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i4KIDS RARE

European Commission presentation

Barcelona, December 3, 2024

CONTENT

1. SJD Children's Hospital
2. i4KIDS Pediatric Innovation Hub
3. Project overview
4. Work packages
5. WP2 - Implementation of the "Acceleration 4RARE" programme
6. WP3 - Implementation of two "Challenge-based 4RARE" programmes
7. WP4 - Communication & dissemination
8. Team
9. i4KIDS-EUROPE: White Paper

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1. SJD Children's Hospital

Sant Joan de Déu Barcelona Children's Hospital:

- Children's & maternity center
- University Hospital
- Private Non-profit
- Long-term agreement with Public System

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1. SJD Children's Hospital

The Hospitaller Order of St. John of God worldwide

5 continents
53 countries
400 centers
925 brothers
45,000 employees
8,000 volunteers

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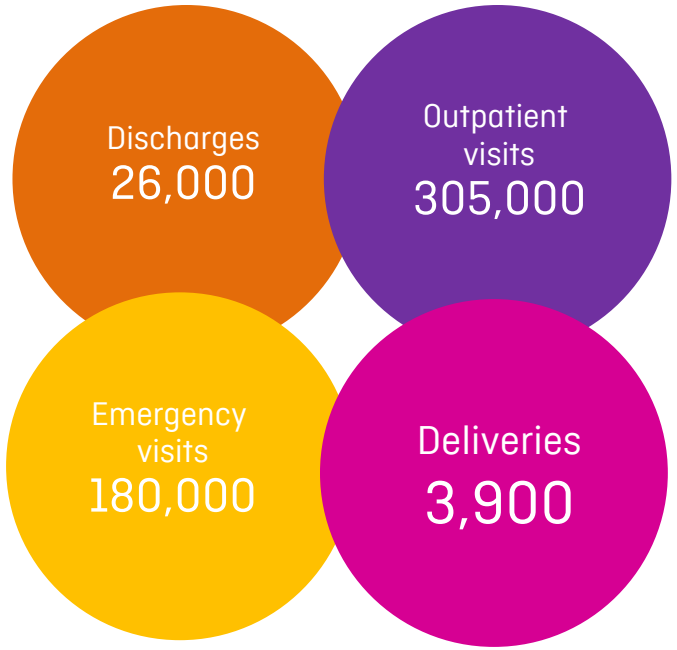
1. SJD Children's Hospital

We treat around 350,000 children per year



Professionals	2.800 professionals
Beds	340 beds
Education	16.000 students
Research	650 Articles 4.480 IF

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1. SJD Children's Hospital

ÚNICAS network

Network for attention to minority diseases

PROMOTOR CENTRE

The reality of minority diseases

9,3%
of our patients have complex minority diseases

4 years
average time to obtain a diagnosis

50%
patients still undiagnosed

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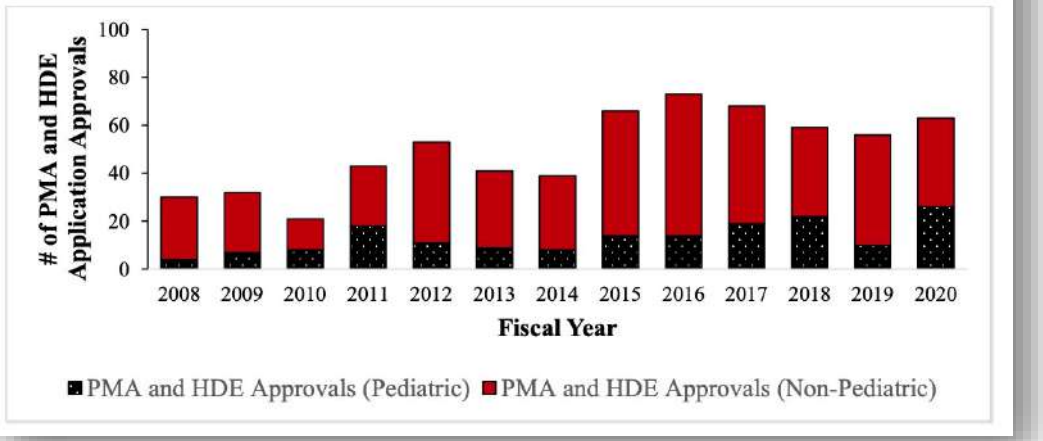
2. i4KIDS Pediatric Innovation Hub

+50% of drugs in pediatrics rare diseases are prescribed off-label
+90% in newborns
Mean of 5 years for a diagnosis

Over the past decade, **only 24%** of lifesaving medical devices approved by the FDA have an indication for pediatric use, and the majority of those are for children 12 years and older.

