EPTRI®
EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

Patient and Public Involvement and Engagement in Child Health Tech Development

Begonya Nafria – Sant Joan de Déu Children's Hospital

The EPTRI Paediatric Medical Devices Platform Webinar
05/03/2024

i4KIDS

PEDIATRIC INNOVATION HUB

SJD Sant Joan de Déu Institut de Recerca

SJD Sant Joan de Déu Barcelona · Hospital

AGAUR Agència de Gestió d'Ajuts Universitaris i de Recerca

Generalitat de Catalunya
Departament de Recerca i Universitats

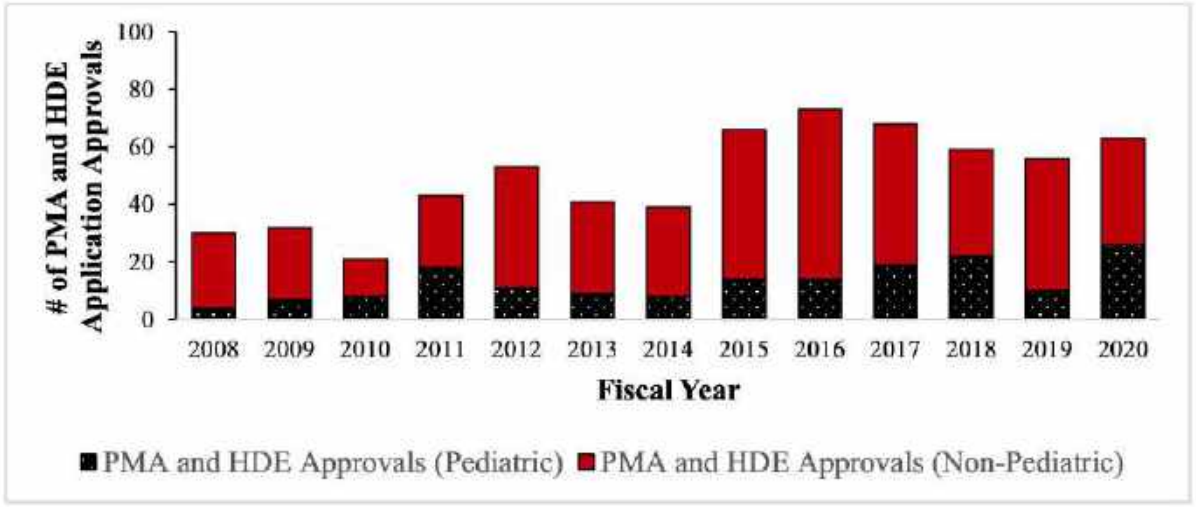
i4KIDS
PEDIATRIC INNOVATION HUB

+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*

i4KIDS
PEDIATRIC INNOVATION HUB

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

i4KIDS

PEDIATRIC INNOVATION HUB

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

The i4KIDS hub gathers 30 top research entities, including Hospitals, Universities and R+D centers

