EPTRI

European Paediatric Translational Research Infrastructure

EPTRI organisation of services, data and access

Fedele Bonifazi – Fondazione G. Benzi onlus

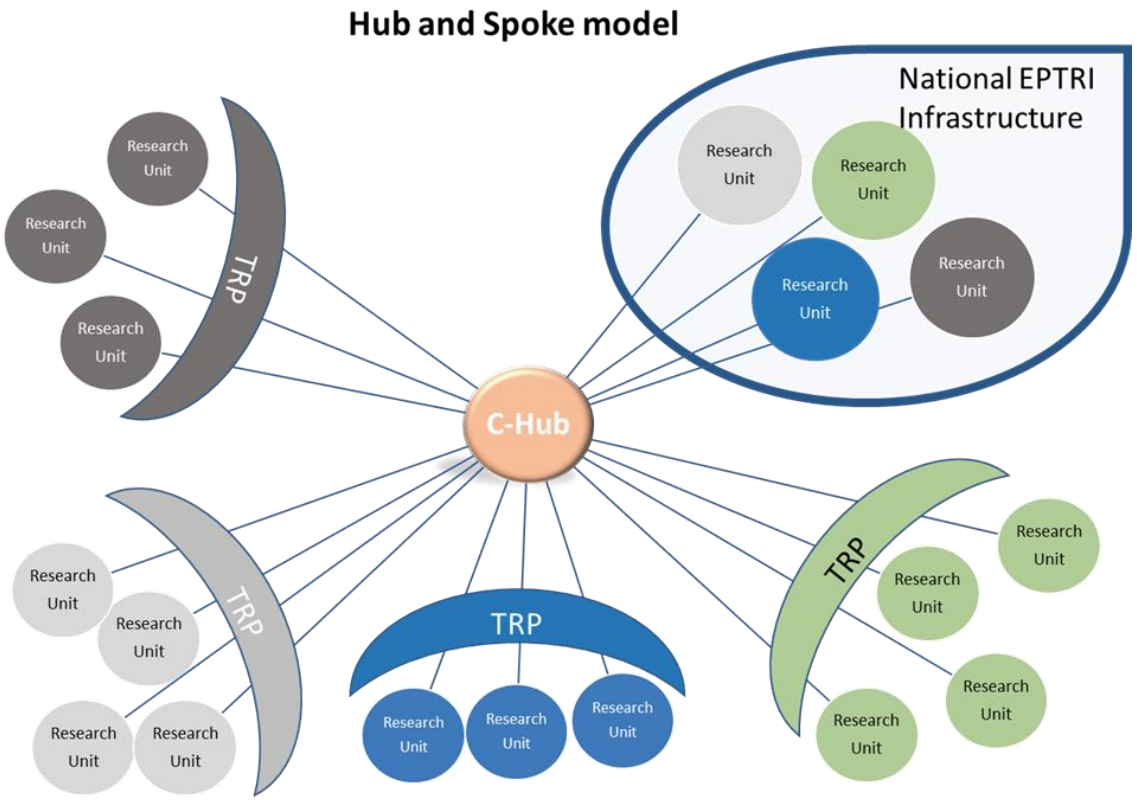
EPTRI Stakeholders Roundtable

Virtual Meeting July 9th, 2020

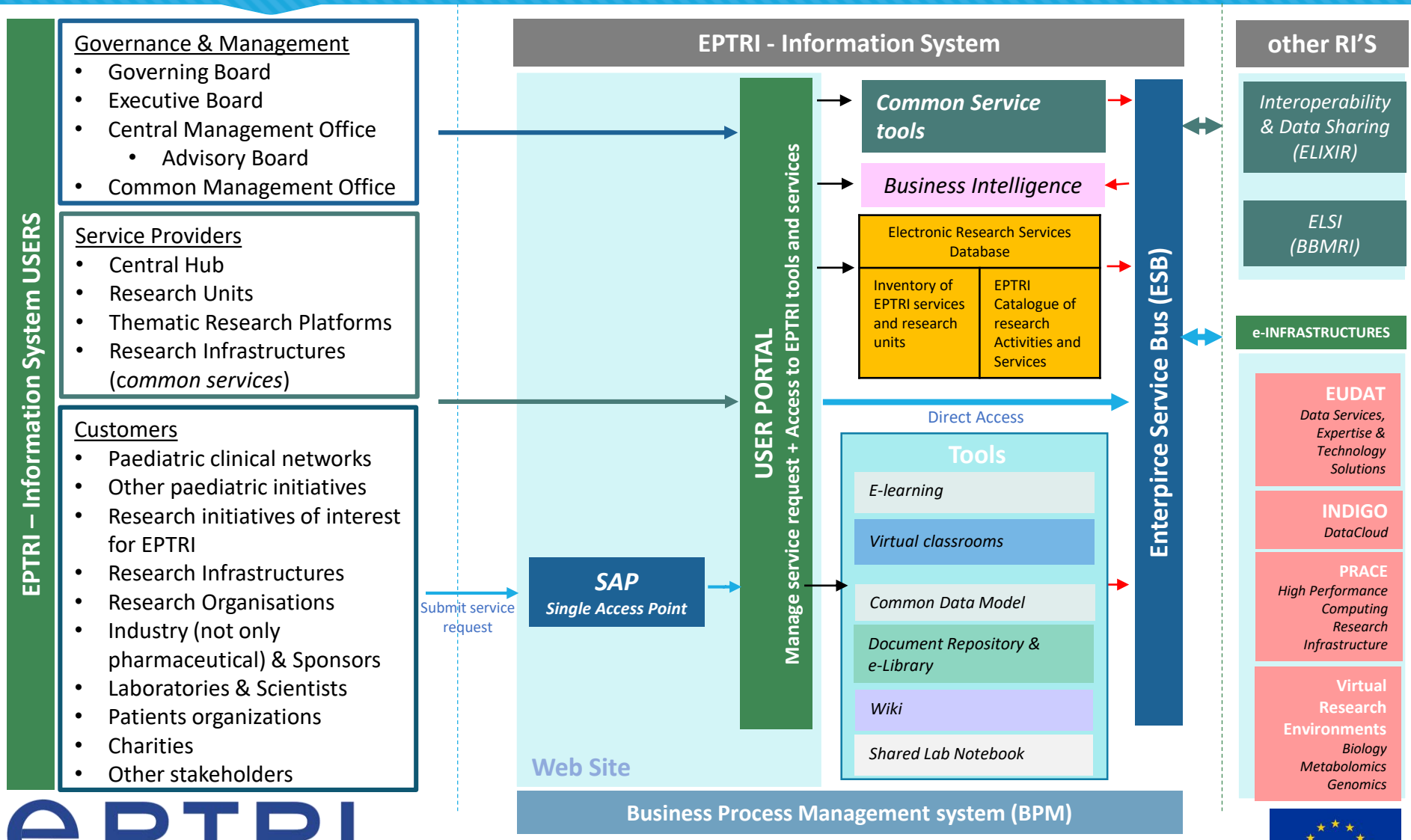
This project has received funding from the European Union's Horizon 2020 research and innovation programme under Grant Agreement No 777554

EPTRI Organisational Model

HUB-and-SPOKE organisation, with a **Central Hub** and several Spokes, represented by the Thematic Research Platforms (**TRPs**) and the national nodes N-EPTRIs (**National EPTRI Infrastructures**) in each member country partner of EPTRI