Source: https://www.statnews.com/2021

Figure 1A. PMA and HDE Approvals from FY 2008 to FY 2020 for Devices with Pediatric Indications and Non-Pediatric Indications.



Source: https://www.fda.gov/media/171143/download?attachment

THE LANCET

Prescribing off-label drugs for children: when will it change?

A study in *Pediatrics*, published on Sept 16, paints a familiar picture. Using data from the US National Ambulatory Medical Care Surveys 2006–15, Divya Hoon and colleagues show that in 44·5% of visits to office-based physicians who prescribed systemic drugs to children, these drugs were prescribed off label. Off-label prescription can be outside the approved age, weight, dose, formulation, route of administration, or indication. In this study, 74·6% of all off-label prescriptions were for an unapproved disorder for the specific drug and 17·6% were off-label by age. Over the study period, there was a rise in off-label orders by indication and the reasons varied by age group. There were more off-label prescriptions for gastrointestinal disorders in the youngest age groups and for psychiatric disorders in the older age groups. Studies of inpatient care have shown even higher percentages, especially for off-label drug use in the neonatal and paediatric intensive-care setting.

Progress to address this issue has been slow. The first paediatric drug development incentive legislation was introduced as part of the US Food and Drug Administration (FDA) Modernization Act more than 20 years ago. This law was followed by the Best Pharmaceutical for Children Act in 2002 and the Pediatric Research Equity Act (PREA) in 2003. Under PREA, the FDA is allowed to require paediatric studies of any drug likely to be used in a substantial number of children or when there are no good alternatives for children. Yet a study published last year showed that at the time of approval only 18 of 114 new drugs or new indications for drugs that would fall under the PREA requirement had any information on efficacy, safety, or dosing in children. Furthermore, after a median follow-up of 6·8 years, only 47 of 114 had any paediatric information.

Children are not small adults and evidence-based treatment is arguably even more important in children. Both the potential of adverse events with lifelong consequences and the danger of ineffective drugs with poor outcomes have far-reaching consequences. The current efforts are woefully inadequate. ■ *The Lancet*

For more on the Pediatrics study see https://pediatrics.aappublications.org/content/early/2019/09/12/peds.2019-0896?sso=1&sso_redirect_count=1&nfstatus=401&nftoken=00000000-0000-0000-0000-000000000008&nfstatusdescription=ERROR%3a+No+local+token

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“Children represent a quarter of our present, but the 100% of our future”


“Children represent a quarter of our present, but the 100% of our future”
“Industry spends more money to develop health technologies that address the last month of life tan technologies that can transform a lifetime”

2. i4KIDS Pediatric Innovation Hub

There is a **large disparity** between the health innovations and opportunities that are developed and brought to market for adults versus children and adolescents.

Main barriers:

- Perceived risk in innovation processes;
- Size of the market;
- Regulatory – Clinical - Biological and ethical complexities;
- Dispersion of paediatric R&D knowledge;
- Lack of investment in paediatrics.

These barriers mean that **there are a large number of unexplored opportunities and innovations in the paediatric field, with great potential for exploitation and impact.**

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Our mission is to foster and promote innovation in the paediatric and maternity field, for a better and safer health and wellbeing.