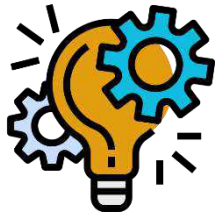
92 research groups in total

i4KIDS
PEDIATRIC INNOVATION HUB

RESULTS

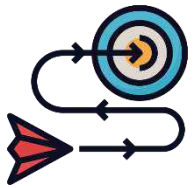
64
Members and associates

92
research groups



>125
Projects identified

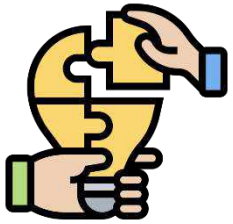
130k€
In innovation grants



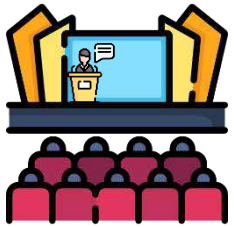
23
Projects mentored

8
Focus Groups

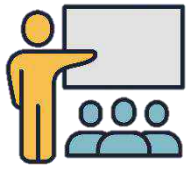
3
Industry Collaborations



2
Challenge-based programmes



3
Pediatric Innovation Day



15
Training capsules

>500
People trained

1580
Total LKN Followers

SJD
Sant Joan de Déu
Barcelona · Hospital

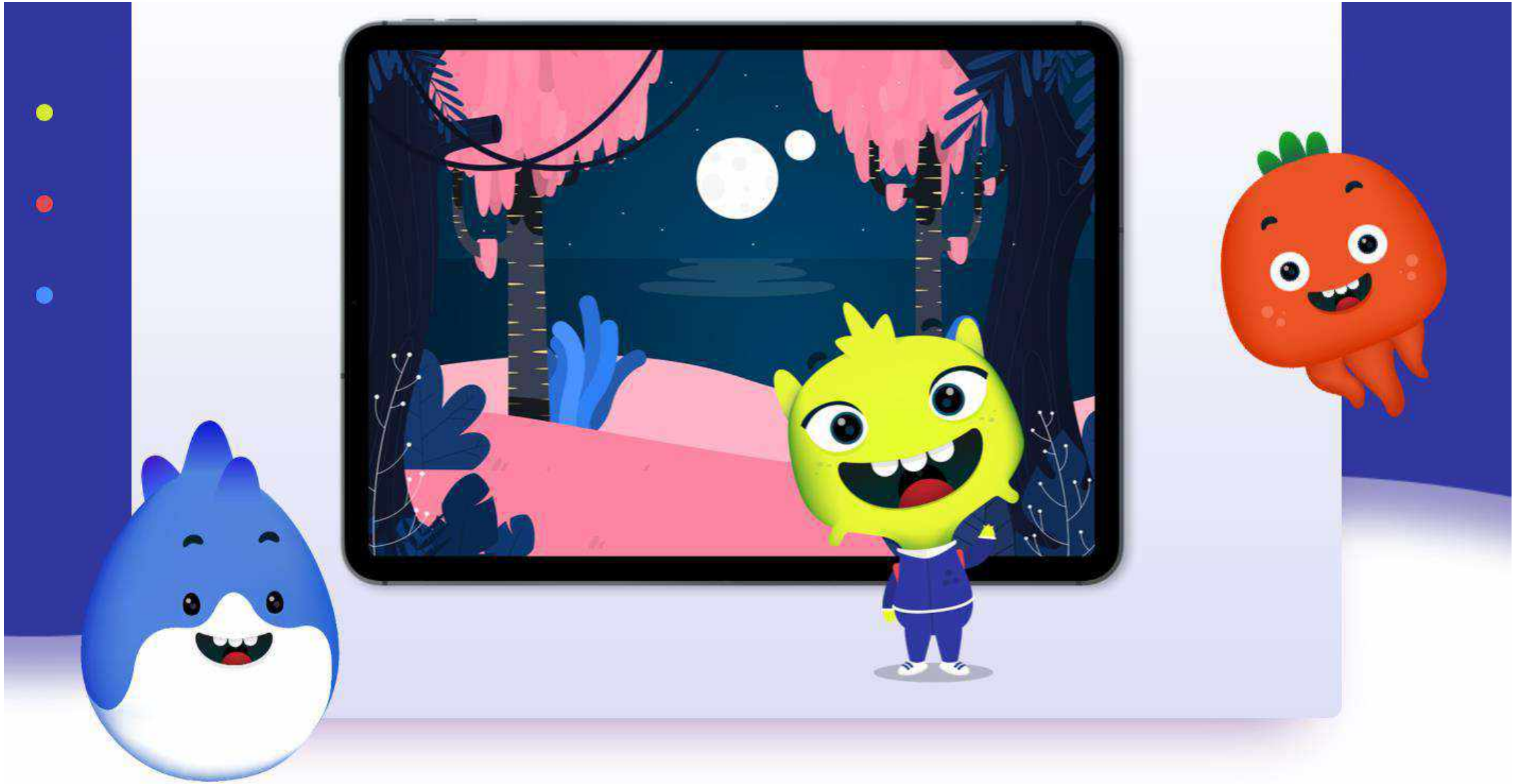
European Medical Devices Regulation

Clinical trials with medical devices are a new opportunity to involve paediatric patients: from the co-creation to the dissemination of the research outcomes.

