



i4KIDS EUROPE

Challenges and opportunities to boost paediatric innovation in Europe

Partners:



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Childhood and adolescence are key life stages for long-term health. However, **paediatric innovation remains underemphasized in Europe** and has opportunities and potential for greater focus.

PROBLEM

1

A LACK OF SPECIFIC SOLUTIONS FOR CHILDREN

GAP IN MEDICAL DEVICES

- **Lack of child-specific devices:** devices often need to be adapted by clinicians from adult's solutions.
- Development of **orphan medical devices significantly lags behind** orphan drugs (for rare disease).
- In Europe, there are **difficulties in accessing data on available medical devices.**

MORE EFFORTS NEEDED IN NEW MEDICINES

- **50% medicines are used off-label in children** (unapproved usage), reaching 90% for newborns.
- Clinical trials in children increased by 50% (2007-2016), but available medicines for them only rose by 5-10%.
- Paediatric medicines are approved ~5 years later than adult medicines in the EU.

2

A FRAGMENTED PAEDIATRIC ECOSYSTEM ACROSS EUROPE

SLOW PACE OF PAEDIATRIC INNOVATION IN EU:

- **A lack of incentives for paediatric solutions** and a fragmented EU ecosystem due to disparities between leading and emerging regions leaves **best practices unshared and limit paediatric innovation** and collaboration opportunities.

NEED

PAEDIATRIC INNOVATION REQUIRES KEY ACTIONS

While efforts have been made in the EU to increase paediatric medicines, a stronger push is still needed.

CALL FOR ACTION TO EU DECISION-MAKERS TO SUPPORT AND BOOST PAEDIATRIC INNOVATION

Investing in children is one of the most important things a society can do to build a better future

CHALLENGES... WITH SUCCESS CASES THAT COULD BE LEVERAGED FOR PAEDIATRICS IN EUROPE

FUNDING

#1 Challenge for leaders and moderate innovation regions

Gap in funding

- EU Public funding is too low, with only 2 calls for paediatrics in 2024
- Private funding only represents 1,6% of total Venture Capital investment in healthcare
- Need to shift mindset towards impact funding

FDA's Pediatric device consortia (PDC)

FDA provides US\$6,75 million annually to develop medical devices for children, supporting consortiums of 5 hospitals in the US during 5 years.

Lack of awareness of successful paediatric projects

- Low visibility of paediatrics success cases create less argument to convince VCs for first investment

Patient innovation platform

A collaborative platform that connects users to develop innovative, patient-centered solutions while showcasing groundbreaking projects.

REGULATION

#2 Challenge for all innovation regions

No small adults

Most paediatric solutions adapt adult designs, overlooking physiological differences and paediatric-specific diseases. Some regulations exist to extend products to paediatrics, but waivers allow industries to partially deviate from these requirements (i.e. in oncology)

New orphan devices in paediatrics

The EMA has launched a pilot initiative to support the development of medical devices by providing free, early guidance to manufacturers, ensuring alignment with patient

Incentives are lacking

Diverse age groups within the paediatric population required tailored solutions, resulting in smaller sample sizes and greater complexity in development and approval.

FDA's breakthrough therapy designation

A fast-track pathway approval for drugs recently developed that demonstrate substantial improvement over existing therapies.

CHALLENGES... WITH SUCCESS CASES THAT COULD BE LEVERAGED FOR PAEDIATRICS IN EUROPE

COMMERCIALISATION, REIMBURSEMENT AND ADOPTION

#3 Challenge for leaders and strong innovation regions

Data gaps harms commercial interests

- Lack of relevant data supporting the real unmet needs due to the off-label use in children.

Stanford Biodesign Program Impact 1

Methodology to accelerate the development of high-impact paediatric technologies, enhancing clinical collaboration and shedding light on real unmet needs in paediatrics.

Reimbursement can be lower than adult solutions

- High development costs of paediatric solutions are not reflected in the reimbursement, which disincentivise companies to invest.

US Rare Pediatric Disease Priority Review Voucher

Incentivise pharmaceutical companies to invest in R&D for rare paediatric diseases, by offering a voucher from the FDA for priority review of another drug or a financial incentive.

HUMAN CAPITAL

#4 Challenge for emerging regions

Hospital structure in emerging regions lacks innovation departments

- There are limited positions dedicated to innovation activities per se. Overload of staff limits innovation potential.

i4KIDS-EUROPE hospitals

Network to share best practices and have resources from public grants to dedicate to start the innovation activities within hospitals from emerging regions and promote annual meetings between regions

Lack of innovation mindset for healthcare providers

- Healthcare professionals are usually not trained to think out of the box, which creates a need to bridge the gap between innovation and care.

