



A EUROPE FOR CHILDREN & ADOLESCENTS WITH CANCER: DON'T LEAVE THEM BEHIND

POSITION PAPER OF THE EUROPEAN PAEDIATRIC CANCER COMMUNITY

INTRODUCTION AND BACKGROUND

PAEDIATRIC AND ADOLESCENT CANCERS:

THE MAJOR ISSUES ARE UNDERSERVED: FOCUSED ACTIONS TO END INEQUITIES ARE NEEDED

- **Cancer remains the first cause of death by disease in Europe in children older than 1 year - more than **35,000** cases are diagnosed and over than **6,000** young patients die annually(1)**
- While improvements in cure rates have been achieved for some childhood cancers with the stringent and concerted use of old older chemotherapeutics (about 80% off-label) over decades alongside with local control modalities (surgery, radiotherapy) **little progress has been made in the cure rate of several other paediatric malignancies and there is a lack of innovation for better cure overall(2)**
- **Age-related specificities call for tailored approaches to paediatric cancer over and above the cross-cutting issues shared with the adult cancer sector(3)**
- There are nearly **half a million childhood cancer survivors in Europe**, with **the majority experiencing adverse long-term effects with increasing incidence over lifetime**, hindering their daily life and participation in activities in relation to that of their peers, that are largely **related to treatment approaches used in the last decades(4)**
- There are substantial **inequalities in access to the best available care and expertise across Europe** causing up to **20% differences in survival rates** among European countries(5)

EU CAN DRIVE TRANSFORMATIONAL CHANGE

The **rarity of individual paediatric cancer types** and their **leading collective health burden across Europe** make coordinated EU level approaches crucial. A comprehensive EU effort dedicated to childhood cancer is needed to boost life-saving gains.

The **SIOPE Europe Strategic Plan – A European Cancer Plan for Children and Adolescents(2)** was developed with EU project support by representatives of the entire paediatric cancer sector. It can serve as a blueprint for the elaboration of EU strategies and implementation dedicated to childhood cancers.

References

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- (2) **Vassal G, Schrappe M, Pritchard-Jones K, et al.** The SIOPE strategic plan: A European cancer plan for children and adolescents. J Cancer Policy 2016;8:17–32. <https://siope.eu/european-strategic-plan/>
- (3) **JARC – Joint Action on Rare Cancers.** Rare Cancer Agenda 2030: Ten Recommendations from the EU Joint Action on Rare Cancers. Casali PG, Trama A, editors. Lecce: Youcanprint; 2019. 131 p. https://jointactionrarecancers.eu/attachments/article/265/Rare_Cancer_Agenda_2030.pdf
- (4) **Hjorth L, Haupt R, Skinner R.** Survivorship after childhood cancer: PanCare: A European Network to promote optimal long-term care. Eur J Cancer 2015; 51(10): 1203-1211. [https://www.ejancer.com/article/S0959-8049\(15\)00306-8/fulltext](https://www.ejancer.com/article/S0959-8049(15)00306-8/fulltext)
- (5) **Gatta G, Botta L, Rossi S, Aareleid T, Bielska-Lasota M, Clavel J, et al.** Childhood cancer survival in Europe 1999-2007: results of EURO CARE-5--a population-based study. Lancet Oncol. 2014;15(1):35–47. <http://www.ncbi.nlm.nih.gov/pubmed/24314616>

KEY AREAS FOR ACTION

- 1.** Improve **access to anti-cancer medicines and innovation for children and adolescents**
- 2.** Enable **research progress in an area of ‘market failure’**
- 3.** Exploit the **Artificial Intelligence** potential for paediatric cancers by overcoming the current limitations related to data silos of current research (infra) structures to gain new insights in paediatric cancer genesis, development and cure
- 4.** Make European Member States committed to counter **inequalities in childhood cancer survival**
- 5.** Ensure **best possible follow-up care, research, and empowerment of childhood cancer survivors** as a growing EU population
- 6.** **Support to families** with severely ill children with cancer by facilitating the care continuum throughout the cancer journey with special psychosocial and financial support inside member states and cross border.

Europe’s Beating Cancer Plan, EU4Health, Pharmaceutical Strategy for Europe, and the Cancer Mission Area are among the initiatives that hold great promise for children and adolescents with cancer.

EU-level support is instrumental to ensure that their urgent needs are not overlooked and instead become an integral part of all relevant policies and programmes.