SJD
Sant Joan de Déu
Barcelona · Hospital

New opportunities … importance of diversity

SJD
Sant Joan de Déu
Barcelona · Hospital



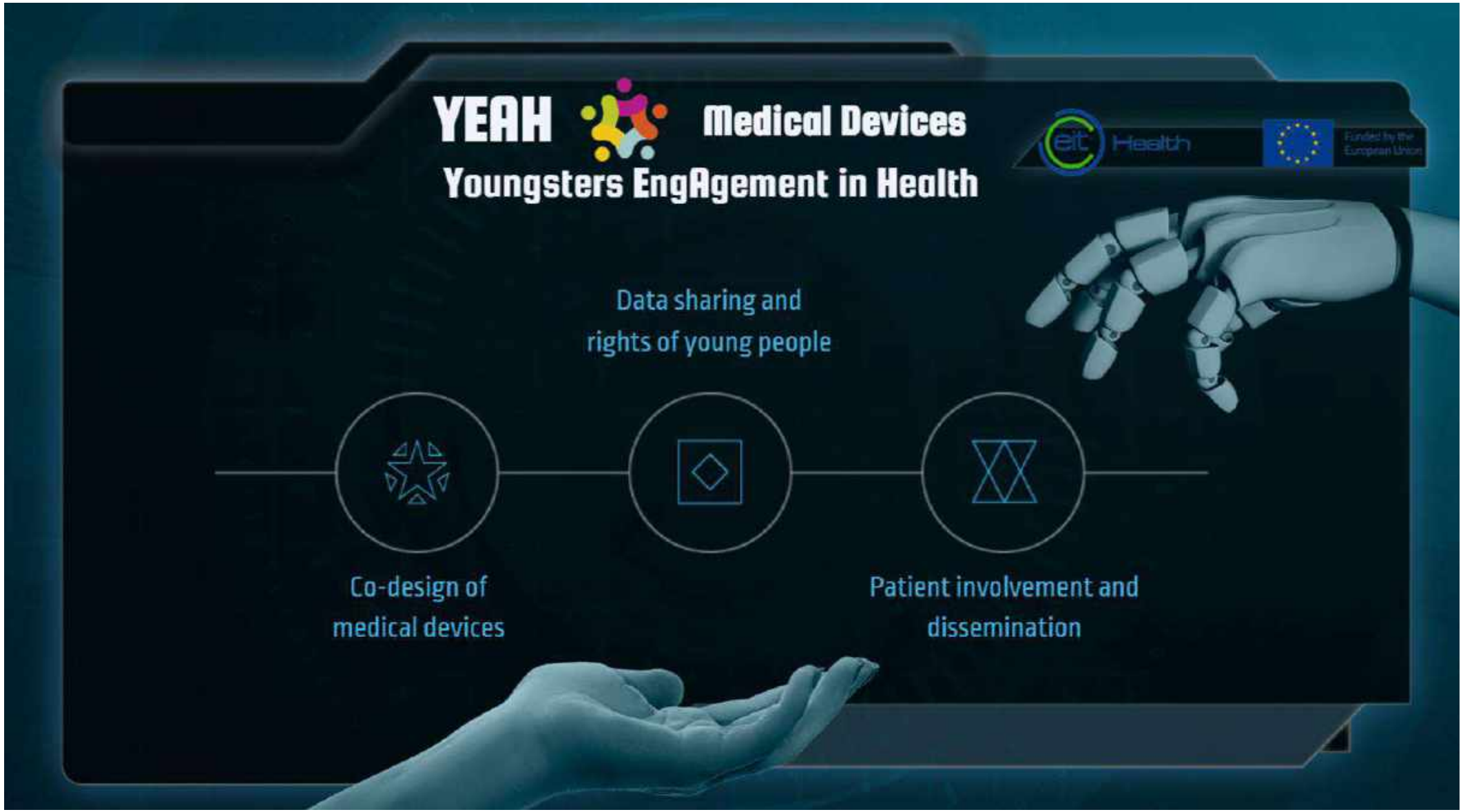
SJD
Sant Joan de Déu
Barcelona · Hospital

SJD
Sant Joan de Déu
Barcelona · Hospital

New opportunities … importance of diversity

Educating young people

SJD
Sant Joan de Déu
Barcelona · Hospital



YEAH

eit Health

Funded by the
European Union

SJD
Sant Joan de Déu
Barcelona · Hospital

Technology and Medical Devices. How can they change lives?