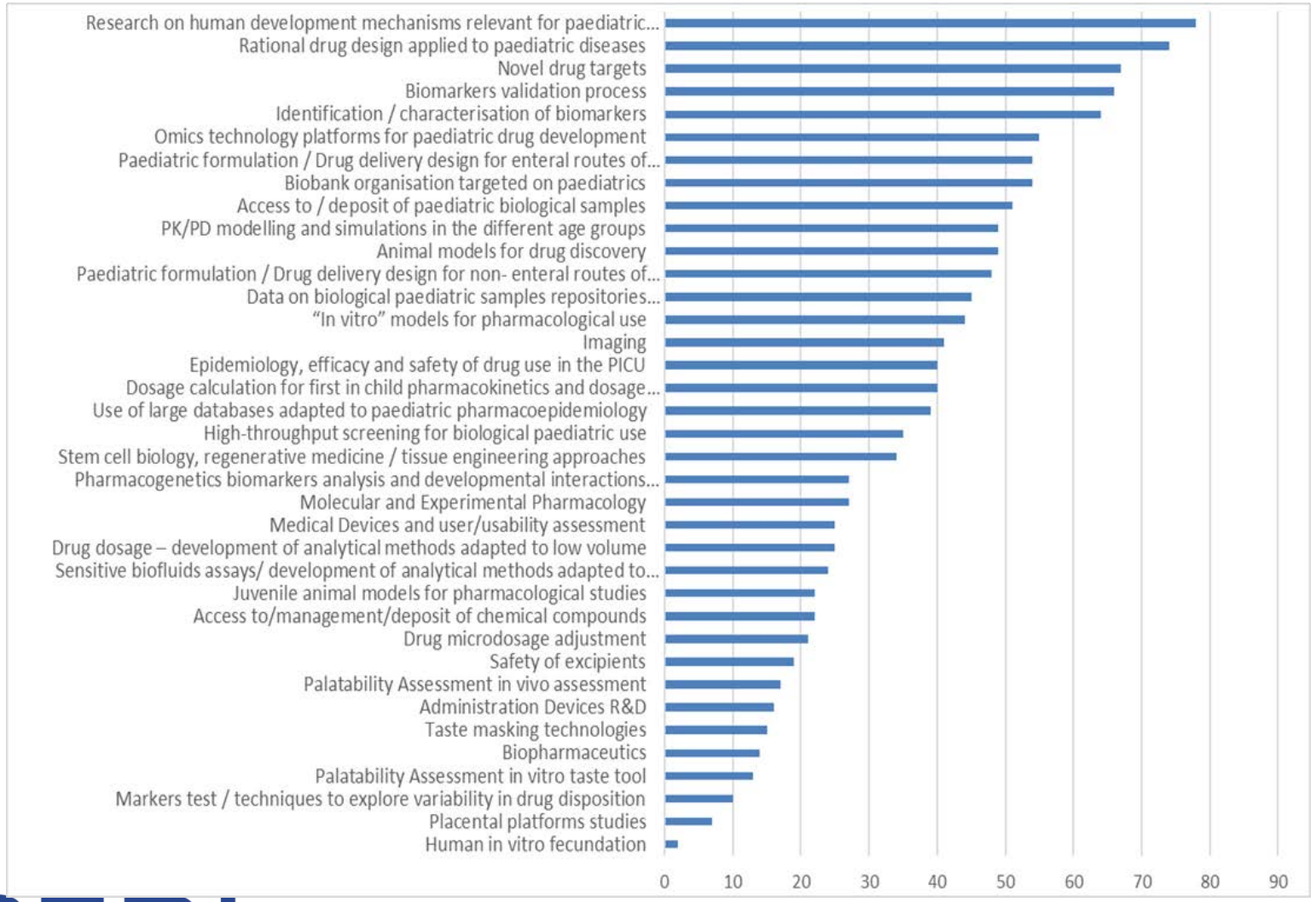


EPTRI high-level IT architecture



EPTRI
European Paediatric Translational Research Infrastructure

Plan of services based on relevant Gaps



ePTRI
European Paediatric Translational Research Infrastructure

Priority list of services to be provided in EPTRI according to users' needs

EPTRI Stakeholders Roundtable – 09/07/2020

Plan of services

Users will have access - through the Single Access Point (SAP) - to three types of services:

a) **integrated services**, provided by the RUs organised in TRPs;

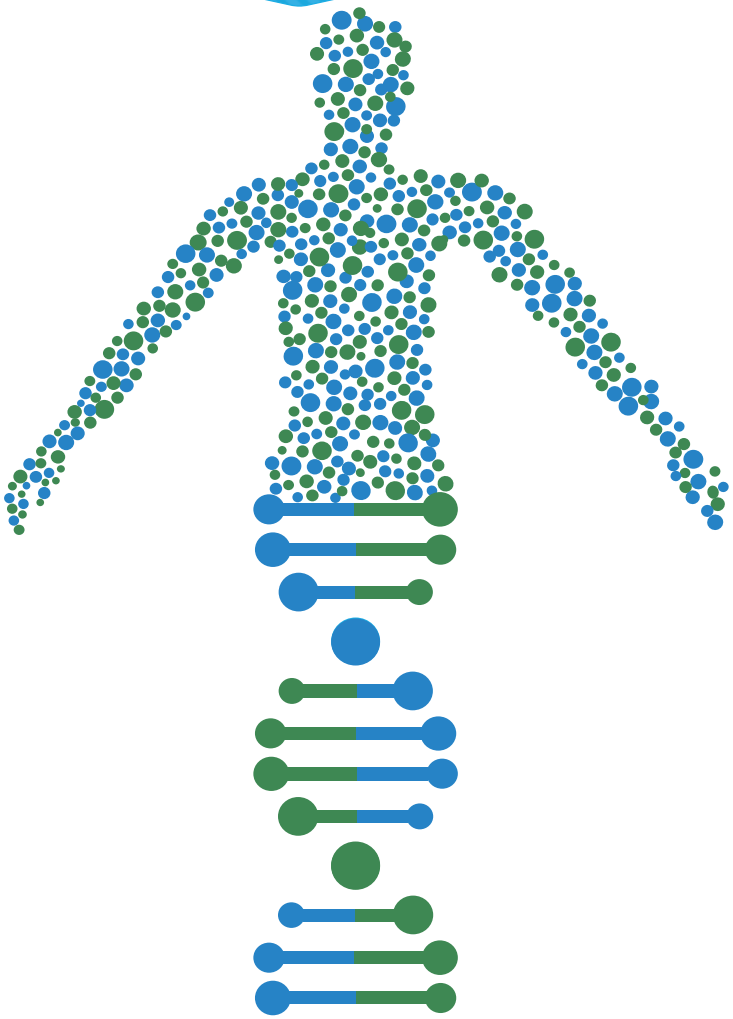
b) **centralised services**, managed and provided at Central Management Office (CMO) level;

c) **common services**, provided in collaboration with other Research Infrastructures.

