

WP3 SURVEY REPORT OF RESULTS

1. EPTRI PROJECT

European Paediatric Translational Research Infrastructure (EPTRI) is a research project aimed to create the framework for the EPTRI Research Infrastructure (RI) aimed to enhance technology-driven paediatric research in discovery and early development phases to be translated into clinical research and paediatric use of medicines.

2. WP3 SURVEYS

In order to collect different information clarifying the scientific and technological context for paediatric research in the European landscape and to identify potential service providers to be included in the future EPTRI a first online survey has been delivered in April-May 2018 in the framework of Task 3.5 Collection of data from the survey. More than 900 contacts have been invited in this survey round to fill out the online questionnaire and 137 answers were got from 29 countries, with Italy, UK and Spain being the most represented countries. Survey results have been detailed in Deliverable 3.5 - Report on survey outcomes.

A second round of the survey has been delivered from January to March 2019 in order to reach the relevant units and leading experts and better focus on all the services that will be provided in the European Paediatric Translational Research Infrastructure. Additional 103 answers were got from 24 countries, with Italy, Germany and Hungary being the most represented countries. Survey results have been considered as extrapolated on 11 March 2019. The survey is still ongoing and the additional results will be considered for the setting up of the platforms.

2.1 SECOND ROUND SURVEYS DEVELOPMENT

The first questionnaire has been used as basis to derive four different questionnaires by focusing on the specific EPTRI scientific domains:

- Human development and Paediatric Medicines Discovery (WP5);
- Paediatric Biomarkers and Biosamples (WP6);
- Developmental Pharmacology (WP7);
- Paediatric Medicines Formulations and Medical Devices (WP8).

Revision and changes have been agreed by WP Leaders. Moreover, the list of services in section 2 has been updated according to the focus of the survey and specific modifications have been included in each questionnaire according to the platform needs.



2.2 MAP OF UNITS

The existing map of units, as identified during the first phase of the project and described in Deliverable D3.2 “Completion of identification of paediatric research units for survey”, has been implemented with previous contacts classified by WP; direct WP leaders indications; additional units from helpdesk and specific request to survey respondents.

Moreover, a literature search has been performed.

Each contact has been linked to a specific WP according to its expertise (preliminary search has been performed) and contacts have been asked to indicate any additional field of expertise.

About 900 contacts have been asked to fill in the second round of the survey. In detail, contacts have been classified by country (table 1 and table 2).

Table 1. Contacts by country (1st round)

COUNTRY	NUMBER OF CONTACTS
Italy	171
Spain	149
UK	107
Germany	100
France	99
The Netherlands	78
Switzerland	27
Austria	25
Belgium	24
Czech Republic	20
Israel	20
Russia	20

OTHERS: Albania, Bulgaria, Croatia, Cyprus, Denmark, Estonia, Finland, Greece, Hungary, Iceland, Ireland, Latvia, Lithuania, Luxembourg, Malta, Norway, Poland, Portugal, Romania, Serbia, Sweden, Ukraine

Table 2. Contacts by country (2nd round)

COUNTRY	NUMBER OF CONTACTS
Italy	217
Germany	113
France	105
UK	85
Spain	82
The Netherlands	69
Poland	53
Switzerland	29
Belgium	25
Hungary	25
Austria	18
Czech Republic	18
Greece	18

OTHERS: Sweden, Finland, Portugal, Israel, Romania, Ireland, Norway, Denmark, Estonia, Russia, Cyprus, Lithuania, Iceland, Slovenia, Malta, Croatia, Serbia

2.3 SECOND ROUND SURVEYS DATA EXTRAPOLATION

Survey preliminary results have been extracted three times from February 4th to March 13th and shared among WP3 team and other WPs Leaders. The results considered for the report are those extracted on March 11th.

A total of 103 questionnaires have been extracted. For evaluating the results the same analysis of the first round of the survey has been performed: an excel file has been used to facilitate the counting of given answers; the mean has been calculated for answers provided as a number on a 5 point scale; the free text answers have been merged where possible and if a merging was not appropriate they have been added at the end of each result. Diagrams have been built for each question displaying the results. Duplicates with the first survey round have been removed and multiple questionnaires from the same research group merged.

The results were sent to work package leaders, responsible for the platforms design, in order to ask them for contribution and to draw conclusions on the impact for each platform.