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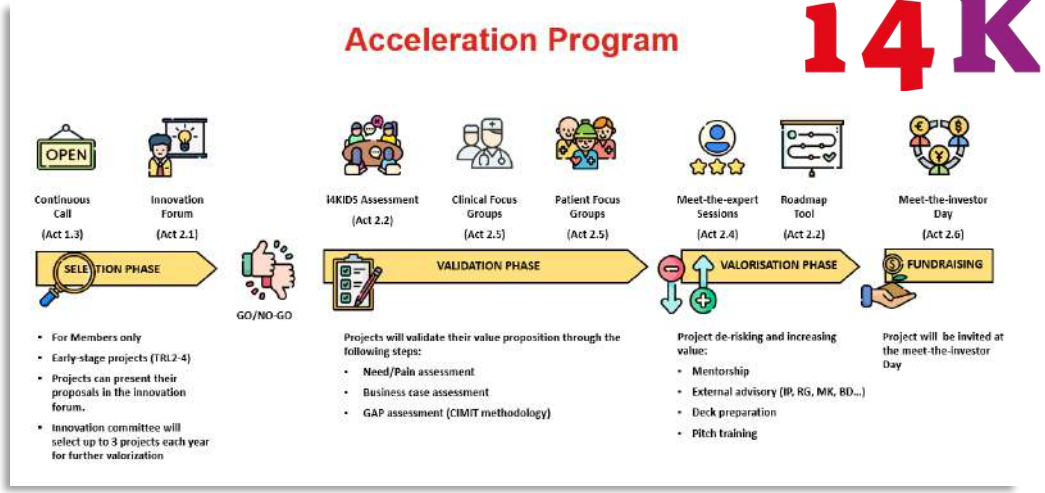
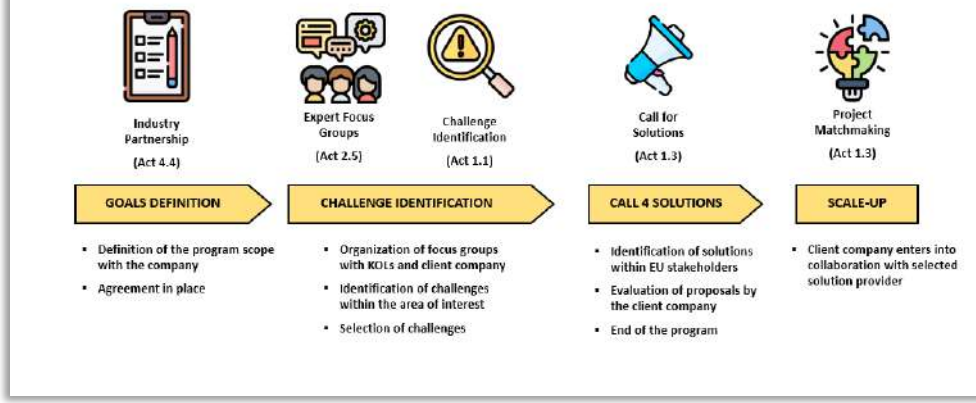
2. i4KIDS Pediatric Innovation Hub