DETAILED INFORMATION AND REFERENCES FOR EACH OF THE ABOVE ACTION POINTS FOLLOW



1. ACCESS TO ANTI-CANCER MEDICINES AND INNOVATION FOR CHILDREN AND ADOLESCENTS

The relative **lack of fast market-driven therapeutic innovation in paediatric cancers** is among the reasons for stagnating cure rates and persisting adverse long-term side effects of using older medicines in survivors. The EU Paediatric and Orphan Regulations have not proven effective for paediatric cancers(1-5). For instance, more than ten years since the implementation of the EU Paediatric Regulation, only 9¹ anti-cancer medicines have been authorised for a specific paediatric cancer indication, in contrast to over 150 for adult cancers. European surveys also point to issues in access to essential medicines(6). Access to early phase clinical trials for children in relapse or treatment failure may provide a second chance for these patients and is available in only few centres across Europe(7).

The European Paediatric and Adolescent Cancer Community calls for:

- **Faster and more efficient development of innovative medicines** for children with cancer - including in relation to the **EU Paediatric Regulation**: based on **European Parliament Resolution on Paediatric Medicines, Dec. 2016**(8) and the regulatory developments overseas such as the **Race for Children Act**(9)
- Measures to **counter shortages of essential anticancer medicines & foster availability of child-friendly formulations**
- **Appropriate pricing and reimbursement strategies** for newly approved expensive medicines gradually becoming available for the paediatric population.
- Recognition of **early clinical trial access** for children with poor prognosis as a standard of care.

References

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- (7) **JARC – Joint Action on Rare Cancers**. D9.2. Report summarizing recommendations to facilitate referral of children to trial centers offering innovative medicines. 2019. <https://jointactionrarecancers.eu/index.php/news-events/262-list-of-the-jarc-deliverables>
- (8) **European Parliament**. European Parliament Resolution of 15 December 2016 on the regulation on paediatric medicines. 2016. https://www.europarl.europa.eu/doceo/document/TA-8-2016-0511_EN.html
- (9) **115th Congress 1st Session**. A bill to amend the Federal Food, Drug, and Cosmetic Act to establish a program to increase the development of new drugs to treat pediatric cancers, and for other purposes. 2017. <https://www.govinfo.gov/content/pkg/BILLS-115hr1231ih/pdf/BILLS-115hr1231ih.pdf>

¹ Until 2018

2. RESEARCH PROGRESS IN AN AREA OF 'MARKET FAILURE'

Due to limited market-driven innovation, therapeutic progress in paediatric oncology has been largely driven by cross-border academic research, often supported by project-based EU funding programmes.

The European Paediatric and Adolescent Cancer Community calls for:

- EU to take the lead in **redressing unequal allocation to paediatric cancer research funding documented globally**(1)
- A specific focus on paediatric cancer research in the **Horizon Europe cancer mission area**(2)(3)
- **Budget allocations to novel developments in paediatric cancer research** to enable stable and sustainable international research platforms for paediatric cancer to enrich research through big data and use of Artificial Intelligence.
- Consideration to leverage the research potential of **European Reference Networks (ERNs)**

References

- (1) **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. p. e672–84. [https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045\(19\)30662-X/fulltext](https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(19)30662-X/fulltext)
- (2) **Kearns PR, Vassal G, Ladenstein R, Schrappe M, Biondi A, Blanc P, et al.** A European paediatric cancer mission: aspiration or reality? Vol. 20, *The Lancet Oncology*. 2019. p. 1200–2. [https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045\(19\)30487-5/fulltext](https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(19)30487-5/fulltext)
- (3) **Philip T, Karjalainen S, De Lorenzo F, Apostolidis K, Lombardo C, Nilbert M, et al.** What could be a cancer mission objective if we join our forces in the fight against cancer? 2019. p. 447–55. <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6952939/>

3. EXPLOIT THE ARTIFICIAL INTELLIGENCE POTENTIAL FOR PAEDIATRIC CANCERS

By overcoming the current limitations related to data silos of current research (infra) structures, AI holds great potential to gain new insights in paediatric cancer genesis, development and cure.

The European Paediatric and Adolescent Cancer Community calls for:

- **Budget allocations to novel developments in paediatric cancer research** to enrich research through big data and use of Artificial Intelligence(1)

References

- (1) **SIOP Europe.** Artificial Intelligence and Big Data: Towards the next frontier in paediatric cancer research & innovation in Europe. 2020. <https://siope.eu/news/iccd-2020-news-release-artificial-intelligence-and-big-data/>

4. ERADICATE INEQUALITIES IN CHILDHOOD CANCER SURVIVAL ACROSS EUROPE

There are substantial inequalities in access to the best available standard care and expertise across Europe causing up to **20% differences in children's survival** (1)(2).