Integrated Services

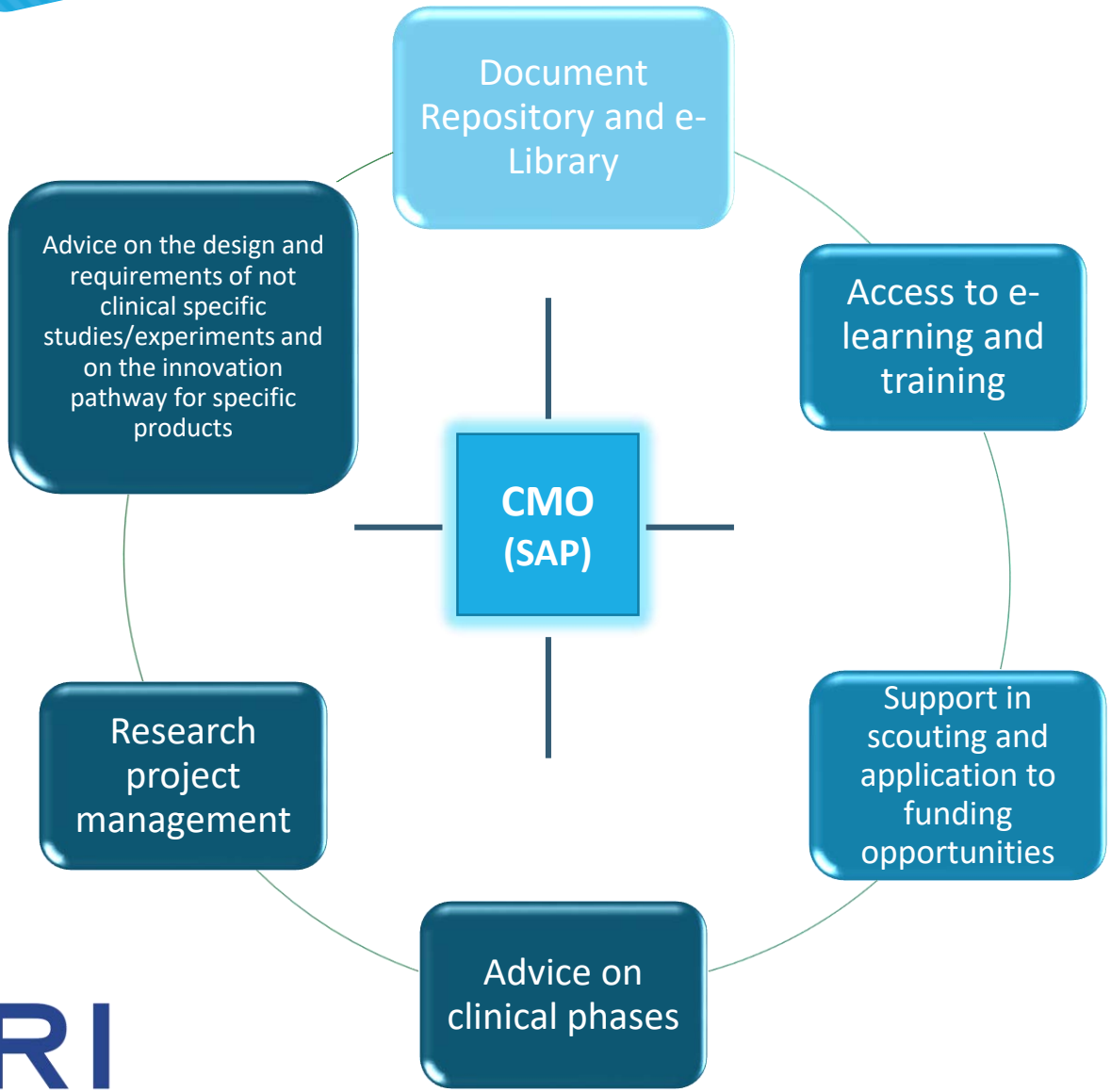
Paediatric Medicines Discovery TRP	Paediatric Biomarkers and Biosamples TRP	Developmental Pharmacology TRP	Paediatric Medicines Formulations TRP
82 RUs & 19 Countries	***78 RUs & 22 Countries***	***28 RUs & 12 Countries***	***28 RUs 28 & 12 Countries***
In vitro screening of novel drugs using paediatric cellular targets	Access to/deposit of annotated paediatric biological samples	Microdosing to establish the "in vivo" PK profile of the new drug	Pre-formulation advice and Pre-formulation studies
In vitro pre-clinical studies (effect, efficacy, biomarkers, etc.) in paediatric cell models	RNA transcripts and DNA variants Biomarker identification and characterisation in paediatric samples	In vitro models to study ontogeny of drug disposition	Formulation of drug for paediatric use for enteral routes of administration
Access to the neonatal and juvenile animal models to screen novel drug for a paediatric specific target	Protein Biomarker identification and characterisation in paediatric samples	Placental studies	Formulation of drug for paediatric use for non-enteral routes of administration
Access to the neonatal and juvenile animal models to perform preclinical studies	Metabolite candidate Biomarker identification and characterisation in paediatric samples.	In vivo toxicity juvenile animal studies	Assessment and design of drug delivery systems for enteral and non-enteral routes of administration
In silico screening of novel drugs for specific paediatric targets	Bioinformatics for the analysis of the data generated by omics platform	Paediatric ADME and modelling and simulation	Paediatric in vitro palatability assessment
In silico prediction of ADME properties & toxicity	Verification in paediatric samples of the presence and levels of biomarkers	Sensitive analytical methods adapted to paediatrics	Paediatric in vivo palatability assessment
Scientific advice on specific	Scientific advice on specific		