Charlotte was diagnosed with ME/CFS when she was 15 after two years of symptoms and tests at her local hospital.

Cose

- Her symptoms can vary hugely each day but include severe muscle and joint pain making it extremely difficult and sometimes impossible to walk.
- She also has very bad headaches, dizzy spells and sickness and suffers from extreme fatigue.
- Her mental and emotional health has been greatly affected as she cannot attend school and is largely bed-bound at home.

Bio

Before her diagnosis Charlotte was a very active teenager and had a large group of friends. She enjoyed school and was a happy and active young person. Now life is very different and her condition has impacted on the whole family.

Some of Charlotte's friends are understanding and come to visit her, but this can also be difficult.

Wants

★ Now life is very different and has impacted on the whole family.

Frustrations

Charlotte would love to get the grades to attend university in a couple of years but, due to her symptoms and the huge impact ME/ CFS is having on her life she worries this might not be possible.

← BACK

YEAH

SJD
Sant Joan de Déu
Barcelona · Hospital

LESSON PLAN MODULE 01

Co-design of medical devices

YEAH
Youngsters EngAgement in Health

Medical Devices

Medical devices form an important component of healthcare and research.

A medical device may be defined as any appliance, instrument, material, apparatus or other article, either used in a singular form in combination with other equipment/devices, including the software essential for its intended purpose.

There are many intended uses of medical devices but mainly the most relevant are:

- diagnosis, prevention, monitoring, treatment or alleviation of disease.
- diagnosis, monitoring, treatment, alleviation of or compensation for an injury or handicap.
- research, replacement or modification of the anatomy or of a physiological process.

Examples of medical devices can be pacemakers, insulin pumps, operating room monitors, defibrillator and surgical instruments, but also a syringe or glasses, and virtual reality implementations to manage specific conditions. The list of medical devices is endless.

Medical Devices
SEE TOOLKIT FOR RESOURCES

Tasks overview

Task 01: Can we live without technology?	**Task 02**: How does technology improve/ hinder our health?	**Task 03**: Technology and Medical Devices- How can they change lives?
Task 04: Are things Equal?	**Task 05**: The history of medical devices	**Task 06** Involving patients in the design of medical devices

Co-design of medical devices

Can we live without technology?

TASK 01

LEVEL	TIME	TYPE
beginner	45 - 60 min	individual/group

AIM	RESOURCES
to recognise the role of technology in our daily lives	flip-chart paper and pens, a time grid that plots the hours in a day (6 a.m. to 11 p.m.), tool kit

INTRODUCTORY TASK

(10 - 20 min)

Facilitator/teacher to watch the video: Technology Is Reinventing Humanity | Jordan Nguyen | TEDxSydney with the young people, to introduce them to the topic of the importance that technology has in our lives.

Facilitator/teacher to start the lesson by asking the questions below.

PROMPTS FOR GROUP DISCUSSION

- When do you start to use technology each day?
- Does it begin maybe with a phone alarm before jumping in the shower or using a toaster/microwave while making breakfast?
- What role does technology play as you travel to school/college and then throughout your day?

Each young person is given a time grid. Thinking individually about their routine and the activities they did the previous day or any given day, they must plot what they did hourly and whether and/or when they used technology.

This part of the task could be completed beforehand, allowing more time for the group discussion.

