



Childhood Cancer
Translational
Challenge

What are the greatest unmet needs in childhood cancer?

Insights from our consultation with
the childhood cancer community



Despite significant advances in medical science, cancer remains a leading cause of death by disease for children and young people around the world.

Survival for some indications remains dismally low, while the treatments available for others are often associated with profound health and psychosocial implications, both in the short term and well into adulthood.

But there is hope. Research has transformed survival for some cancer types: now, around 9 in 10 children with acute lymphoblastic leukaemia survive for at least 5 years post-diagnosis. Immunotherapies are showing great promise for some indications, as a result of advances in our understanding of the immune system. We're growing our knowledge of the unique biology of childhood cancer, information that is ready to be translated into new, targeted treatments.

As a research community, we need to build on this progress to help more children and young people survive their cancer, with a good, long-term quality of life. We need to collaborate and work closely with experts – whether it's people with a lived experience, or the professionals who are involved in treatment – to ensure we focus our efforts in the most impactful areas.

To inform our own research priorities, we have sought the perspectives of young people affected by cancer, their families and carers, and healthcare professionals working in childhood oncology. We particularly wanted to understand what they feel are the key unmet needs in the field, and which areas of the patient pathway are calling out for improvement.

Ultimately, it's the lives of these people we want to improve, and we're incredibly grateful for their involvement in this process to help drive the field forward. We're pleased to share their invaluable insights with the wider research community through this report.

Addressing the unmet needs highlighted here is vital to improve both survival and quality of life for young people affected by cancer and their families. By working together, and actively involving those affected, we will do better for children with cancer.

Identifying the greatest unmet needs in childhood cancer research

Childhood cancer is one of our Translational Challenges – priority areas of unmet healthcare need, where the science is ripe for translation. In September 2024, we announced our research strategy to drive progress in this space.

We wanted our strategy to be guided by the expertise of the childhood cancer community.

We ran a consultation process to understand what the community feels are the greatest unmet needs in the field, including leading:



A workshop with people with a lived experience of childhood cancer



A survey of healthcare professionals across North America, Europe and Oceania



A landscape analysis, focused on biomarkers, novel therapeutic modalities and digital and data research

We're pleased to share the insights from this work with you.



The patient pathway for children with cancer, from the perspectives of those with lived experience

Understanding the priorities of people affected by childhood cancer is paramount if we are to drive progress.

We ran a Patient Voice workshop in early 2024 to understand areas of the patient pathway that desperately need improvement, from diagnosis to end-of-life care, or cure and beyond. Thank you to all who took part for sharing their perspective and experiences.

