ECHO
European Children's Hospitals Organisation
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IMI Stakeholder Forum

Innovation to promote child health

The European Children's Hospitals Organisation (ECHO) welcomed the opportunity to participate in the IMI Stakeholder Forum, 'Broader horizons: growing Europe's health partnership.' Most of our members are reference centres for both paediatric cancer research and care, therefore the focus of this forum is especially important to the healthcare professionals and patients we represent. We also look forward to contributing to the development and implementation of the new European Partnership for Health Innovation (IHI).

Children's hospitals help drive innovation

ECHO is a new organisation made up of leading tertiary care children's hospitals across Europe aimed at leveraging the power of children's hospitals to promote and protect child health.

- Children's hospitals bring together many of the actors targeted by IHI including researchers, healthcare professionals, and patients.
- They are also key to the implementation and uptake of new health technologies and services, making them an important stakeholder across the research and innovation cycle.
- Due to their involvement with targeted stakeholders and their role in research and innovation, we recommend that children's hospitals are represented on the IHI Innovation Panel.

Opportunities to address childhood cancer in IHI

The new IHI partnerhsip with its broader and more hollistic scope has the potential to greatly benefit children and young people. Building on the Stakeholder Forum case study of childhood cancer, we make specific recommendations as to how IHI can support Europe's Beating Cancer Plan and the Cancer Mission, highlighting where additional partnerships are needed.

Specific IHI Objectives and corresponding childhood cancer actions

1. *Integrate fragmented health R&I efforts across sectors and technologies, academia, and industry/public and private stakeholders, focusing on unmet public health needs*

   Breaking down barriers to accessing clinical trials: Accessing clinical trials is still a challenge for many paediatric cancer patients in the EU, especially if the patient does not live in the country or region where the trial is being conducted. Efforts such as the IMI2-funded Conect4Children and the Innovative Therapies for Children with Cancer in Europe network aim to

address this issue, but more work is needed. **IHI can help address this fragmented access through the further integration of research, clinical care, and the development cross-border healthcare delivery models.**

2. *Deliver tools, data, platforms, technologies and processes for improved prediction, prevention, interception, treatment and management of disease, meeting the needs of end users*

   Curing better: We need to rethink how we define "cure" when we talk about childhood cancer. Although 80-85% of children survive cancer, by the age of 50, child and adolescent cancer survivors have an average of 17 chronic health conditions. **Addressing the long-term toxicity of associated with treating childhood cancer will require the application of new technologies and cross-sector partnerships proposed in IHI.** This can be accomplished through Research and Innovation Actions supporting, for instance, tumor molecular characterization studies. These studies will more acurately identify risk groups categories, allowing physicians to tailor the intensity of treatment to the individual patients and reduce unnececary exposure. Importantly, germline studies including pharmacogenomics are also needed to increase our understanding of genetic susceptibilities to mid- and long-term toxicities of current therapies. Clinical trials groups across Europe need to be coordinated and asking similar questions so results can be easily compared and pooled which is, again, an action that could be supported with IHI. Studies identifying novel therapeutics with reduced toxicity are also needed.

   Improving coordination to improve outcomes: Modern treatment of paediatric cancer patients requires the establishment of proper sampling, sophisticated fresh tumour analyses, characterization of tumour tissue by RNA and DNA sequencing, and tumour boards for interpretation of the results. A specific drug arsenal should be available as soon as the molecular profile of the cancer becomes clear. This coordinated approach is important for all childhood cancers, but especially for those children with cancers that are rare, resistant to standard treatments, or difficult to characterize. Achieving this type coordination and access will only be possible with the the cross-sector partnerships of supported by IHI.

