



BEATING CANCER IN EUROPE: LET'S NOT FORGET OUR CHILDREN AND ADOLESCENTS

Attachment to SIOPE Europe response to Europe's Beating Cancer Plan Roadmap Consultation

About SIOPE Europe (www.siope.eu)

The European Society for Paediatric Oncology (SIOPE Europe, or SIOPE) is the single, united European organisation of academia and healthcare professionals dedicated to childhood and teenage cancer and is working in close partnership with patient, parent and childhood cancer survivor groups across Europe.

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SIOPE EUROPE RESPONSE TO EU BEATING CANCER PLAN ROADMAP CONSULTATION

SIOPE welcomes Europe's Beating Cancer Plan and its links with research, global agenda, and prior EU projects.

-Neglected area: PAEDIATRIC CANCERS should not be overlooked: all individually rare, they are jointly the leading cause of death by disease in children older than 1 year in Europe, contribute to up to 60% life-long morbidity in survivors, and are marked by inequalities. As recognised in the Joint Action on Rare Cancers (JARC), PAEDIATRIC CANCERS specificities require tailored approaches beyond simple extrapolation of adult services.

-Best practices, evidence: The WHO Global Initiative on Childhood Cancer as well as the JARC Book with dedicated sections on PAEDIATRIC CANCERS and the SIOPE EUROPEAN CANCER PLAN FOR CHILDREN & ADOLESCENTS, both created with EU support, are valuable resources.

-Prevention: 10% of PAEDIATRIC CANCERS are currently known to be linked to predisposition syndromes based on somatic genetic mutations. Here, surveillance and genetic counselling for early family guidance are needed. In contrast, 40% of adult cancers are potentially preventable through external exposure modification. More dedicated research on oncogenic drivers, big data integration, and artificial intelligence (AI) are needed to find new prevention avenues for PAEDIATRIC CANCERS.

-Early detection, diagnosis, treatment: Professional education and public information may improve early detection of PAEDIATRIC CANCERS. Investment into advanced diagnostics is imperative. There are major inequalities in access to this resource but also to standard treatment, innovation, care, and research, resulting in inferior survival especially in 'widening' EU countries. The European Reference Network for PAEDIATRIC CANCERS can address this but requires enabling cross-border governance and sustainable funding. In contrast to the market-driven adult cancer sector with over 150 new medicines in the last decade, only 9 of these were seen for a truly PAEDIATRIC CANCER indication. Most PAEDIATRIC CANCER drugs are older and used off-label. Access to essential medicines and innovation across the PAEDIATRIC CANCERS spectrum (>100 molecular subtypes) is urgently needed. Dedicated international academic research platforms, cross-linked and informed by adult cancer and industry-driven research, are vital to foster therapeutic innovation in PAEDIATRIC CANCERS.

-Quality of life: A child's right to parental presence during illness requires social protection and employment security for carers. There are around 500,000 PAEDIATRIC CANCER SURVIVORS in Europe and this number may grow. The Cancer Control Joint Action identified this group as in need of specific measures to improve their quality of life and reduce the socio-economic impact of long-term morbidities. Health infrastructures supported by digital tools must enable surveillance of long-term side effects and follow-up care delivery, incl. transition to adult care settings and the psychosocial dimension. Measures to enable full integration of PAEDIATRIC CANCER SURVIVORS into society and the economy are also needed, such as the 'right to be forgotten' legislation.

-Knowledge, data, evidence: Population-based standardised registry data collection across the patient pathway is needed. Parents, patients, survivors want to share data to advance research. E-health, AI, and European data integration can drive substantial progress.

-Areas for EU level cooperation: A comprehensive EU effort is ideally positioned to steer progress in PAEDIATRIC CANCERS due to their individual rarity and high overall burden.

-Conclusion: Europe's Beating Cancer Plan must acknowledge that children and adolescents are not small adults and deserve specific recommendations as a vulnerable group with high underserved needs. A synergetic Horizon Europe Mission focused on the complex needs of PAEDIATRIC CANCERS can be transformative, generating evidence to advance diagnosis, innovate treatment, and improve care standards.

POSITION PAPER WITH REFERENCES FOLLOWS

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 **SIOP 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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- (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
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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 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). 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.

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- (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>

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)**

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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)

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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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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.

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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.

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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.