i4KIDS

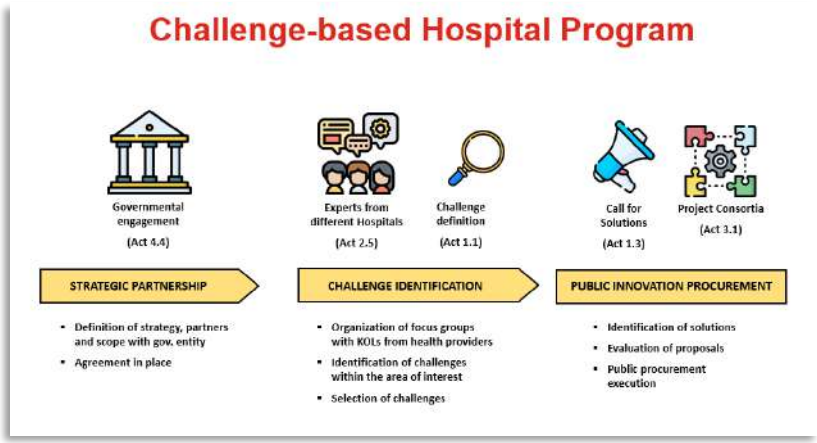
Acceleration Program



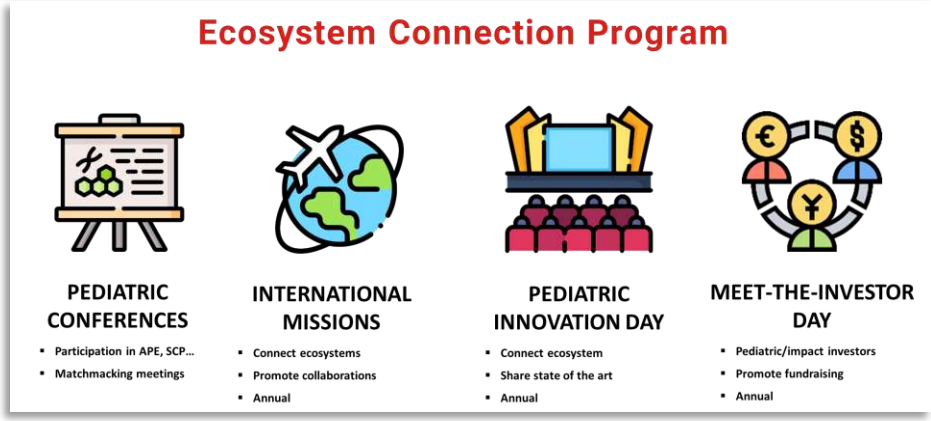
Challenge-based Industry Program



Challenge-based Hospital Program



Ecosystem Connection Program



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3. PROJECT OVERVIEW

- **TITLE**

  i4KIDS 4RARE - From challenge to adoption: acceleration of paediatric orphan medical devices

- **MAIN OBJECTIVE**

  i4KIDS 4RARE main objective is **to develop and execute a specialised accelerator programme** dedicated to fostering the creation and advancement of innovative solutions specifically tailored for paediatric patients afflicted with rare diseases in areas characterised by unmet medical needs.

- **SPECIFIC OBJECTIVES**

  SO1: Demonstrate validation and valorisation capability of **2 identified medical devices** solving unmet medical needs in the areas of paediatric congenital heart diseases and spinal muscle atrophy in young children and adolescents

  SO2: Demonstrate the potential of a **challenge-based programme** (industry and clinical) in paediatric rare diseases.

  SO3: Communicate and disseminate the activities and results of i4KIDS 4RARE **to raise awareness** and actively involve and engage stakeholders.

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4. WORK PACKAGES

Work Package	Dates	Deliverables
WP1 Project Management	1 - 24	D1.1 – Project Management Plan D1.2 – Internal final Project report D1.3 – Project Monitoring and Reporting D1.4 – Reporting on action level indicators D1.5 – Report on the advancement if the devices supported for further development and expected future milestones until Market access
WP2 Implementation of the “Acceleration 4RARE” programme	1 – 21	D2.1 – Market Access report D2.2 – Videogame prototype
WP3 Implementation of two “Challenge based 4RARE” programmes	7 – 24	D3.1 – Rules for participation in the call for solutions D3.2 – List of solutions D3.3 – List of SMEs and large companies
WP4 Communication and Dissemination	1 - 24	D4.1 – Communication and dissemination plan D4.2 – Lecture on the PID D4.3 – List of training capsules implemented D4.4 – Content of publications

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5. WP2 - Implementation of the "Acceleration 4RARE" programme

ArcoeRIS platform

Advanced Real-Time Cerebral Electronic Risk Score for Children with Congenital Heart Disease.

Development of a medical device to **improve patient monitoring during complex surgery** in patients with congenital heart disease.

Activities

- Clinical investigational plan
- Regulatory Roadmap
- Patentability Assessment
- Early Market Access study

Fantastic Quest

Virtual Reality (VR) videogame for home rehabilitation of SMA-II-III (Spinal Muscular Atrophy) patients to **improve adherence to home rehabilitation** treatment and **monitor** their exercises to follow their progress.

Activities:

- Perform a technical PoC to validate feasibility
- Identify market access strategies to support the introduction of this solution to the market
- If the validation is positive, i4KIDS 4RARE team will work on the regulatory roadmap to move to the valorisation phase.