3. *Exploit the full potential of digitalisation and data exchange in health care*

   Leveraging big data for little patients: Addressing the challenges of childhood cancer will require the full exploitation of available data. With small numbers of patients scattered across Europe, the capacity to pool data into shared data lakes with subsequent application of artificial intelligence will be pivotal for improving patient outcomes and addressing this unmet public health need. Children's hospitals, where much of these data are generated

and stored, will be a critical partner in this endeavour but this will also depend on the participation of IHI partners such as medical technology companies.

4. *Deliver new and improved methodologies and models for comprehensive assessment of the added value of innovative and tegrated health care solutions (e.g. through tools that support regulatory assessment)*

   Examining barriers to access: Currently not all children have access to immunotherapies, such as Chimeric Antigen Receptor (CAR) T-cells which are classified as advanced therapy medicinal products (ATMPs) and as such are regulated as a drug. This is a pressing issue as there are more CARs/ATMPs in the immediate pipeline (as the rapidly emerging CAR-based simmunotherapy approach). These therapies now offer real hope where there was none to a group of children with refractory / relapse disease. They also come at significant cost and decision making around their clinical use should not be based on traditional health economics. IHI can increasese access to these therapies by supporting an evaluation of how ATMPs are regulated and how their value is assessed, ensuring that their added value to patients and society is fully captured.

Coordination and governance to maximise impact

Addressing limited access through partnerships: Close ties with other European Partnerships and pan-European networks are needed to ensure that patients are able to receive the full benefits of outcomes from IHI supported projects. In the case of childhood cancer, the Partnership on Health and Care Systems Transformation, Rare Diseases, the PaedCan ERN, and end user networks like ECHO will be especially important. Corresponding innovation in the design and financing of health systems will be critical to ensuring children have access to the specialists and therapies required to help them achieve the best possible outcomes.

Coordination between IHI sponsored initiatives and other partnerships and other networks could address the challenge of limited geographic availability of appropriately staffed treatment facilities that limits access to the most advanced cancer therapies. As an example, children in Ireland must go to the UK to receive CAR-T therapy as funding is not available to employ nursing and laboratory staff to carry out such procedures. These partnership can also help address access issues posed by limited and inconsistent coverage by national health systems and insurance schemes.

Paediatric voice on the Innovation Panel: One governance body of IHI will be the Innovation Panel. The panel will help "Identify and/or review potential areas and topics" suitable for IHI. Children's hospitals already have established networks of patients advising them on research and innovation prirorities and are in a key position to represent the needs of the patients, researchers and

healthcare professionals that we work with. Therefore, we recommend that children's hospitals be represented as a stakeholder on the Innovation Panel.

Children's hospitals as innovation hubs

The time is now to prioritize child health. This means financing innovation to address the unmet health needs of children and young people, especially those with complex chronic and rare diseases like childhood cancer. Children's hospitals from Barcelona to Helsinki are already creating innovation ecosystems to address many of the same challenges identified by IHI. To obtain the full potential of these efforts, partnerships like IHI will be critical for linking efforts across Europe. As the voice of children's hospitals in Europe, ECHO looks forward working with this new partnership to help improve child health in Europe.

About ECHO

The European Children's Hospitals Organisation (ECHO) advocates for children's health and their access to the best quality care through the collaborative work of children's hospitals. Find out more at https://www.echohospitals.org or write us at ECHOinfo@sjdhospitalbarcelona.org

ECHO members

Barcelona: Sant Joan de Déu Barcelona Children's Hospital – **Copenhagen:** Rigshospitalet – **Dublin:** Children's Hospital Group – **Florence:** Meyer Children's Hospital – **Helsinki:** HUS New Children's Hospital – **London:** Great Ormond Street Hospital for Children – **Munich:** Dr. von Hauner Children's Hospital – **Oslo:** Oslo University Hospital – **Paris:** Necker-Enfants Universitary Malades Hospital – **Riga:** Children's Clinical University Hospital – **Rotterdam:** Erasmus – MC Sophia Children's Hospital – **Petach-Tikva:** Schneider Children's Hospital of Israel – **Warsaw:** Children's Memorial Health Institute