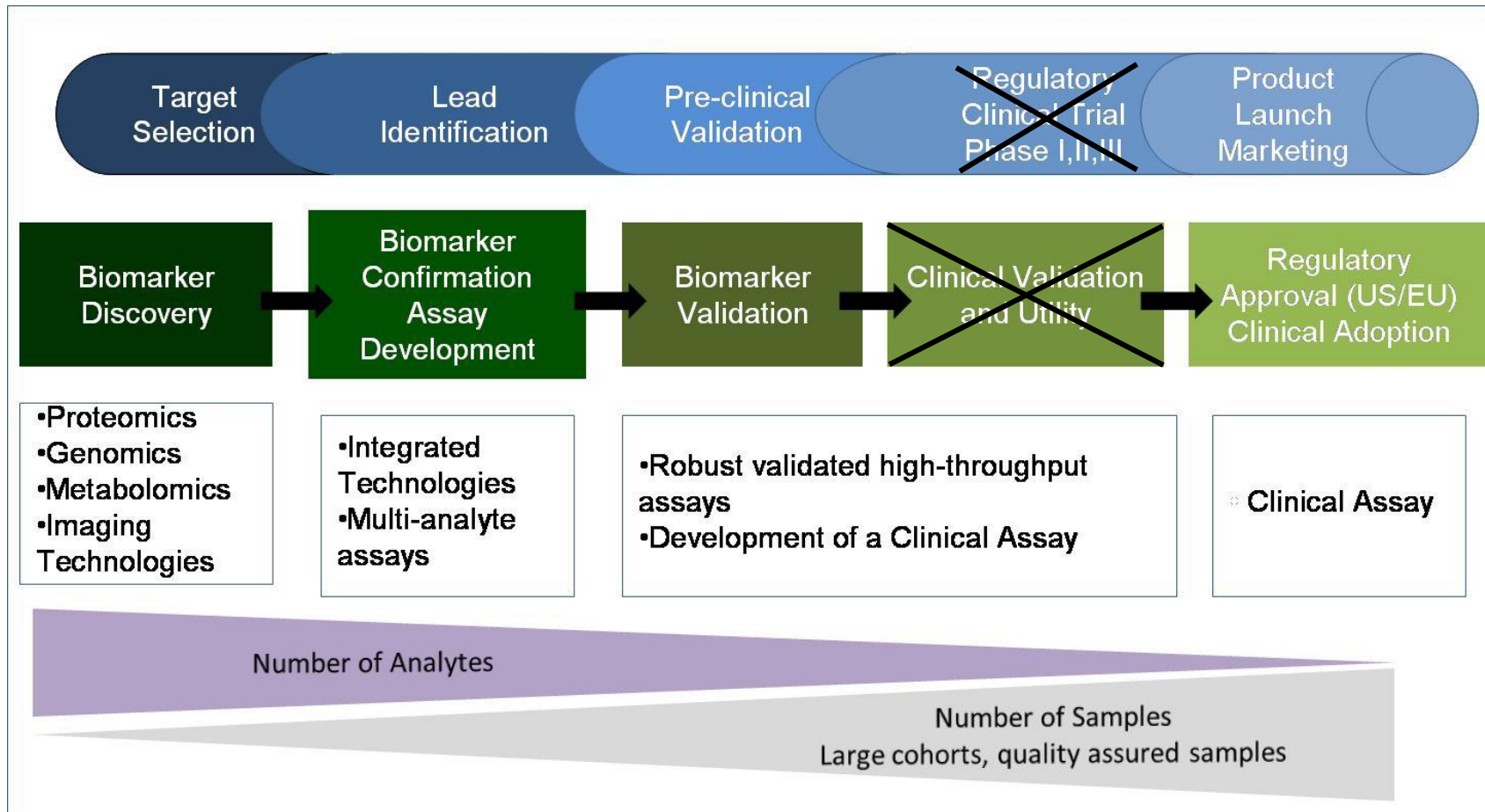
Overall, paediatric biomarkers hold immense promise for enhancing drug development efforts targeted at children.