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5. WP2 - Implementation of the "Acceleration 4RARE" programme

- **MAIN OBJECTIVES**

Demonstrate acceleration capability of **2 identified medical devices** solving unmet medical needs in the areas of paediatric congenital heart diseases and spinal muscle atrophy in young children and adolescents

ArcoeRIS platform

Specific objectives

- Identify **market access strategies** to facilitate the final adoption in the healthcare system **(T2.1)**
- Identify at least **1 potential strategy to protect assets** developed on the ArcoeRIS platform.
- To increase the Business Readiness Level (BRL).

Contributors

- Cristina Ruiz, Joan Sánchez de Toledo, Julia Meca

Deliverables

- D2.1 – Market access report

Fantastic Quest

Specific objectives

- To develop a **Proof of Concept (PoC)** for early validation **(T2.2)**
- To increase the Technology Readiness Level (TRL)

Contributors

- Julita Medina, Martí Engli, Lluis Cassou, Sofía Ferreira

Deliverables

- D2.2 – Video Game prototype

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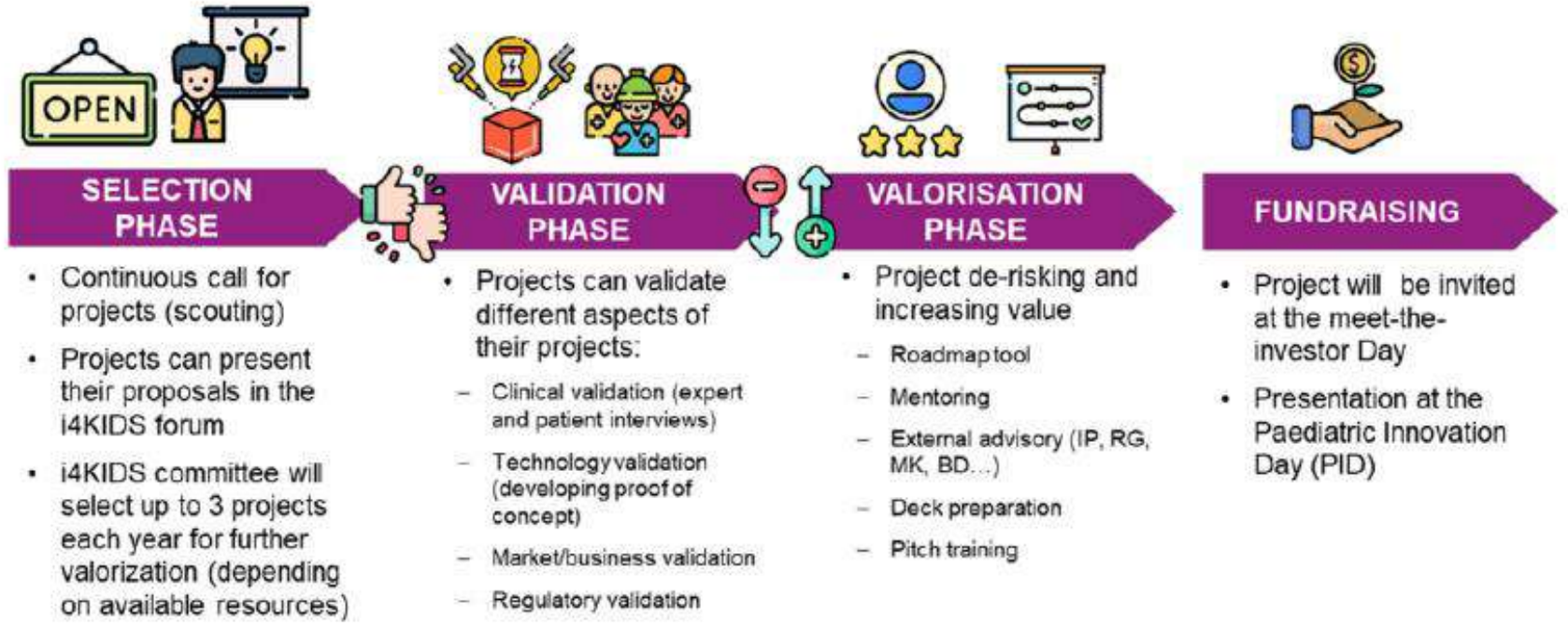
5. WP2 - Implementation of the “Acceleration 4RARE” programme

