OrphaDev4Kids

EUROPEAN HEALTH AND DIGITAL EXECUTIVE AGENCY (HADEA)

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INSTYTUT „POMNIK-CENTRUM ZDROWIA DZIECKA"

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General objective: implement a complex innovation ecosystem in the orphan and paediatric MDs fields to maximise the value-for-patients of the research and development projects by supporting academy, research centres, and SMEs that invest on paediatric devices development.

Specific objectives

1. **Grant access to a paediatric MDs Platform for Users in need to receive dedicated research and development services.**

2. **Implement a complex ecosystem including tools, facilities, and supportive services.**

3. **Develop three orphan and paediatric case studies, one dealing with osteogenesis imperfecta and two concerning Cyanotic Congenital Cardiac Diseases.**

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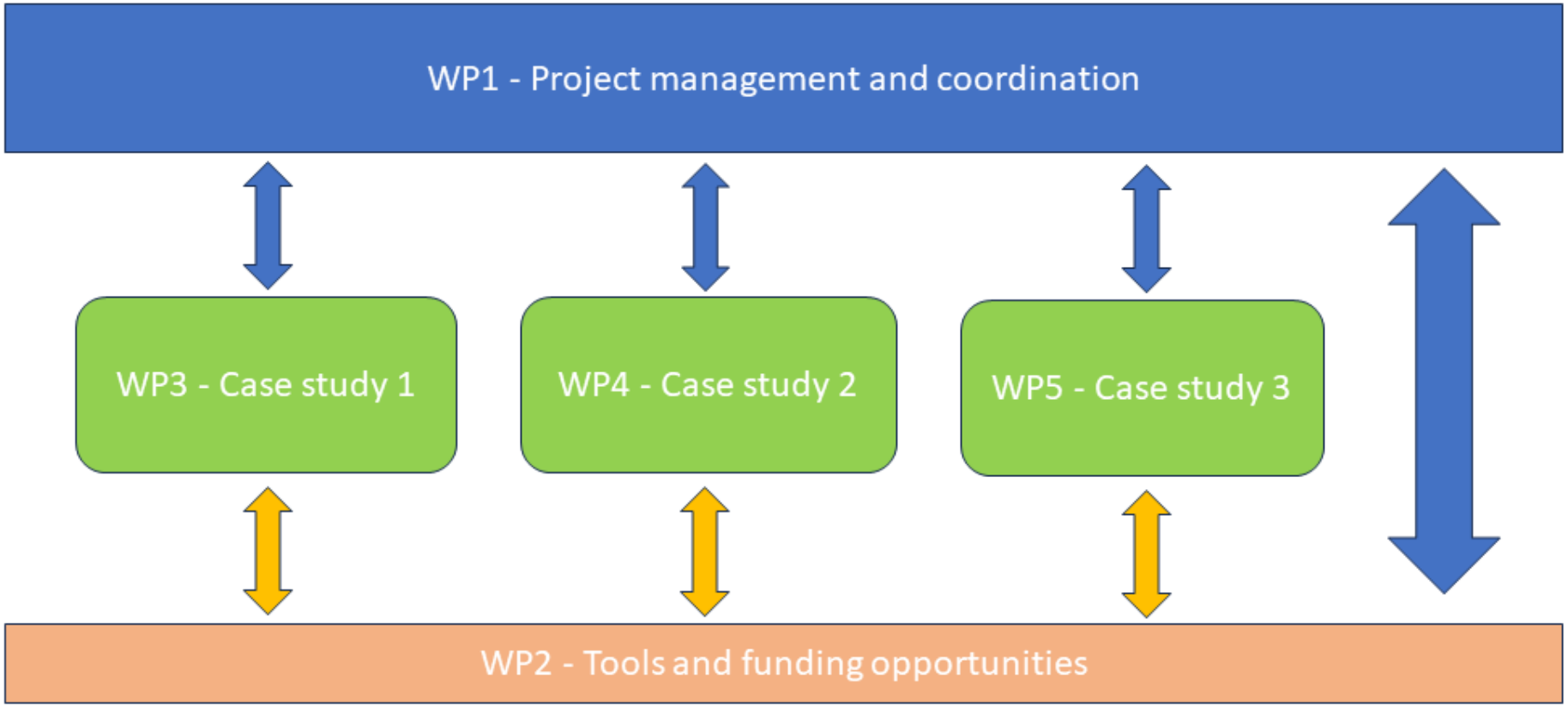
Workplan