Centralised Services



Services managed at Central Management Office (CMO) level and provided through the Single Access Point (SAP)

Centralised Services

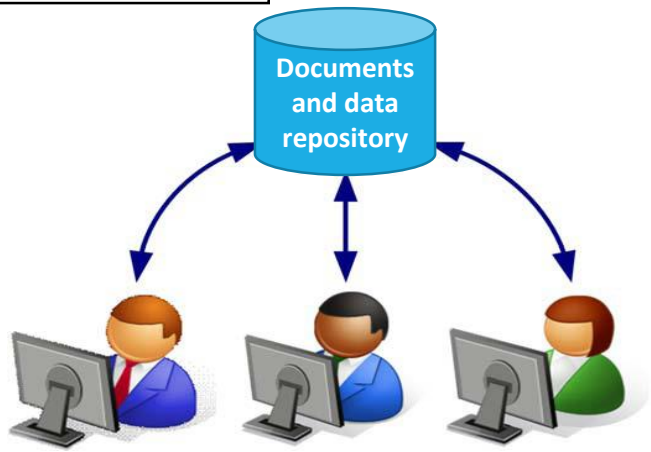


ePTRI
European Paediatric Translational Research Infrastructure

Centralized Services

Document Repository and e-Library

This will provide users with a centralised **e-library** for storing, archiving and retrieving documents and data produced as a result of research activities.



Access to e-learning and training

Contents:

- ✓ provided from TRPs leads
- ✓ Covering EPTRI main topics
- ✓ Taking into account training needs explored in the survey

The training contents could be delivered in several ways: **webinars, online training, face to face courses, staff exchanges.**

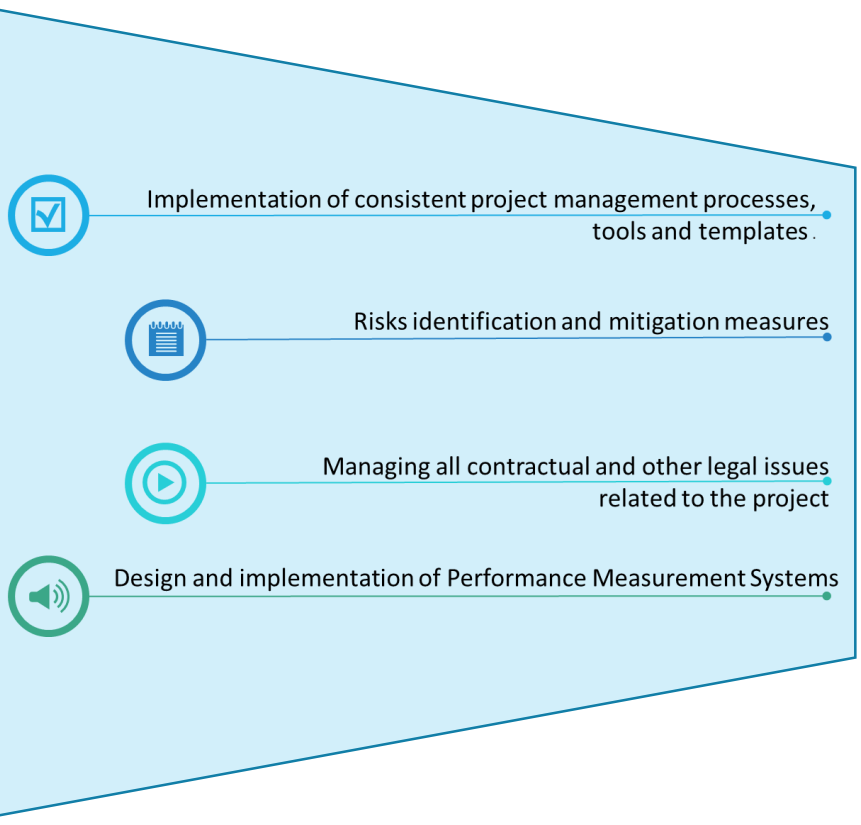
Centralized Services

Support in scouting and application to funding opportunities

Aims at identifying possible grants opportunities in research areas related to EPTRI activities and providing support to apply for such funding.