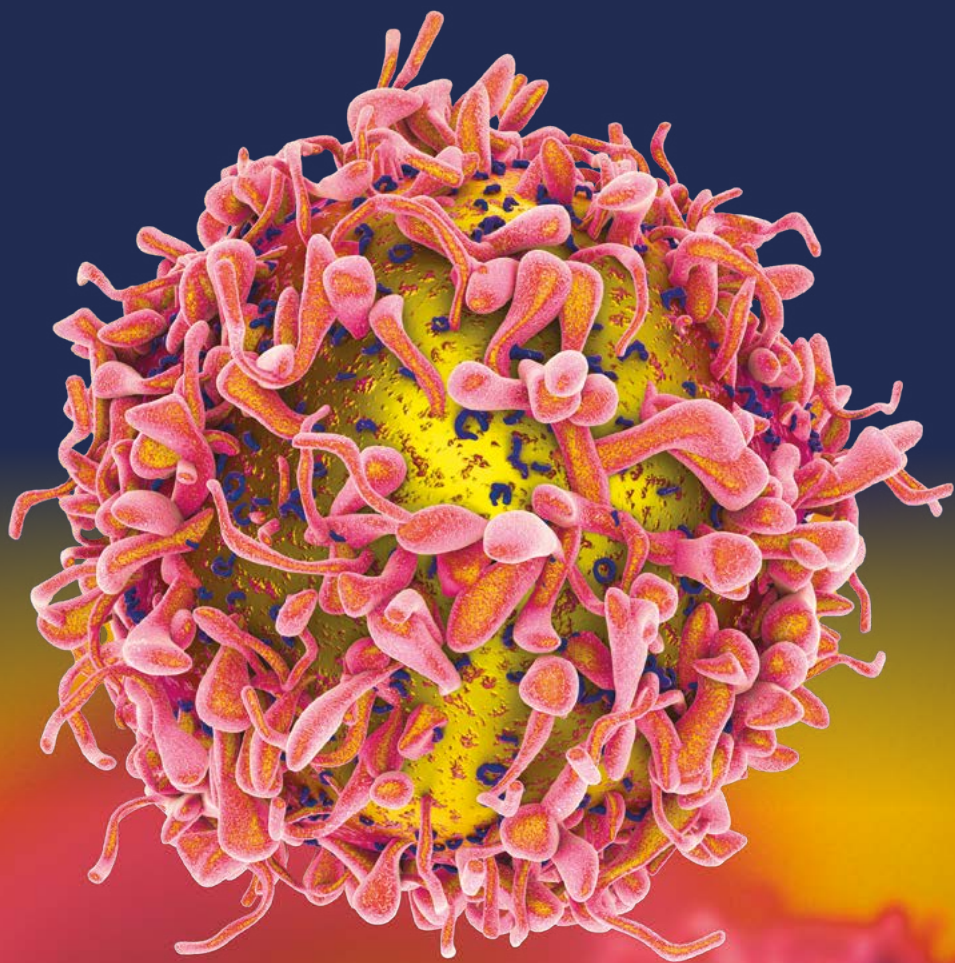


Who took part in our Patient Voice workshop?

Participants included people aged 16+ who are either living with or have survived childhood cancer, and parents or carers of a child, teenager or young person with cancer. We also invited some of our key charity partners.

Our aim was to understand:

- the diagnosis and treatment pathway from the perspective of those experiencing it
- the unmet needs and gaps in the patient pathway
- how we at LifeArc could begin to drive meaningful impact



Unmet needs across the childhood cancer patient pathway



The path to diagnosis

- greater awareness of childhood cancer symptoms
- improved communication from healthcare professionals
- minimising misdiagnoses and avoiding delays to treatment
- fewer children presenting as an emergency
- innovation to improve diagnosis – such as biomarkers, blood tests, digital pathology and improved scan interpretation

Treatment: therapy, trials, side effects, response

- more treatment options, with fewer side effects and late effects
- a roadmap for the development of new drugs
- bespoke, targeted and individualised therapies and dosing strategies
- equitable access to treatments and trials, regardless of location
- precautionary approaches for late effects



Post-treatment: monitoring, rehabilitation, relapse

- innovation: tools like blood tests, artificial intelligence and digital pathology to make monitoring and diagnosing relapse more effective
- less painful and invasive monitoring tests
- more treatment options for children who relapse
- greater understanding of relapse risk for different conditions and subtypes
- more support for parents to reduce burden of responsibility
- dedicated rehabilitation specific to children's needs

End-of-life care

- specialist hospice care so children can maintain their childhood
- flexibility to access at-home care at end-of-life

Priority areas across and beyond the patient pathway...

Psychosocial and financial

Patients and their families need greater specialist support. The group also highlighted the financial and travel burden of attending treatment, along with disruption to employment and education.

Regulatory and policy

Childhood cancer should be prioritised in government strategy, commissioning policy and NICE guidelines, to ensure consistency and equity across the healthcare system.

Data

Data should be shared across all settings to improve the patient experience, and internationally to improve research efficiency.