i4KIDS-EUROPE Training Capsules

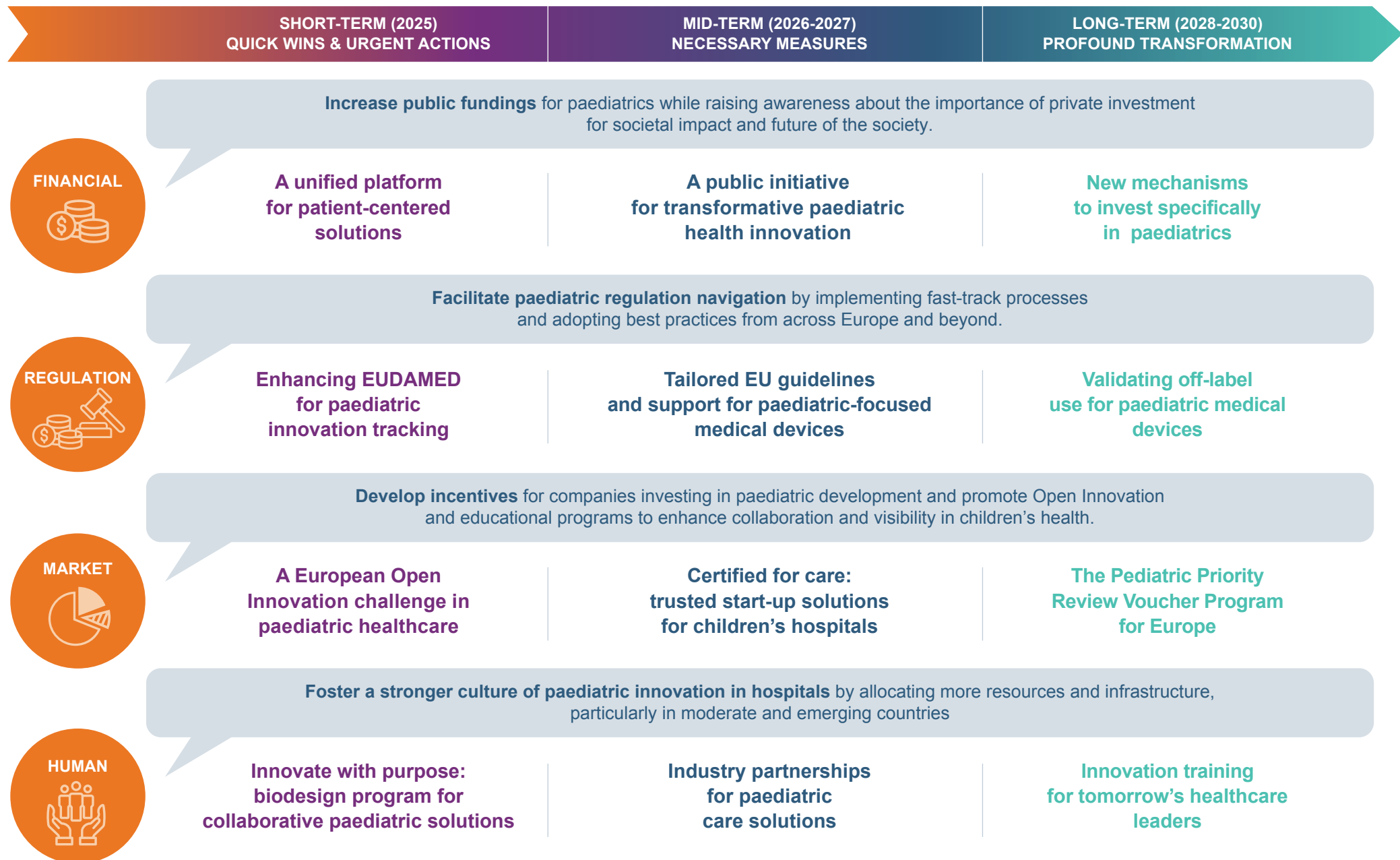
Online training focused on paediatric innovation challenges.

EIT Health Education Programmes

Online programmes to foster an innovation mindset.

... LEADING TO OPPORTUNITIES TO BOOST EU PAEDIATRIC INNOVATION

To capitalize on current momentum and advance paediatric innovation in Europe, unique measures are required to achieve a European boost in paediatric innovation



WHY INVESTING IN PAEDIATRICS IS NECESSARY TO BUILD A HEALTHIER SOCIETY TOMORROW?

PERCEPTION

REALITY OF PAEDIATRIC IMPACT

1 The market is too small

Growing Market Demand

Children represent over 25% of the global population. With advancements in healthcare and increasing awareness, there is a rising demand for specialized paediatric treatments and medical devices tailored to children.

2 Children are not sick

Unmet Needs

Many diseases and conditions in children still lack effective treatments (over 6,000 rare diseases, 15 million preterm birth, 7,4 million autism spectrum disorder, etc.) representing a burden for healthcare systems as children grow into adults with unresolved health problems. The market presents significant opportunities for breakthroughs and market leadership.

3 The regulatory path is too long and risky

Regulatory Incentives

Paediatric innovation has received increased attention in Europe recently, with new regulations that have shortened approval time and provided some incentives, such as the extended market exclusivity, encouraging innovation.

4 Paediatric projects will not translate to adult solutions

Long-Term Impact

It is often easier to adapt development and regulatory framework from kids to adult application, than the other way around.

Successful paediatric products can lead to lifelong brand loyalty and establish early market dominance.

5 There is no good project or success case in paediatrics

Involvement of users and stronger ecosystem

188 start-ups in Europe are focusing on paediatrics. On top of that, final users and institutions are particularly involved, helping with the development and facilitating its potential adoption.

Because children are 100% of our future