ArcoeRIS platform

Fantastic Quest

Use case #2: Cardiology: Congenital heart disease in children
Use case #3: Rehabilitation: Paediatric spinal muscular atrophy (SMA)



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6. WP3 - Implementation of two “Challenge-based 4RARE” programmes

- **MAIN OBJECTIVES**

Demonstrate the potential of an acceleration programme for paediatric rare diseases, based on the identification of needs and challenges from the expert community and patients and families:

- To **identify and prioritize** the most relevant **unmet clinical needs** in the field of rare paediatric epilepsy.
- To **identify innovators and companies** interested in developing new solutions in the field of paediatric rare epilepsy.
- To highlight the **opportunities** for innovation in rare diseases.
- Establish a group of **'innovation champions'** with experience in paediatric rare epilepsy.

- **CONTRIBUTORS**

Alexis Arzimanoglou, Sébile Tchaicha, Belén Trebino (EpiCare)
Patricia Burgos, Sofía Ferreira (HSJD)

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6. WP3 - Implementation of two “Challenge-based 4RARE” programmes

- **ACTIVITIES**

  Task 3.1 – “Challenge-based 4RARE clinical programme” in paediatric rare epilepsy
  month 7 – month 18: December 24’ – February 25’

  Task 3.2 – Establishment of the basis for the implementation of a “Challenge-based 4RARE industry programme”
  month 13 – month 24: June 25’ – August 25’

- **DELIVERABLES**

  D3.1 – Rules for participation in the Call for solutions (month 15 – PU)
  D3.2 – List of solutions (month 18 – SEN)
  D3.3 – List of SMEs and large companies (month 18 – SEN)

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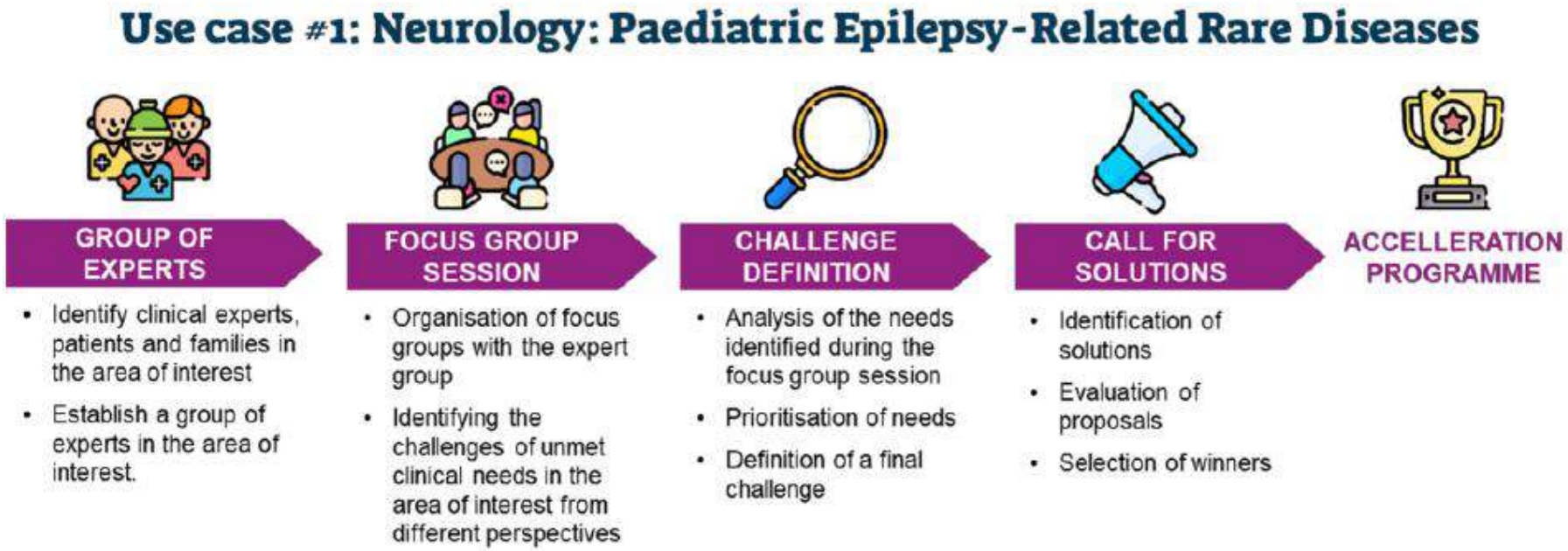
6. WP3 - Implementation of two “Challenge-based 4RARE” programmes