The greatest unmet needs in childhood cancer, according to healthcare professionals

In early 2024, we ran a survey with healthcare professionals to capture their views on the challenges of treating children with cancer. Participants were asked to:

- share information about their expertise
- select up to 3 indications they feel have the greatest unmet need
- explain their selection, including expanding on any subtypes that demand greater research focus to drive progress

With grateful thanks to all those who took part.



Who took part in our survey?

We're grateful to 3 paediatric oncology associations who shared the questionnaire with their members and networks: the European Society of Paediatric Oncology, Innovative Therapies for Children with Cancer in Europe and the Children's Oncology Group. We received responses from:

- 135 healthcare professionals across 26 countries in North America, Europe and Oceania
- clinicians, clinician scientists, researchers, radiologists, pharmacists and nurses
- various specialisms, most commonly haematolymphoid oncology, followed by general paediatric oncology and solid tumours



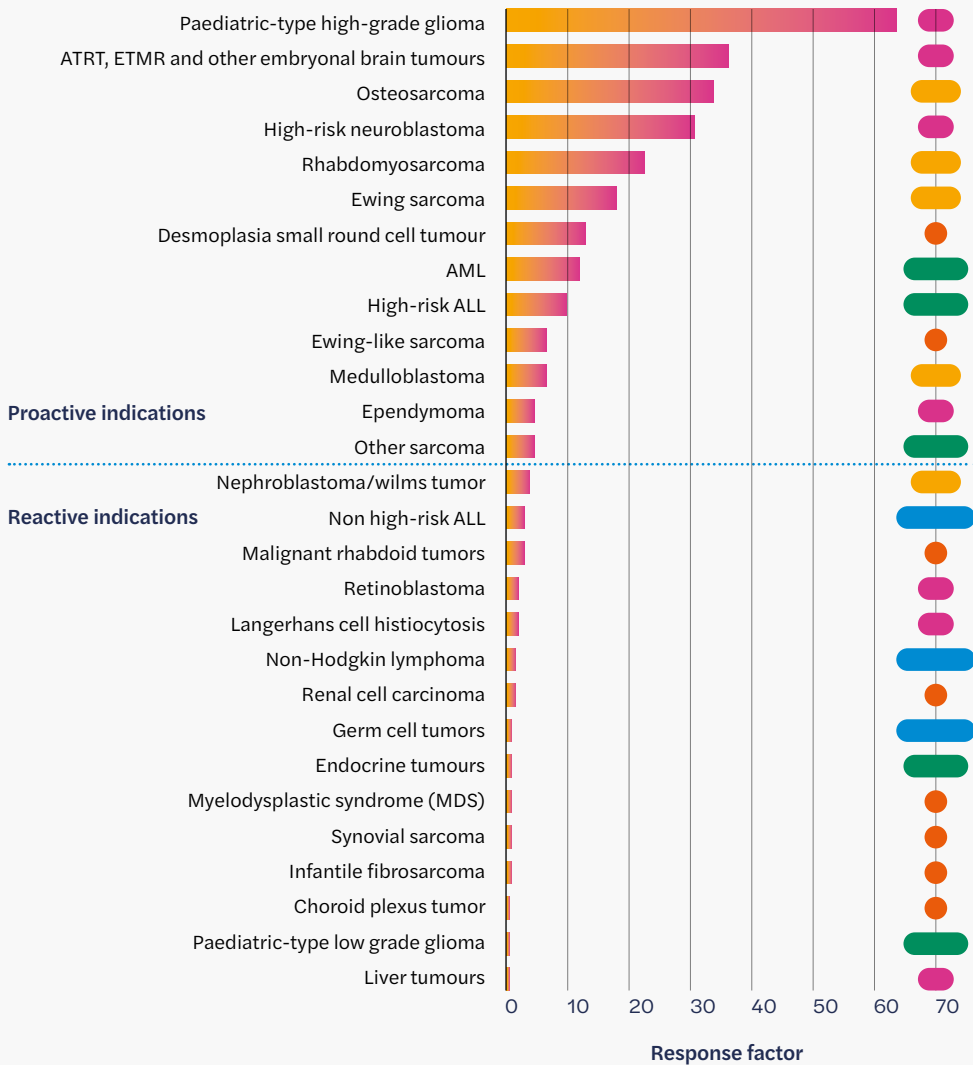
Which indications have the greatest unmet need?