SJD Sant Joan de Déu Fundació de Recerca

Patient Engagement in Research Area

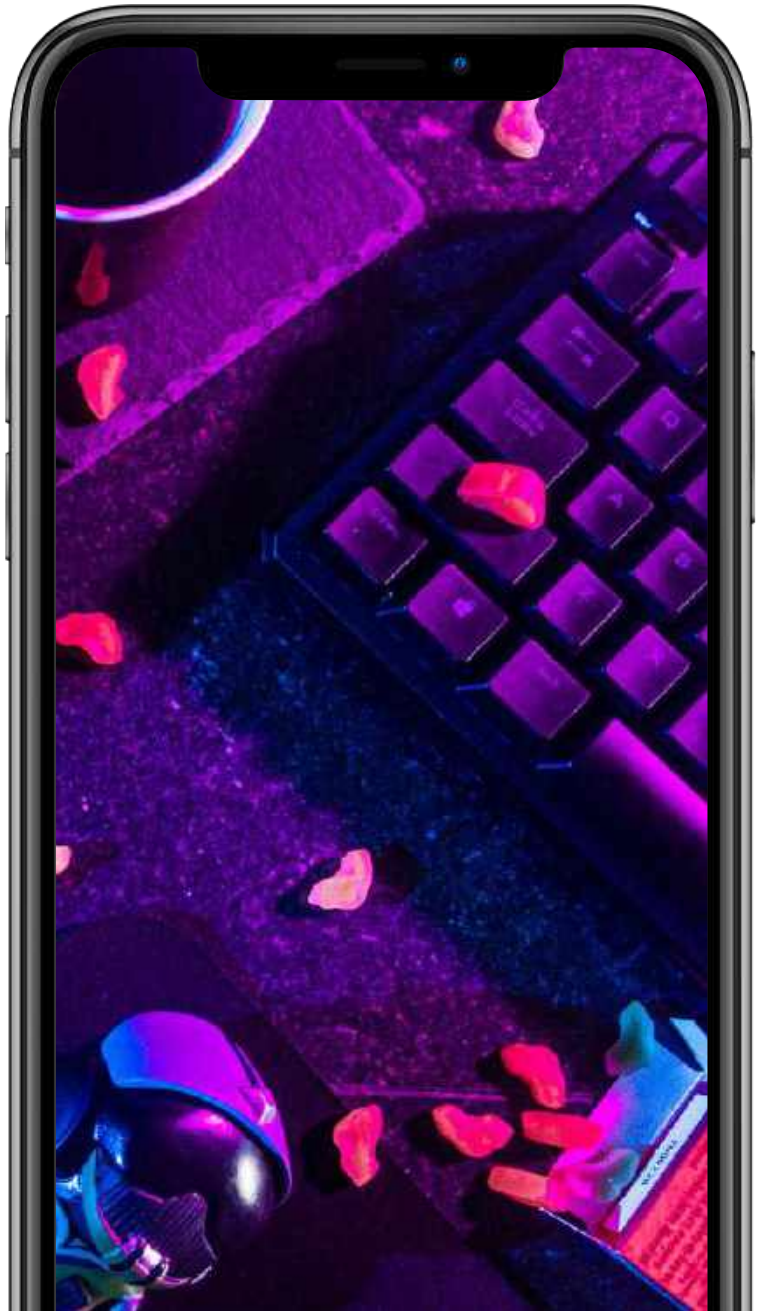
eYPAGnet

Funder teams
- Generation R
- Kids France
- ScotCRN
- Kids Barcelona

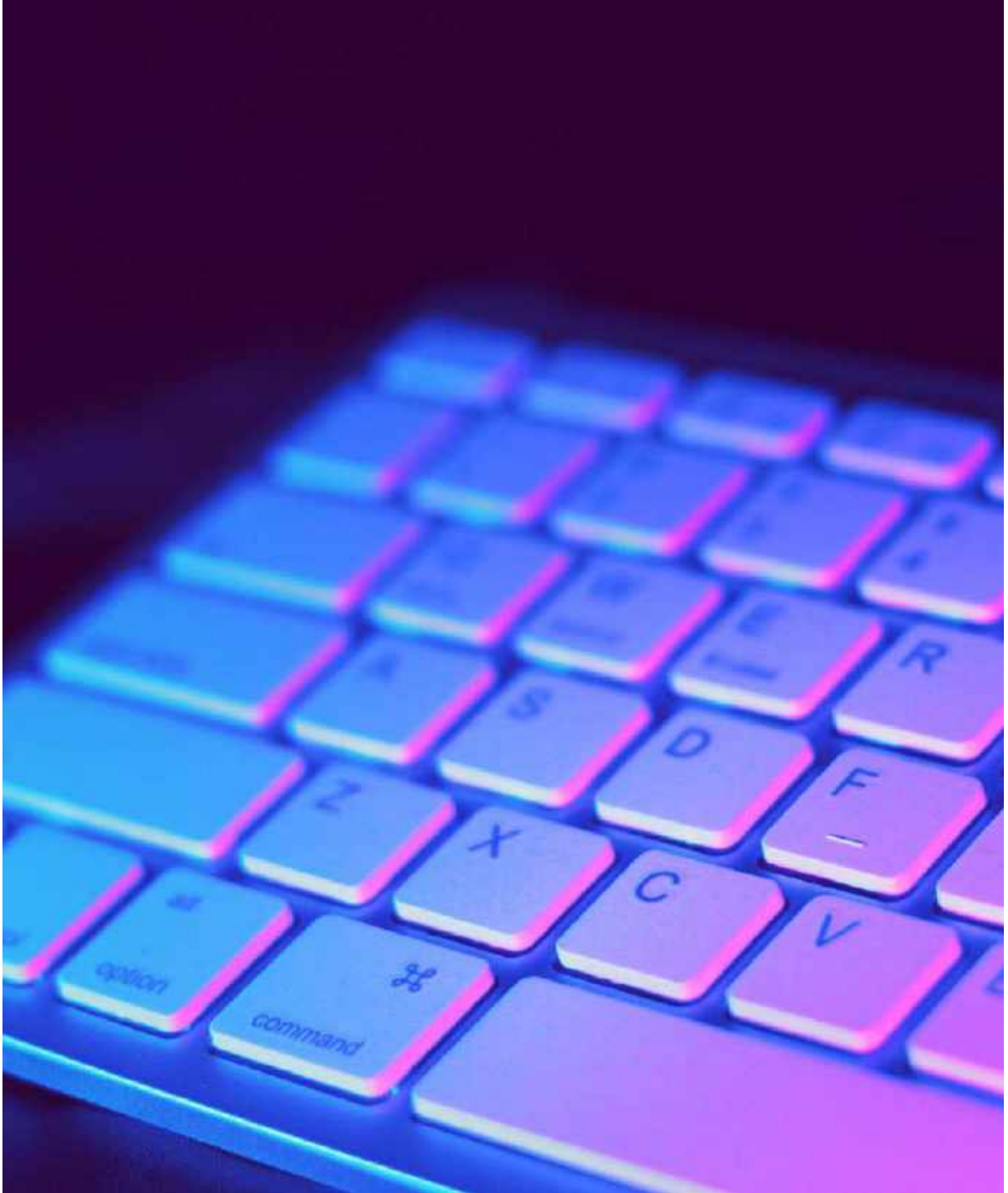
+ 30 YPAGs accross Europe

EnprEMA
European network of paediatric research at the European Medicines Agency

Lessons learned



1. Educate young people in the field of health technology is feasible.
2. Tailored materials are necessary.
3. Involve children and young people allows patient-centric design of medical devices.
4. Diversity is a key element.
5. Groups of patients and YPAGs are ready to be involved.



1. Patient involvement would be beneficial as a mandatory process.
2. Involve patients in early stages.
3. Report the feedback to CYP.
4. Change our mindset.
5. Analyse and publish the impact of involving young people.

Right to Science

Children and young people have the right to freely express their views (CRC art. 12), the right to the highest attainable standard of health and to facilities for the treatment of illness and rehabilitation of health

Thank you so much!

begonya.nafria@sjd.es
info@eypagnet.eu

ePTRI®
European Paediatric Translational Research Infrastructure