- Scouting funding opportunities
- Support for writing applications
- Virtual notice board

Research project management



Centralized Services

Advice on the design and requirements of not clinical specific studies/experiments and on the innovation pathway for specific products

1. Consultancy on technical design of not clinical experiments in a plan of development for specific studies.
2. Consultancy on innovation pathway for specific product.s
3. Consultancy on Preparation of Regulatory applications and queries from the Regulatory Agencies.
4. Consultancy on Exploitation and Intellectual Property Rights (IPR) valorization.

Advice on clinical phases to facilitate EPTRI results translation into paediatric clinical research

1. Dialogue between pre-clinical and clinical expertise
2. Information sharing
3. Introductions to expertise
4. Mediating collaborations with clinical resources

Common Services

ELSI paediatric service

This service will deal with the relevant ethical, social and legal problems arising in the field of paediatric translational research (from pre-clinical phase to access policies and training) and will include follow up of **European/national legislations/regulations/guidelines** aimed at providing with **ethical and legal advice** on specific issues.
To be developed also in collaboration with **BBMRI.**

Paediatric data interoperability service

This service is aimed to support **use and re-use** of data for research purposes, focused on discovery, access, integration and analyses of biological data from a paediatric point of view in collaboration with **ELIXIR**.
Users could access tools to facilitate **sharing and re-use of data according to the FAIR principles**.

Integrating EPTRI and other RIs: identification of possible area for collaboration

Research areas and services delivered by EPTRI	RI providing similar services	EPTRI platforms involved
In silico screening of novel drugs for specific paediatric targets	**EATRIS INSTRUCT**	**Paediatric Medicines Discovery**
Animal models for pharmacological studies	**EATRIS INFRAFRONTIER ELIXIR**	**Paediatric Medicines Discovery**
In vivo toxicity juvenile animal studies	**EATRIS INFRAFRONTIER**	**Developmental Pharmacology**
Access to (paediatric) biosamples and related data	**EATRIS BBMRI ELIXIR**	**Paediatric Biomarkers & Biosamples**
Analysis of omics platforms data	**EATRIS BBMRI**	**Paediatric Biomarkers & Biosamples**
Modelling and simulations in the different age groups	**EATRIS**	**Developmental Pharmacology**
Microdosing and Sensitive analytical methods adapted to children	**EATRIS**	**Developmental Pharmacology**
Paediatric formulation/drug delivery design for enteral/non-enteral routes of administration and Biopharmaceutics	**EATRIS**	**Paediatric Formulations and Medical Devices**

Integration Working Group Conclusion

- ✓ EPTRI should be considered as complementary to other existing RIs as it is aimed to covering an existing gap that is not currently assumed by other RIs;
- ✓ The landmark RIs can usefully support EPTRI in the implementation of some relevant technologies to develop paediatric medicines.
- ✓ A limitation of the services to be provided by EPTRI is foreseen in case the same services are already available through another landscape RI in order to avoid duplications;
- ✓ Setting up legal agreements establishing a collaborative management of some services of paediatric interest with other landscape RIs.
- ✓ For technologies and services **of common application** that need to be adapted from the general to the paediatric settings **Common Services** would represent the right solution.