By incorporating biomarker data into research and clinical practice, researchers can

- drive innovation
- address unmet medical needs
- improve treatment outcomes
- promote the development of safe and effective medications for paediatric populations.
- Introduce personalized and precision medicine approach in paediatric drug development

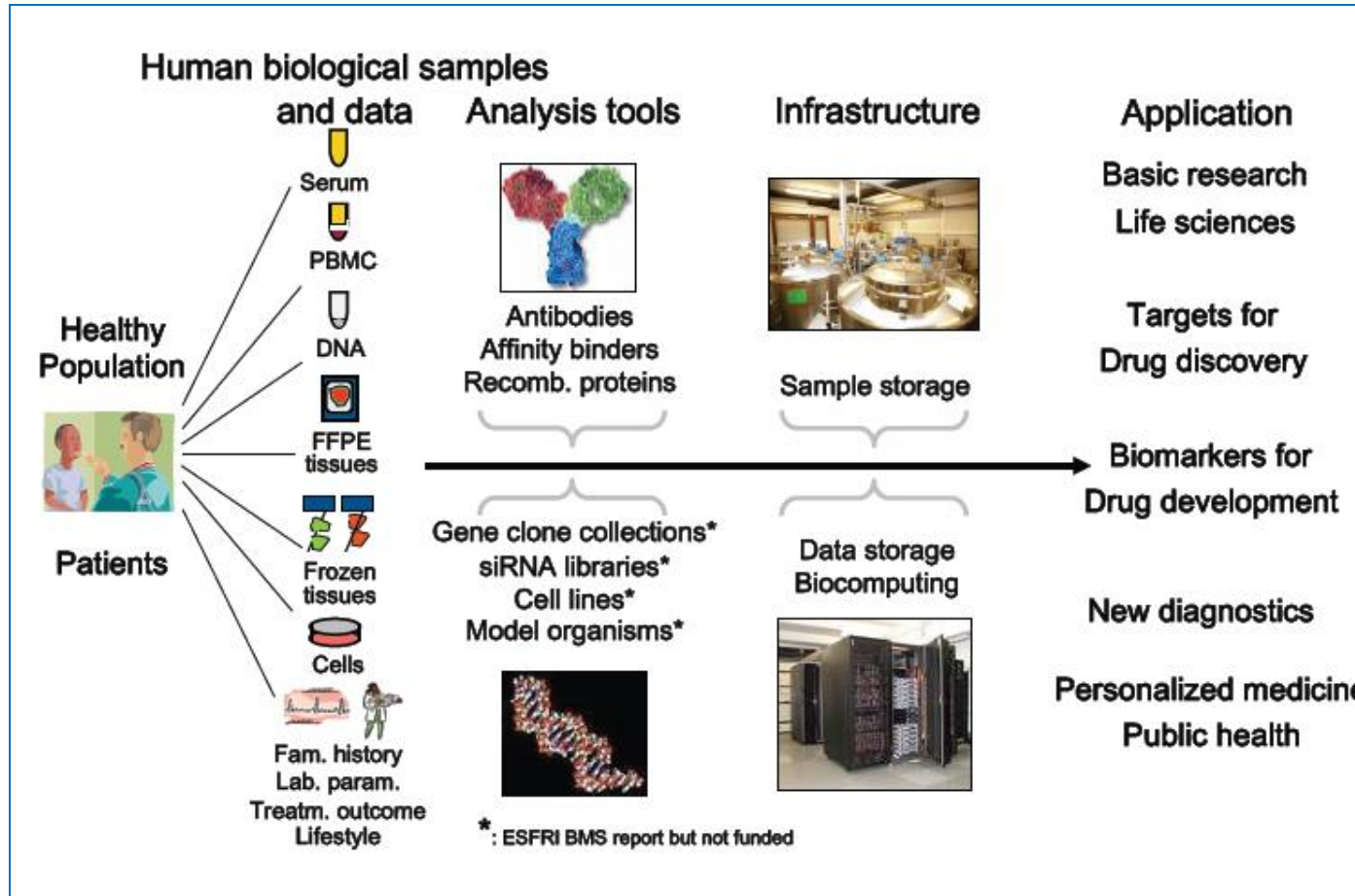


Biomarker Discovery, Development and Validation



<http://education.crdi.ie/page/g/s/91>

A biobank is a key area for clinical research













Availability of high quality, annotated patient and control samples

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limiting factor for research