The project is organized in 5 Work Packages
The duration is 36 months
The consortium is composed of 8 partners, including EPTRI AISBL

#	Participating Organization Legal Name	Country	Role
1	Consorzio per Valutacioni Biologiche E Farmacologiche (CVBF)	Italy	Coordinator
2	Universita degli Studi di Bari Aldo Moro (UNIBA)	Italy	Affiliated
3	Teddy European Network of Excellence for Paediatric Research (TEDDY)	Italy	Affiliated
4	IRCCS Eugenio Medea (Medea)	Italy	Partner
5	Instytut Pomnik-Centrum Zdrowia Dziecka (IPCZD)	Poland	Partner
6	Medical University Graz (MUG)	Austria	Partner
7	Fondazione per la Ricerca Farmacologica Gianni Benzi-onlus (FGB)	Italy	Partner
8	European Paediatric Translational Research Infrastructure (EPTRI)	Belgium	Partner

WP2 – Service access and tools management of the MD platform

Main activities:

Manage the services' access to the MD platform, including the contractual issues The selection process will prioritize requests for services to develop MDs intended for paediatric cardiology for rare diseases. Within this group, we have already identified 3 service requests (case studies 1-3, WPs 3-5 respectively).

Enable the co-creation of innovation in the orphan and paediatric MDs field to maximise the value-for-patients of the research and development projects by providing access to an ecosystem that will allow:

1. the connection between relevant players (e.g. academia, scientific societies, users) with orphan device ideas to potential manufacturers (SMEs and companies);
2. the updating of existing financing resources (considering both grants and private funds);
3. the updating of relevant regulatory documents released by the competent authorities.

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WP3 - Case study 1 “Wearable multiplexed biosensor for cyanotic congenital heart diseases”

WP3 will support the development of a **wearable multiplexed biosensor** for the prevention of **lactic acidosis** in children with **unrepaired congenital heart defects** and/or the monitoring and evaluation of the physiologic response to pulmonary valve replacement in children with **repaired congenital heart defects**.

Technology Readiness Level (TRL): level 1 (Basic Principles Observed)

Main services:	**Advice by clinical experts**
	Business and plan development
	Advice on MD design and manufacturing
	Development of a clinical evaluation plan
	Ethical, social and regulatory advice

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WP4 - Case study 2 "Medical device for non-invasive cardiac assessment in patients with tetralogy of Fallot"

WP4 will focus on the development of a **videocardiograph (VCG)**, a non-invasive MD for the **assessment of the mechanical function** of the heart in real time during open-chest surgery in patients with **tetralogy of Fallot**

Technology Readiness Level (TRL): level 7 (System Prototype Demonstrated in Operational Environment)

Main services:	**Development of a clinical evaluation plan**
	Ethical, social and regulatory advice for clinical study submission
	Regulatory advice for European conformity (EC) obtainment

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WP5 - Case study 3 “Medical device for osteogenesis imperfecta”

WP5 will support the development of a magnesium-, zinc-, calcium-based **resorbable implant** to provide a **structural support** to fragile bones, already fractured or prone to fractures, in children living with **osteogenesis imperfecta**

Technology Readiness Level (TRL): level 6 (Technology Demonstrated in Relevant Environment)

Main services:	**Advice by clinical experts**
	Advice on MD design and manufacturing
	Development of a clinical evaluation plan
	Ethical, social and regulatory advice

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THANK YOU!

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