EPTRI
European Paediatric Translational Research Infrastructure

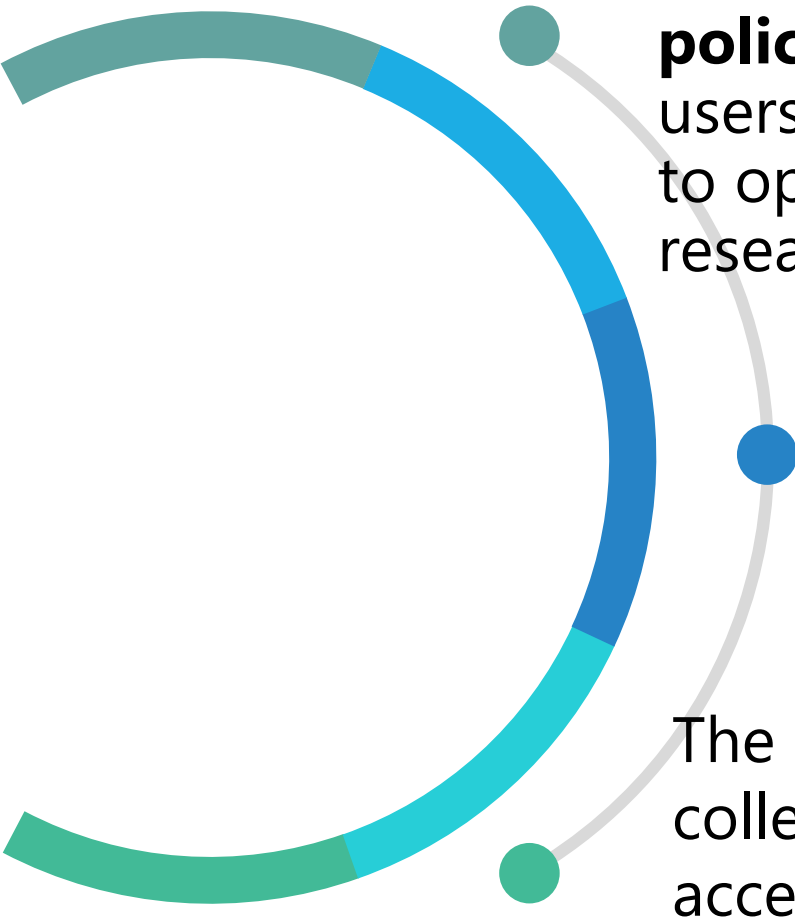
EPTRI Access to services

Access to services will be based on a well-defined procedure: **application, evaluation, feedback to users' service requests, selection, admission, negotiation, setting-up, service provision and monitoring.**

Access will be facilitated through:

- Single Access Point
- Service Desk
- Users Guide

Single Access Point (SAP)

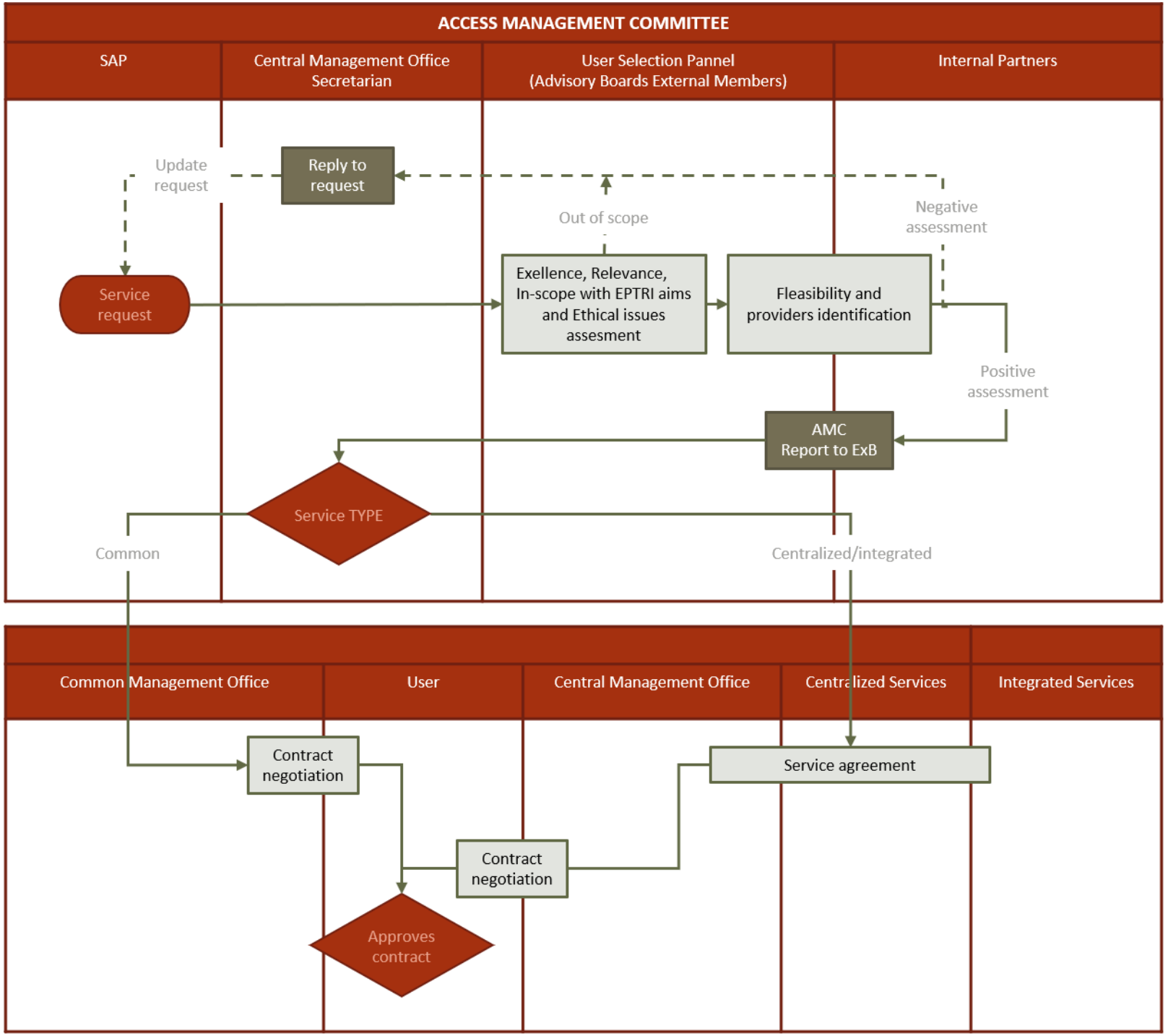


The central Hub provides a unique access policy and a single point of access for all users with a supporting structure dedicated to optimise the access for the proposed research

- The central Hub will host a **Single Access Point (SAP)** for all contacst and requests to EPTRI.