Pediatric Biobanking: Kids Are Not Just Little Adults

Edited by Daniel R. Catchpoole , Authors: David Carpentieri , Suzanne Vercauteren , Lalita Wadhwa , William Schleif ,
Li Zhou , Junmei Zhou , Rania M. Labib , Elke Smits , and Engela H. Conradie 

Published Online: 5 Aug 2020 | <https://doi.org/10.1089/bio.2020.29071.djc>

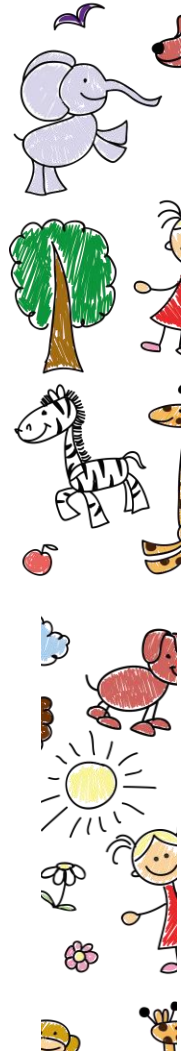
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Editor's Introduction

Pediatric biobanking brings a unique set of challenges and complexities to an already multifaceted system. To address these specific issues, an ISBER Pediatric Special Interest Group (SIG) was formed in September 2017. This pediatric biobanking SIG focuses on the complex and unique aspects of biobanking as related to pediatric biobank participants and pediatric samples. This SIG consists of members from New Zealand, Australia, China, Egypt, Belgium, United States, Europe, South Africa, and Canada who manage biobanking activities that have a focus on facilitating pediatric research. Many of these biobanks operate as embedded facilities within children's hospitals that presents a biobanking environment that requires management of biospecimens from a highly defined and vulnerable patient population.

We asked leading pediatric biobanks from across the world to tell us what makes pediatric biobanking special?