3. SURVEY RESULTS

Paediatric research units to be considered in EPTRI setting up process have been derived from the analysis of the 2018 WP3 survey results implemented with the results of the second survey collected (up to 11 March 2019). The number of adhering centres has been defined through a cleaning procedure of survey respondents. Duplicates have been removed, incomplete surveys have been checked and validated, different research groups belonging to the same Institution have been identified while respondents belonging to the same Institution and the same research group have been merged.

A total of 242 research units resulted at the end of this process. Table 3 summarises the distribution of respondents per platform as a sum of the first and second rounds of the survey, the number of represented countries and the number of adhering centres. The highest response rates correspond to the platforms of basic research and of paediatric biomarkers, while the lowest number of research units correspond to the formulation science platform.

PLATFORM	NUMBER OF RESPONDENTS (1 st round)	NUMBER OF RESPONDENTS (2 nd round)	NUMBER OF REPRESENTED COUNTRIES	NUMBER OF RESEARCH UNITS*
<i>Human Development and Paediatric Medicines Discovery</i>	70	34	20	82
<i>Paediatric Biomarkers and Biosamples</i>	49	25	20	73
<i>Developmental Pharmacology</i>	27	28	19	52
<i>Paediatric Medicines Formulations and Medical Devices</i>	22	16	15	35
TOTAL				242
<i>*duplications have been removed</i>				

Table 3. Distribution of respondents per countries

Figure 1 shows the distribution of proposed research units by country and platform. In particular more than 5 research units have been identified in 17 countries, and more than 10 in 6 countries.

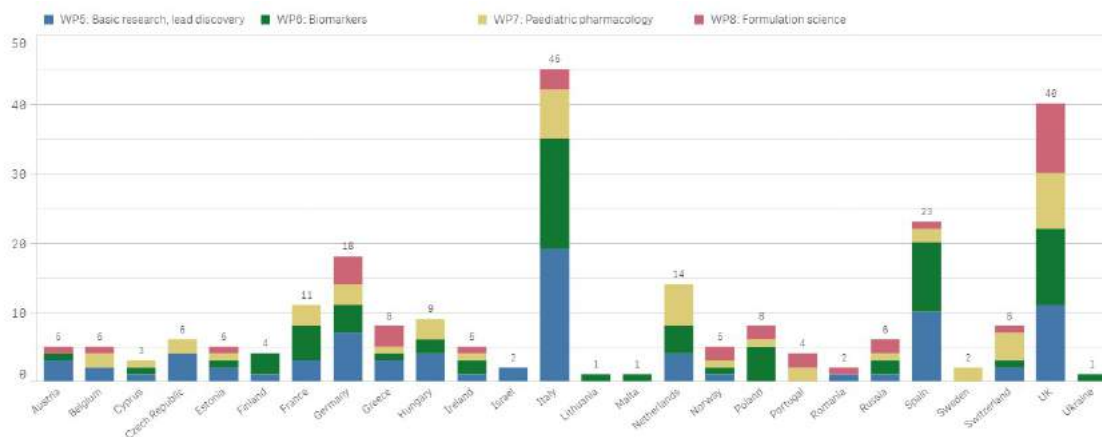


Figure 1. Distribution of proposed research units by country and platform

Details on the geographical distributions of research units and on the proposed services to be provided within each platform are summarised below:

➤ **WP5 – Human Development and Paediatric Medicines Discovery**

The most represented countries in the Human development and Paediatric Medicines Discovery platform are Italy (19 units), United Kingdom (11 units) and Spain (10 units). 20 countries are globally involved, one country (Romania) has been added in the second round of the survey. Three non-EU countries have contributed to the survey (Russia, Israel, Albania).

The list of services derives from both rounds of WP3 survey and a consultation for centers participating in the survey filtered by the WP leaders is still ongoing in order to add useful information on services, facilities and laboratories to be provided within the platform. Moreover, the internal consultation at WP5 platform level will allow the identification of two main area of research activities - Research on Biomodel and Research on Chemical Compound - and the Institutions performing activities in only one area or in both the areas. (Figure 2)