Participants chose up to 3 cancer types they feel have the greatest unmet need in the clinics. To correct for the inherent bias of their own area of expertise, we normalised their answers based on their specialism.

Results are summarised opposite. Respondents recorded a total of 371 unmet needs for children with cancer – including 345 relating to specific indications. High-grade glioma was the indication most frequently mentioned and was selected as an unmet need by participants from all specialisms.

The same was true for embryonal brain tumours, which recorded the second most mentions. The case for normalisation was strengthened by the fact that certain indications were highly selected by individuals working in that speciality area.

We'll use this data to guide our research priorities, including informing a series of paediatric bespoke therapeutic development workshops, to understand the needs of specific cancer types in more detail. We look forward to sharing more information with the community soon.



Incidence (approx. avg no cases/year in UK)

- 0 - 25
- 26 - 50
- 51 - 100
- 100 - 200
- >200

Normalisation factor

1: selected by general paediatric oncologist, solid cancer oncologist or respondent specialised in a cancer group different from that which the indication belongs to

0.5: selected by respondent specialised in the cancer group that indication belongs to

What are the main challenges of these cancer types?

Participants expanded on the main challenges associated with their top 3 indications – summarised in the table opposite. Relapse and refractory disease were highlighted across all indications.



Indication(s)	Summary of key challenges
High grade glioma	A growing understanding of biology yet survival remains poor. Limited treatment options with long-term side effects.
Brain embryonal tumours	Poor prognosis, frequent relapse, long-term side effects from a young age. Research hampered by rarity of disease.
Osteosarcoma	Poor prognosis, treatments unchanged for decades. Biomarkers and new treatments incl. immunotherapy and antibody-drug conjugates show promise.
High-risk neuroblastoma	Poor prognosis, few targeted therapies in front-line, challenges to move innovative therapies into the clinic. Immunotherapies show promise.
Rhabdomyosarcoma (RMS)	Untargeted treatments with late effects; improvements have stalled. Poor prognosis at relapse and metastasis.
Ewing sarcoma	Untargeted treatments with long-term effects. Good understanding of biology; PROTACs could target driver genes.
Desmoplastic small round cell tumours (DSRCT)	Survival remains very low, no curative therapies. Patients often diagnosed late stage. Research hampered by rarity of disease.
Acute myeloid leukaemia (AML)	Challenges in moving new drugs into the clinic.
High-risk acute lymphoblastic leukaemia (HR-ALL)	Poor prognosis for high-risk disease. Treatments have toxic side effects. Cell therapies show promise.
Medulloblastoma	No targeted treatments. Radiotherapy is pivotal in therapy, resulting in toxic late effects (esp. cognitive impairment).
Ependymoma	Surgery and radiotherapy are main treatment options; drug discovery research is needed for new options.
Other sarcomas	Poor knowledge of biology. Poor survival for metastatic disease. Few actionable targets or response biomarkers.

Learnings from our consultation: where should we prioritise focus?

The lived experience experts and healthcare professionals who took part in our consultation provided invaluable perspectives into the unmet needs of childhood cancer. Below, we summarise their insights into opportunities for consideration by the wider community.