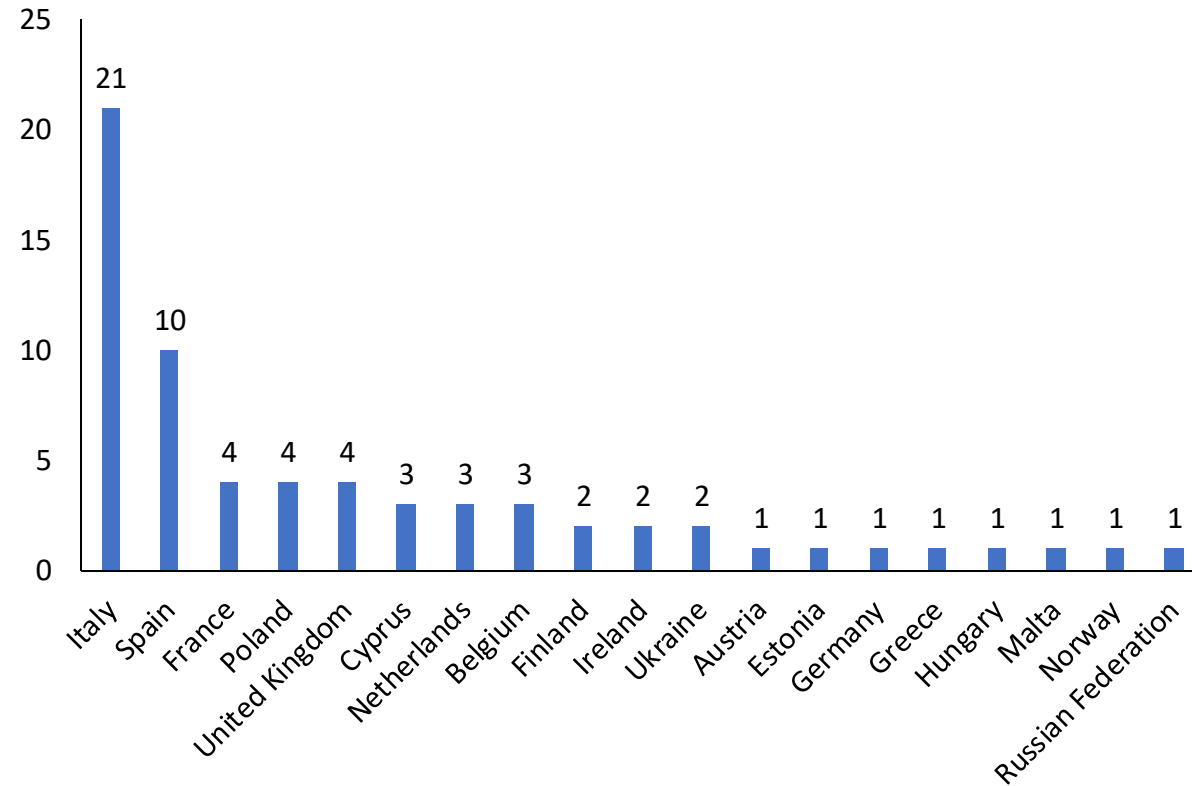


Services by the Paediatric Biomarkers and Biosamples TRP

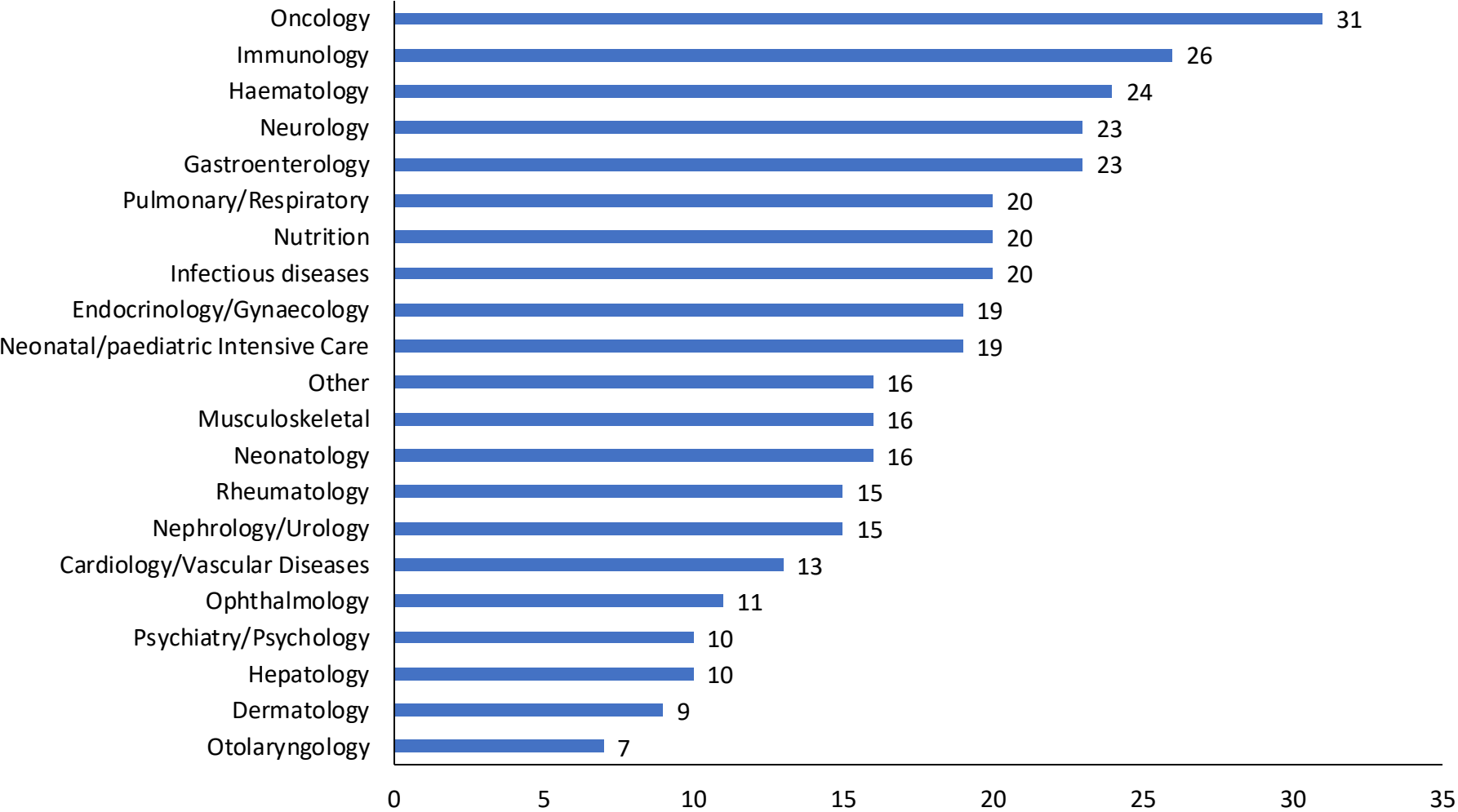
- Biobank organisation (targeted on paediatrics)
- Access to/deposit of human paediatric biological samples and data (biobanks)
- Identification/ characterisation of the biomarkers for paediatric use (incl. omics technologies)
- Validation process of the biomarkers for paediatric use

