

Children's hospitals across Europe call for a strategic and targeted focus on childhood cancer

The European Children's Hospitals Organisation (ECHO) welcomes and supports the Europe's Beating Cancer Plan. ECHO members represent leading tertiary care children's hospitals from across Europe and are on the front lines of caring for children and young people with cancer. Given our membership, we offer a unique perspective on the needs of children, their families, and the professionals working to beat childhood cancer.

Paediatric Cancer (or developmental cancer) is a rare condition, but the leading cause of death by disease for children over one-year-old in Europe¹. To fully achieve success, the Plan must address the **needs of paediatric patients and their families in a strategic and targeted way**. In particular, the pillars of treatment and follow up care should have a specific paediatric agenda underpinned by data and scientific evidence. Targeting paediatrics will not only benefit children and their families now, but will have a **lasting impact on the efficiency of healthcare systems and improve the health of individuals and communities of future generations**.

Childhood cancer is distinct from adult cancer

Paediatric cancer is its own entity, biologically distinct from adult cancer.

The vast majority of childhood cancers do not have a known cause and are not linked to environmental exposures or habits like smoking that cause cancer in adults. So although prevention and early detection strategies have the potential to dramatically affect adult cancers, these strategies will have minimal impact on children and young people. Addressing childhood cancer will require a **cross-sectional strategy that goes from the bench to the bedside and beyond** encompassing basic research, the development of new therapies and care delivery models, and changing the way we support and care for survivors. Europe's Beating Cancer Plan offers a once in a generation opportunity to address childhood cancer. **Only by focusing on the distinct needs of the paediatric population will we succeed in beating childhood cancer.**

Treatments for childhood cancer are lacking and inequity in outcomes exists throughout Europe

- The **basic approach to treating childhood cancer has not changed** since the 1970's. Although these techniques have yielded dramatic increases in

survival for some cancers, like leukaemia, other cancers, like solid tumors, remain difficult to treat. Furthermore, overall survival rates have plateaued in the last 20 years indicating an urgent need for new therapies. **It's time to give all children and young people the treatment they deserve so they have the best possible start in life.**

- Existing treatments for childhood cancers come at a price. Currently 80% of paediatric cancers can be eradicated, but this often requires the use of **aggressive non-specific therapies that can be extremely toxic** in both the short and long term.
- The EU Paediatric Regulation was enacted to incentivise the development of new medicines for children. Unfortunately, **paediatric oncology remains neglected with very few new therapies authorized for childhood cancer** since the implementation of the Regulation in 2007^{2,3}. This is in sharp contrast to the numbers of new therapies for adult cancer.
- Across Europe there are also **significant inequalities in access to medicines and the most advanced care**. The 5-year survival rate varies by as much as 20% between countries and within country disparities also exist⁴. These inequalities are even more pronounced when looking at survival in low- and middle-income countries.

Treatment and care recommendations

1. ECHO supports the Plan's proposed **development of new strategies to incentivise the pharmaceutical industry to do dedicated paediatric cancer research and develop new therapies for childhood cancer**. Specifically, we recommend a review of article 11b of the European Paediatric Regulation, which provides waivers for products treating conditions that do not occur in children.
2. Any new drug development strategies should **involve the full range of stakeholders** including patients and patient advocates, regulators/payers, academics, and clinicians.
3. New drug development must be **driven by scientific data** and the **mechanism of action of drugs**, not by the adult market potential.
4. ECHO also recommends that the Plan include strategies to ensure **health systems across Europe can afford to provide new therapies** once they come to market.
5. Strategies should be developed to **concentrate the delivery of highly specialised care in regional centres of excellence** to ensure all children receive the highest quality care possible.
6. Finally, **best practices that take holistic approach to health care organisation and delivery** need to be established. Resources should be dedicated to scaling these up to **eliminate current disparities in childhood cancer survival**.

Quality of life is a pressing issue for individuals and health systems now and in the future

- As a result of aggressive non-specific cancer therapies, many childhood cancer survivors have **long-term effects that last well into adulthood**. These include second cancers, heart and lung damage, osteoporosis, cognitive impairment, or fertility problems⁵.
- Nearly half of childhood cancer survivors will have a **severe or disabling chronic condition**, limiting their ability to grow into fully autonomous members of society and placing an increased burden on public health systems caring for them⁶.
- The **psychological impact** of cancer is long-lasting with many survivors **suffering post-traumatic stress disorder, bullying, and isolation**⁷.
- **Transition to adult care** is also problematic and can lead to disengagement and **worsening health outcomes**⁸⁻¹⁰. Recommendations on transition exist, but standardized implementation is not widespread and **most youth receive less than optimal support**¹¹.

Quality of life recommendations

1. Any initiatives addressing quality of life should take a **holistic view of health** that accounts for the unique **physical, psychological, and social** needs of childhood cancer survivors.
2. ECHO supports the proposed use of platforms, structures and resources to facilitate the **dissemination of best practices**. ECHO also supports the focus on **digital solutions** to harmonize care throughout Europe and further the development of person-centred care.
3. **Teens and young adults have different emotional, physical, and medical needs** from infants, toddlers and children and suffer from distinct forms of cancer. ECHO recommends that plans to address quality of life include the development of **dedicated teams capable of addressing the specific needs of teens and young adults**.
4. Addressing transition to adult care is also an essential part of quality of life for survivors of childhood cancer. We recommend that the Plan support **the development and implementation strategies on transition to adult care**. These should take an **integrated care approach**, and include the development of **multidisciplinary teams**. The creation of appropriate **reimbursement and incentive schemes** to support the adoption of best practices in transition to adult care are also needed.
5. Finally, **we call for a comprehensive, horizontal approach across different policies** included in the European Green Deal, the Sustainable Development Goals and other relevant EU activities **to tackle environmental risk factors** for cancer and chronic disease that disproportionately impact survivors of childhood cancer.