The SAP, through an online form, is the collector for all the requests aimed at accessing to ETPRI and its offered services.

ePTRI
European Paediatric Translational Research Infrastructure


ACCESS MANAGEMENT COMMITTEE
SAP
Central Management Office Secretarian
User Selection Pannel (Advisory Boards External Members)
Internal Partners
Update request
Reply to request
Out of scope
Negative assessment
Service request
Exellence, Relevance, In-scope with EPTRI aims and Ethical issues assesment
Fleasibility and providers identification
Positive assessment
AMC Report to ExB
Service TYPE
Common
Centralized/integrated
Common Management Office
User
Central Management Office
Centralized Services
Integrated Services
Contract negotiation
Service agreement
Contract negotiation
Approves contract

Services assignment

- **Contract between User and Central Management Office for the Centralised and the Integrated services**

EPTRI CMO prepares and signs an agreement with the relevant institution(s) providing the services, to establish criteria for service provision (terms of provision, standards, quality, an option to exit the agreement, …)

- **Contract between User and the dedicated Common Management Office set up in collaboration with one or more RI(s) in case of Common services**

A Common Management Office will be set up and will draft the contract to engage the provider(s) and the User, contract the revenues and the efforts (to be shared between EPTRI and the RIs) and the process timeline.

Data Management Plan of the future RI

EPTRI activities are considered **"data-intensive"** due to the expected production of large amounts of data. The continuous flow of data produced in EPTRI will need to be appropriately managed, stored, analysed and interpreted.

A Data Management Plan (DMP) to be adopted in the next step of EPTRI preparation/implementation has been prepared according to Horizon 2020 guidelines.

The DMP contains information on:

- ✓ types of data that will be produced and collected in the framework of the future EPTRI
- ✓ standards and tools to be used
- ✓ FAIRification
- ✓ allocation of resources
- ✓ ethical aspects

Data Management Plan of the future RI

DATA SOURCES	PURPOSE OF THE DATA COLLECTION
Data collection on the Research Units willing to participate to EPTRI as users or service providers	To collect interest and identify potential service providers and to create an Inventory of the EPTRI RUs able to provide specific services
Data from EPTRI Networking and outreach	Further enlargement of EPTRI is foreseen during the preparation and implementation phases. Data collected will be used to consolidate existing TRPs, to identify new TRPs, to set up National EPTRI-Infrastructures
Data collected at Central Hub level	To support coordination, management, networking, outreach and other Central Hub activities.
Research services provided in EPTRI	Reports from the research performed in EPTRI and other scientific documents provided by the RUs, the TRPs, and the centralised and common services, will represent the contribution of EPTRI to advance and disseminate knowledge. According to the inputs and specificities of each thematic platforms, or under users' specific requests, all these data from preclinical or clinical research will be stored and made available for future research.

Data Management Plan of the future RI

RESEARCH DATA SOURCE	TYPE OF DATA	STANDARDS
Results from the Inventory of RUs	Electronic documents -structured and unstructured	CSV, XLS
Coordination, Data Management, Networking, communication, scientific advices, outreach and other central Hub activities	Electronic documents -structured and unstructured	PDF, CSV, XLS, JPEG and metadata
Results from RUs providing services (TRP ***Paediatric Medicines Discovery)***	Electronic documents -structured and unstructured	PDF, CSV, XLS, JPEG and metadata
Results from RUs providing services aTRP (***Paediatric Biomarkers and Biosamples)***	Electronic documents -structured and unstructured	RDBMS, JPEG, PDF and metadata
Results from RUs providing services (TRP ***Developmental Pharmacology)***	Electronic documents -structured and unstructured	PDF, CSV, XLS, JPEG and metadata
Results from RUs providing services (TRP ***Paediatric Medicines Formulations and Medical Devices)***	Electronic documents -structured and unstructured	PDF, CSV, XLS, JPEG and metadata
Results from **centralized and common services** provided at C-HUB level	Electronic documents -structured and unstructured	PDF, CSV, XLS, JPEG and metadata

Exploited and/or shared/made accessible

↓

EOSC Data Sharing

Expected size of the data

↓

Up to 1TB per year

- ✓ **FAIR principles**
- ✓ **GDPR Compliance**
- ✓ **IPR issues**

ePTRI

European Paediatric Translational Research Infrastructure

Thanks for your attention

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