Research Units-Location

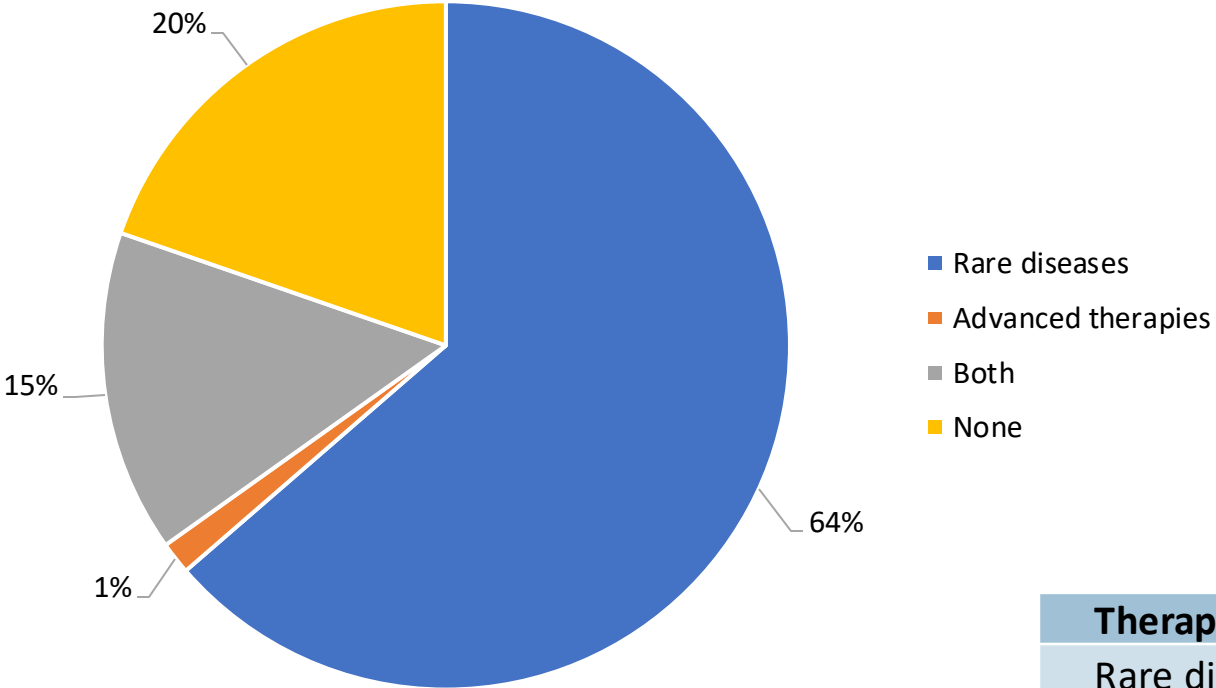
- 66 RU with interest in the B&B TRP



Research Units-Therapeutic area

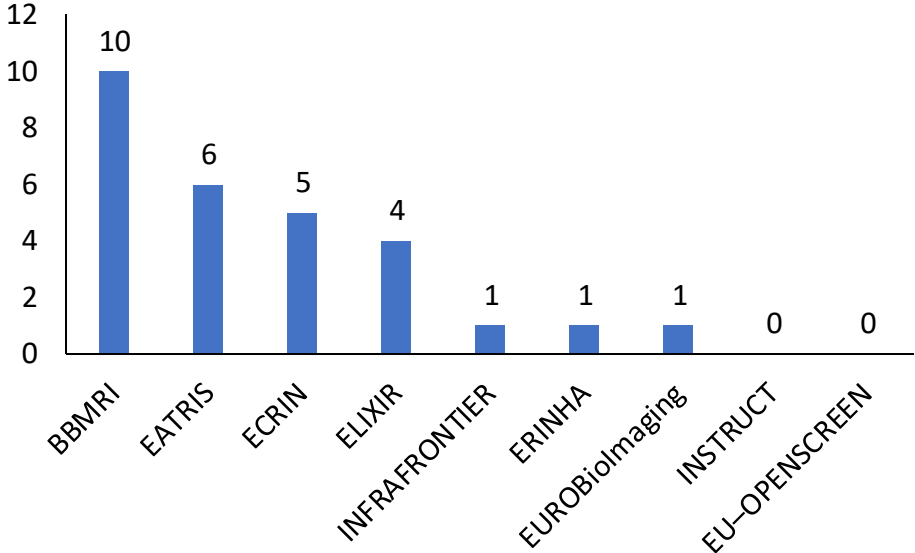
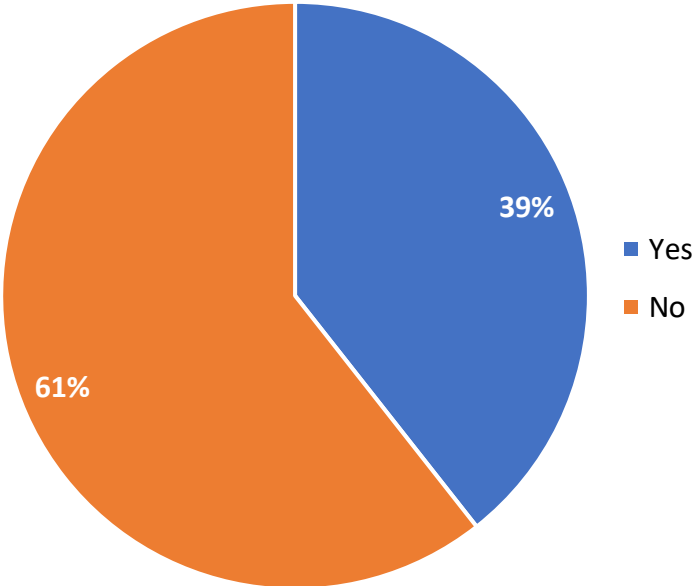