The European Paediatric and Adolescent Cancer Community calls for:

- **Best possible care for any child with cancer no matter where in Europe they live**
- Support the sustainability of **European Cancer specific Reference Networks (ERNs)** to further develop them to their full potential(3) and foster their integration in national health systems(4)
- Full realisation of **patients' rights to cross-border care**, including clarity on rules governing reimbursement of treatment received outside of one's home country(3)

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- (3) **European Parliament.** European Parliament report on the implementation of the Cross-Border Healthcare Directive. 2018. http://www.europarl.europa.eu/doceo/document/A-8-2019-0046_EN.html
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5. MEET THE NEEDS OF CHILDHOOD CANCER SURVIVORS AS A GROWING POPULATION IN EUROPE

The rarity of individual paediatric cancer types and their associated physical, social and psychological long-term side effects magnifies the importance of the European dimension to enable a smooth transition for childhood cancer survivors into a productive and healthier adulthood(1-3).

The European Paediatric and Adolescent Cancer Community calls for:

- EU support to collaborative approaches to further define, disseminate and enable:
 - Standardised **surveillance guidelines for late-occurring side effects** of childhood cancers(4)
 - **Organised health care transition** (paediatric to adult) as a standard of care across Europe(5)
 - Survivorship **follow-up care plans, infra(structures) & interoperable IT tools** including a **Survivorship Passport** and integration of patient-reported outcomes to facilitate an appropriate life-long informed care model allowing childhood cancer survivors to live their life at its best potential(6)
 - The facilitation of prospective long-term follow-up of novel therapies against childhood cancer for these growing and developing individuals.
- Full realisation of survivors' **rights and non-discrimination** in all areas of life.

References

- (1) **Hjorth L, Haupt R, Skinner R.** Survivorship after childhood cancer: PanCare: A European Network to promote optimal long-term care. Eur J Cancer 2015; 51(10): 1203-1211. [https://www.ejancer.com/article/S0959-8049\(15\)00306-8/fulltext](https://www.ejancer.com/article/S0959-8049(15)00306-8/fulltext)
- (2) **JARC – Joint Action on Rare Cancers.** D9.5. Guidelines and recommendations on models of healthcare to assure adequate follow-up of children surviving cancer, transition to adult medicine, and the use of the Survivorship Passport. 2019. <https://jointactionrarecancers.eu/index.php/news-events/262-list-of-the-jarc-deliverables>
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6. SUPPORT PARENTS CARING FOR SEVERELY ILL CHILDREN

The right of the hospitalised child to “constant and continuous parental involvement” is defined in the European Standards of Care for Children with Cancer developed in the European Partnership Against Cancer Joint Action (EPAAC, EU Health Programme)(1). A parent's presence during the child's treatment is essential.

The European Paediatric and Adolescent Cancer Community calls for:

- Upholding the **rights of children with cancer to parental presence** and support throughout the patient journey.
- **EU resolution on ensuring secure sustained livelihood for parents** of severely ill children, such as through social support and employment security.

References

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ABOUT THE EUROPEAN PAEDIATRIC CANCER COMMUNITY

SIOP EUROPE - www.siope.eu



The European Society for Paediatric Oncology (SIOP Europe or SIOPE) is the only pan-European organisation representing all professionals working in the field of childhood cancers. With more than 1,900 members across 36 countries, SIOP Europe is leading the way to ensure the best possible care and outcomes for all children and adolescents with cancer in Europe.

Representing childhood cancer professionals, SIOP Europe is working in Memorandum-based partnership with European organisations of parents, patients and survivorship community:

CCI EUROPE - www.ccieurope.eu



CCI Europe represents childhood cancer parents' and survivors' groups as well as other childhood cancer organisations in Europe: 64 organisations in 32 European countries are members of CCI. CCI Europe works together with all relevant stakeholders for the same aim, namely to help children and adolescents with cancer to be cured, with no - or as few as possible - long term health problems/late effects.

PANCARE - www.pancare.eu



PanCare is a multidisciplinary pan-European network of professionals, survivors and their families that aims to reduce the frequency, severity and impact of late side-effects of the treatment of children and adolescents with cancer. Together with the paediatric oncology community PanCare is working to achieve equity of access to care for childhood cancer survivors across Europe, to perform collaborative research, to develop evidence based surveillance guidelines together with the International Guideline Harmonisation Group and to act as a resource of research-based information concerning all late side-effects of cancer treatment.