Investment in targeted paediatric targeted research is critical, but Europe lags behind in funding

- ECHO supports the Plan's objective of understanding cancer better and filling in knowledge gaps. This is especially critical for childhood cancers where **tumours do not follow the principles of adult oncology**.
- In spite of the need for a better understand of childhood cancer, worldwide specific funding for **childhood cancer research is on the decline**¹².
- Furthermore, almost two thirds of funding for childhood cancer is dedicated to preclinical studies and there **is limited investment in translation to new medicines, care models, or prevention strategies**¹².
- Funding for research on paediatric cancer is **disproportionately supported through philanthropy**, with some centres funding as much as 80% of the paediatric cancer research budget with donations.
- Similarly, nearly **80% of paediatric cancer funding comes from and is awarded to organisations in the USA**¹². This makes children, family, researchers and clinicians in Europe vulnerable to changes in US funding and health policy priorities. **Children and young people in Europe deserve a secure and dedicated source of support for research on childhood cancer**.

Knowledge, data and scientific evidence recommendations

1. ECHO recommends that the Plan include resources to **support basic research into the mechanisms and biology of paediatric cancers**. This can be achieved by supporting both **individuals and networks of researchers** and through investment in **research infrastructure**.
2. To ensure that research yields the maximum benefit to end users, ECHO recommends that **patients and families be systematically included** in the development of research questions and in the design of clinical trials.
3. Support is also needed for structural paediatric clinical research units to facilitate the running of clinical trials.
4. ECHO also supports the Plan's proposal to **leverage big data and artificial intelligence** to accelerate the development of **new therapies and precision medicine**.
5. Finally, in order to reduce health inequities and increase efficiency, investment in health services research is needed to identify the **best ways to organize and deliver care**. This includes research into **new roles for existing healthcare providers**, such as nurses and pharmacists, as well as **the development of new professionals**, like child life specialists.

Summary

Europe's Beating Cancer Plan has the potential to make dramatic gains in the health and wellbeing of children, young people, and families affected by childhood

cancer. We thank the Commission for the opportunity to comment on the Plan and look forward to continuing to participate in the public consultation process. **By addressing the needs of children and young people within the Plan, we will collectively ensure that all children in Europe have the opportunity to thrive and unfold their full potential.**

References

1. Being young in Europe today - Statistics Explained. https://ec.europa.eu/eurostat/statistics-explained/index.php/Being_young_in_Europe_today. Accessed January 28, 2020.
2. Pearson AD, Heenen D, Kearns PR, et al. 10-year report on the European Paediatric Regulation and its impact on new drugs for children's cancers. *The Lancet Oncology*. 2018;19(3):285-287. doi:10.1016/S1470-2045(18)30105-0
3. Vassal G, Zwaan CM, Ashley D, et al. New drugs for children and adolescents with cancer: the need for novel development pathways. *The Lancet Oncology*. 2013;14(3):e117-e124. doi:10.1016/S1470-2045(13)70013-5
4. Gatta G, Botta L, Rossi S, et al. Childhood cancer survival in Europe 1999–2007: results of EUROCARE-5—a population-based study. *The Lancet Oncology*. 2014;15(1):35-47. doi:10.1016/S1470-2045(13)70548-5
5. *Childhood Cancer Survivorship: Improving Care and Quality of Life*. Washington, D.C.: National Academies Press; 2003. doi:10.17226/10767
6. Phillips SM, Padgett LS, Leisenring WM, et al. Survivors of Childhood Cancer in the United States: Prevalence and Burden of Morbidity. *Cancer Epidemiology Biomarkers & Prevention*. 2015;24(4):653-663. doi:10.1158/1055-9965.EPI-14-1418
7. The impact of childhood cancer on emotional health and wellbeing. <https://www.cclg.org.uk/researchfunds/ehwb/impact>. Accessed March 3, 2020.
8. Watson AR. Problems and pitfalls of transition from paediatric to adult renal care. *Pediatr Nephrol*. 2005;20(2):113-117. doi:10.1007/s00467-004-1763-y
9. Rachas A, Tuppin P, Meyer L, et al. Excess mortality and hospitalizations in transitional-age youths with a long-term disease: A national population-based cohort study. *PLoS One*. 2018;13(3):e0193729. doi:10.1371/journal.pone.0193729
10. Samuel SM, Nettel-Aguirre A, Soo A, Hemmelgarn B, Tonelli M, Foster B. Avoidable hospitalizations in youth with kidney failure after transfer to or with only adult care. *Pediatrics*. 2014;133(4):e993-1000. doi:10.1542/peds.2013-2345
11. Mouw MS, Wertman EA, Barrington C, Earp JAL. Care Transitions in Childhood Cancer Survivorship: Providers' Perspectives. *Journal of Adolescent and Young Adult Oncology*. 2017;6(1):111-119. doi:10.1089/jayao.2016.0035
12. Loucaides EM, Fitchett EJA, Sullivan R, Atun R. Global public and philanthropic investment in childhood cancer research: systematic analysis of research funding, 2008–16. *The Lancet Oncology*. 2019;20(12):e672-e684. doi:10.1016/S1470-2045(19)30662-X

About ECHO

ECHO advocates for children's health and their access to the best quality care through the collaborative work of children's hospitals.

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