Research Units-Therapeutic field

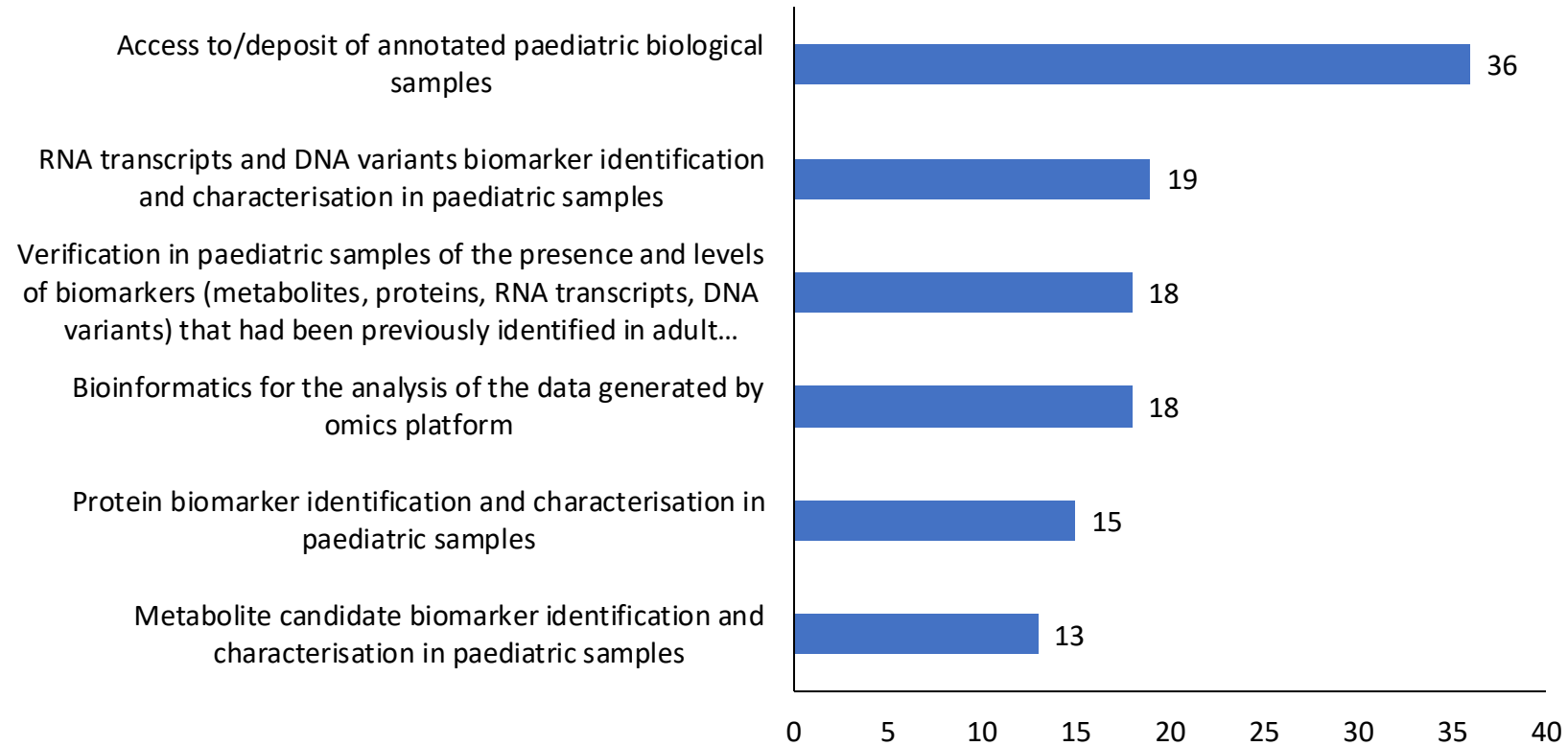


Therapeutic Field	RUs
Rare diseases	42
Advanced therapies	1
Both	10
None	13

RU-Members of a European Infrastructure



RU- Services



Research Units

Based on results of initial EPTRI surveys and specific TRP surveys (Old Data)

Before the establishment of the AISBL and little communication with the exception of a few close collaborators.

We need to re-engage under the new terms of AISBL and determine **commitment for participation** in EPTRI AISBL.

We need to:

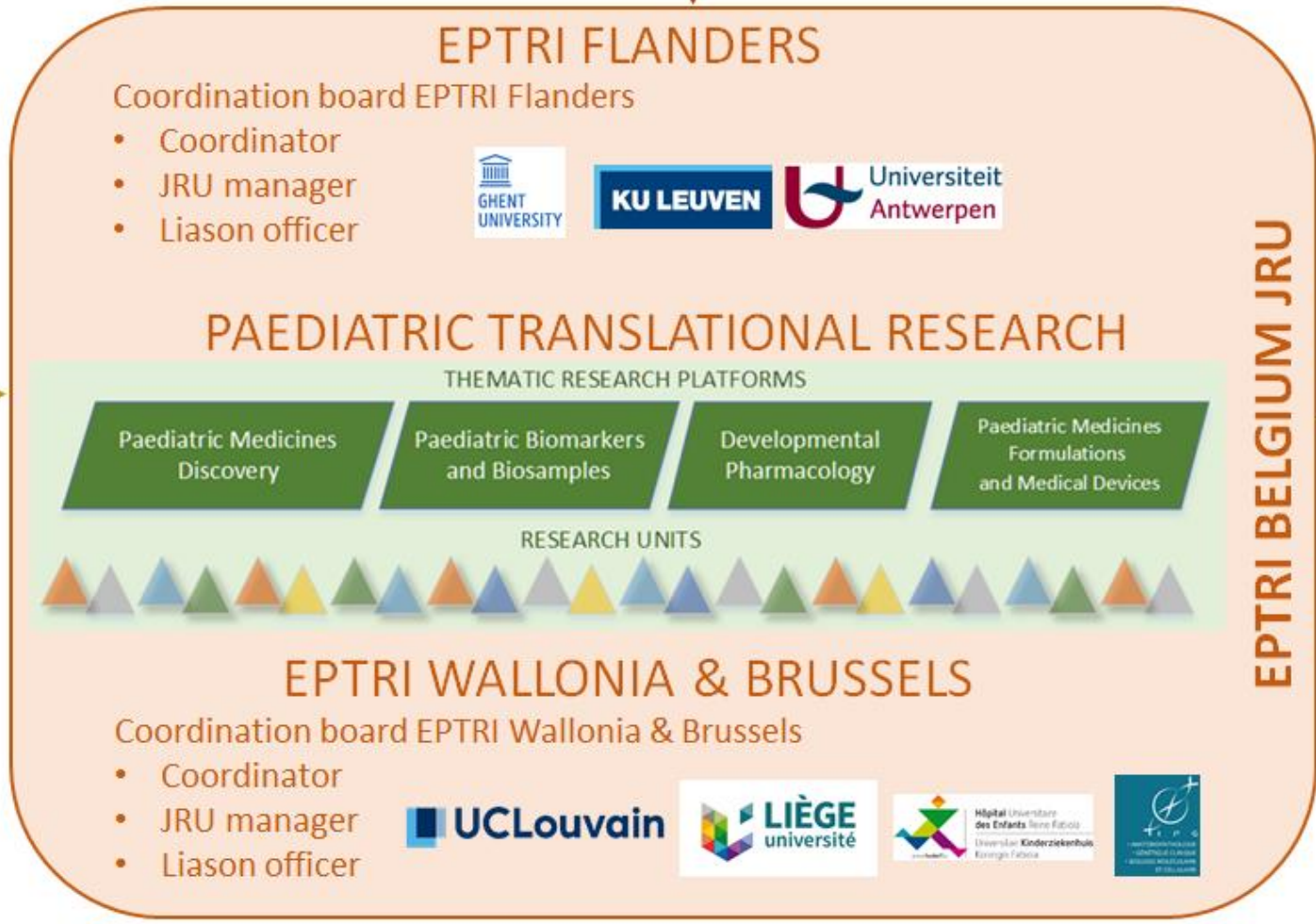
1. Inform them of the new developments
2. Provide well-defined benefits of participation (as a service provider and user **within** EPTRI AISBL) as well as clear terms and conditions for participation, clear set of procedures
3. Clearly define services they wish to provide: list of services, volume, time frames, service costs, QC, internal structures and procedures

Belgian national EPTRI Joint Research Unit (JRU)



- 1 UZ Gent & UGent
- 2 UZA & UAntwerpen
- 3 HUDERF & ULB
- 4 Cliniques Universitaires Saint-Luc & UCLouvain
- 5 UZ Leuven & KU Leuven
- 6 CHU Liège & ULiège
- 7 Institut de Pathologie et de Génétique (IPG)

The **Belgian national EPTRI Joint Research Unit (JRU)** involves paediatric research organisations and Universities/University Hospitals from Flanders, Brussels and Wallonia



QUALITY ASSURANCE IN PAEDIATRIC BIOBANKING

The Framework

- Legal
- Quality
- Focus
- Integration

The Patient

- Hospital brochures
- Consent and opting out

Samples and Data

- Harmonized procedures
- Harmonized storage
- Central Biobank Inventory
- Minimal Patient Data Set (MDS)

IMPACT & ACHIEVEMENTS



- ✓ To **be part of a distributed European Research Infrastructure** able to better coordinate and foster cooperative efforts integrating existing expertise in the paediatric research
- ✓ To **reduce fragmentation of the paediatric research landscape** through harmonisation of procedures, implementation of common standards and the promotion of scientific excellence
- ✓ To **enhance the paediatric research** and to implement the Key Enabling Technologies (KET) for drug development, Clinical Trials & Biobanking
- ✓ To **improve in the long term the health conditions of children**