Targeted treatments, tailored to the unique biology of childhood cancer

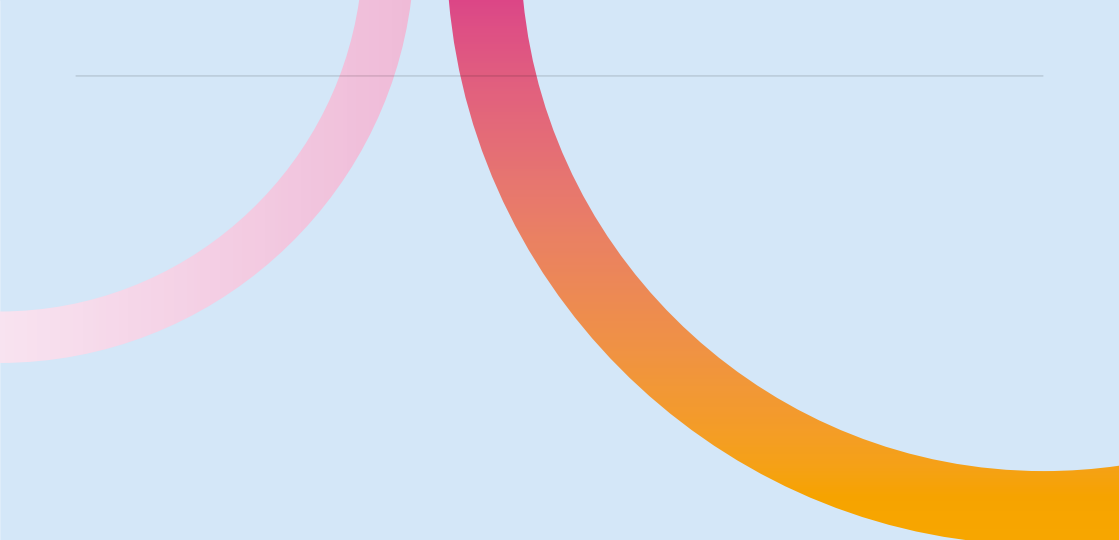
We need more effective treatments, with fewer side effects and late effects: childhood cancers are fundamentally different to adult cancers, yet often rely on repurposed or non-specific drugs. More preclinical research is needed to fuel drug discovery efforts, which also requires additional resource and prioritisation.

Translation of discoveries into new innovations

There's a growing understanding of some indications' biology that is ripe for translation into new tests, tools and treatments. New immunotherapies such as CAR T-cells, and targeted drug approaches, such as PROTACs and antibody-drug conjugates, could harness advances in our understanding.

Streamlining the path to diagnosis

Innovation in biomarkers and data could support faster diagnosis for children with cancer. A greater awareness of symptoms, particularly for rare conditions, is key to avoiding misdiagnoses and delays to treatment.



Better clinical decision-making

Biomarkers show great promise for stratifying patients and matching them to the best treatment, including individualised dosing strategies.

Prioritising areas of unmet need

Working closely with the community to understand how we could expand efforts to include cancer types with poor outcomes could kickstart progress that has stalled for decades.

Improving the patient pathway

Tailored support is needed along the entire patient pathway. Changes to policy and regulations could provide more children with access to new treatments through clinical trials, regardless of their location.

Collaboration and infrastructure

Research is hindered by the rarity of some diseases, with few preclinical models or samples and some types excluded from clinical trials. Collaboration, including building infrastructure needed for safe data sharing, is critical to boosting the efficiency of research.

A new focus on unmet needs

We're incredibly grateful to everyone who took part in our consultation to identify the unmet needs of childhood cancer.

We hope their insights will spark new ideas for how we as a research community could help drive progress for children.

As part of our strategy at LifeArc, we're building a portfolio of Translational Challenges: priority areas of unmet healthcare need where the science is ready for translation.

Childhood cancer is one of our areas of focus and the work outlined in this document has been invaluable in helping to shape our research priorities.

In September 2024, we announced our research strategy for childhood cancer, which will focus on developing new treatments, improving clinical trials, and supporting clinicians to make better decisions – areas where we feel our unique expertise, resource and infrastructure could make the greatest difference.

We are also committed to exploring the unmet needs in specific childhood cancer types, and we look forward to working with many of you to shape this, and to sharing the outcomes with you in due course.

Find out more and get in touch

lifearc.org/childhood-cancer
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