1. Epilepsy-focused ERN (**EpiCARE**) and Dr. Alexis Arzomanoglou will participate to the success of this activity.
2. Integration of insights from experts, patients and their families will enable us to develop a comprehensive strategy for epilepsy care.
3. i4KIDS 4RARE will demonstrate its effectiveness in a use case focused on paediatric neurology, highlighting its ability to identify clinical, innovation and regulatory requirements.

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7. WP4 – Communication & dissemination

- **MAIN OBJECTIVES**

Communicate and disseminate i4KIDS 4RARE's activities and results to actively involve and captivate stakeholders.

- **Engage with key stakeholders** including healthcare professionals, patient advocacy groups, caregivers, investors, industry, and researchers to understand their needs and interests.
- **Create content** that educates about the challenges and opportunities in paediatric rare diseases.
- Develop a comprehensive **multi-channel communication strategy** that leverages various platforms to reach a broad and diverse audience.
- Create a **detailed plan for the timely dissemination** of information.
- **Identify potential partners**, organizations, and influencers in the field of rare diseases who can help amplify the project's message and extend its reach.

- **CONTRIBUTORS**

Patricia Burgos and all partners

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7. WP4 – Communication & dissemination

- **ACTIVITIES**

  Task 4.1 – Paediatric Innovation Day (month 12 and month 21)
  Task 4.2 – Training Capsules (all project)
  Task 4.3 – Increase industry awareness of paediatric rare disease innovation and i4KIDS 4RARE's unique value (all project)

- **DELIVERABLES**

  D4.1 – Communication and Dissemination Plan (month 2 – PU)
  D4.2 – Lecture on the PID (month 21 – PU)
  D4.3 – List of training capsules (month 24 – PU)
  D4.4 – Content of publications (month 24 – PU)

1. i4KIDS 4RARE will use the existing i4KIDS channels to implement its communication strategy, by implementing an open and dynamic communication strategy. (Website, LinkedIn, Twitter, YouTube, Newsletter).
2. These platforms will be used to disseminate the activities linked to i4KIDS 4RARE (specific initiative in orphan medical devices, share content of value to the orphan community and calls of interest to the ecosystem).
3. i4KIDS 4RARE contents will also be published on i4KIDS YouTube channel, with a specific mention to this orphan medical devices project.
4. This project will have a specific space in i4KIDS website, dedicated to its activities, news, calls and projects.
5. i4KIDS 4RARE wil maintain presence in the i4KIDS newsletter and will prepare specific mailing whenever the need ari

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8. TEAM

Julita Medina (HSJD) ▶ Rehabilitation use case
Martí Engli (HSJD) ▶ Rehabilitation use case
Lluis Cassou (HSJD) ▶ Rehabilitation use case

Cristina Ruiz (HSJD) ▶ Congenital heart diseases (CHD) use case
Julia Meca (HSJD) ▶ Congenital heart diseases (CHD) use case
Joan Sanchez de Toledo (HSJD) ▶ Congenital heart diseases (CHD) use case

Alexis Arzimanoglou (EpiCare) ▶ Challenge-based in neuroepilepsy)
Sébile Tchaicha EpiCare) ▶ Challenge-based in neuroepilepsy)
Belén Trebino (EpiCare) ▶ Challenge-based in neuroepilepsy)

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Marta Grau (HSJD) ▶ Business Developer of i4KIDS 4RARE
Raquel Battle (HSJD) ▶ Promotor of i4KIDS
Sofía Ferreira (HSJD) ▶ Project Coordinator and Project Manager of i4KIDS 4RARE

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9. i4KIDS EUROPE – White Paper

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THANK YOU!

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