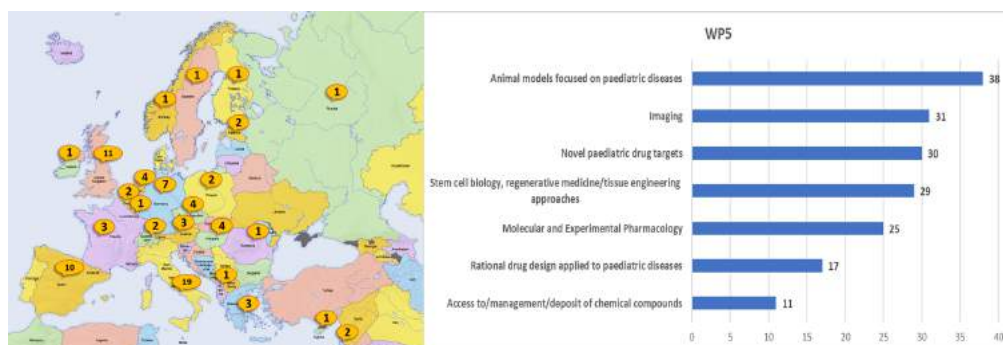


Figure 2. WP5 - Research units and proposed services

➤ **WP6 - Paediatric Biomarkers and Biosamples**

The most represented countries in the Paediatric Paediatric Biomarkers and Biosamples platform are Italy (16 units), United Kingdom (11 units) and Spain (10 units). Importantly, three countries (Hungary, Malta and Switzerland) have been added after the first round of the survey. Three non-EU countries have also declared interest in this Platform (Russia, Switzerland and Ukraine).

The list of services derives from both rounds of WP3 survey. Moreover, the following services have been indicated by the WP6 leaders in order of prioritisation: validation of biomarkers for paediatric use, access to/deposit of annotated human paediatric biological samples and identification/characterisation of the biomarkers for paediatric use. Technologies and facilities necessary to provide the above mentioned services and expertise within the platform are detailed consisting in: omics technology platforms, high-throughput screening facilities and biobanks. (Figure 3)

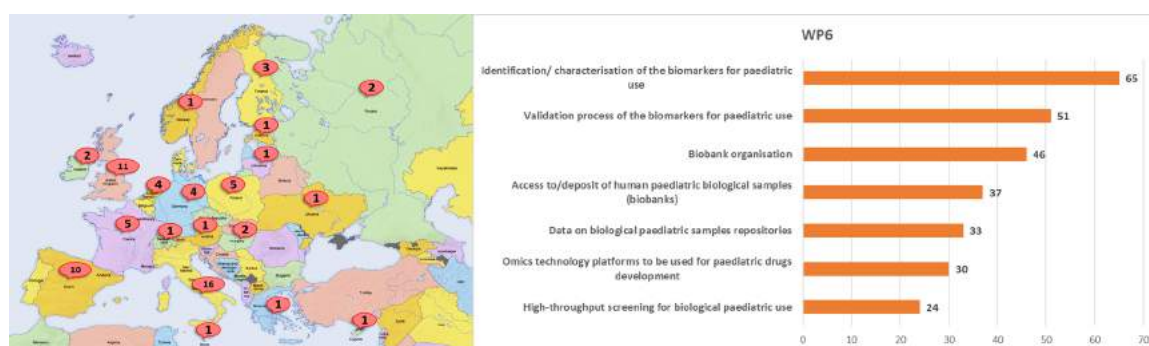


Figure 3. WP6 - Research units and proposed services

➤ **WP7 – Developmental Pharmacology**

The most represented countries in the Developmental Pharmacology platform are United Kingdom (8 units), Italy (7 units) and Netherlands (6 units). Importantly, six countries (Russia, Hungary, Switzerland, Cyprus, Czech Republic and Greece) have been added after the first round of the survey. The services to be provided within the Developmental Pharmacology platform, deriving from WP3 survey results, have been revised according to classification in Deliverable 7.6 - Description of the research activities to be provided within the developmental pharmacology platform. (Figure 4)



Figure 4. WP7 - Research units and proposed services

➤ **WP8 - Paediatric Medicines Formulations and Medical Devices**

The most represented countries in the Paediatric Medicines Formulations and Medical Devices platform are United Kingdom (10 units), Germany (4 units) and Italy and Greece (3 units). Importantly, five countries (Austria, Belgium, Romania, Russia and Switzerland) have been added after the first round of the survey. This list of services derives from both rounds of WP3 survey. (Figure 5). Moreover, additional information on medical devices has been asked during the second round of the survey.



Figure 5. WP8 -Research units and proposed services