CHILDREN WITH CANCER UK

Registered Charity 298405

# OUR FREE GUIDE TO **UNDERSTANDING CHILDHOOD CANCER**

childrenwithcancer.org.uk

# Welcome

This guide has been created to explain the reality of childhood cancer in the UK today – both the remarkable progress that's been made and the challenges that remain for children and young adults up to age 25.

Whether you're:

- **Considering supporting childhood cancer research**
- **A family member or friend of someone affected**
- **Simply interested in learning more**

This guide provides evidence-based information to help you understand why research matters.

# Who we support

Children with Cancer UK funds lifesaving research and supports families affected by childhood cancer from birth through to age 25.

This includes:

- **Children (0-14 years)**
- **Teenagers and young adults (15-25 years)**
- **Families impacted by childhood cancer**
- **Adults who experienced cancer as a child**

Throughout this guide, when we say "children" we mean all young people, 0-25, recognising that cancer affects them differently at different life stages.

# The good news: survival rates are improving

By 2016, after decades of research into better treatments, early detection and understanding how childhood cancers develop, **an 86% survival rate five years after diagnosis**[1] **had been achieved**.

**2012 - 2016**
five-year survival rate

[1] Source: UK Health Security Agency, Cancer in children and young people statistics

# The Challenge: life after treatment – what most people don't know

Whilst survival rates have improved dramatically, many treatments haven't significantly advanced in decades. **This results in over 50% of childhood cancer survivors experiencing long-term side effects** from their treatment, also known as **'late effects'**.[2]

These can include:

- **Fertility problems** – inability to have children in the future
- **Growth issues** – stunted physical development
- **Heart problems** – damage from chemotherapy or radiotherapy
- **Cognitive difficulties** – problems with memory, learning or processing
- **Hearing loss or deafness**
- **Dental problems**
- **Organ damage** – to kidneys, liver, lungs
- **Bone and joint problems** – osteoporosis, fractures, osteonecrosis
- **Increased risk of secondary cancers**
- **Psychological effects** – anxiety, depression, PTSD

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[2] Sources: Children with Cancer UK, Late Effects from Treatment

# Why do late effects happen?

**The biological reality is that many current childhood cancer treatments were originally developed for adults and haven’t been sufficiently adapted for growing bodies.**

Here’s why this matters – **children’s bodies are fundamentally different from adults**:

- Their organs, bones and tissues are still developing.
- They’re more vulnerable to damage from toxic treatments.
- The same treatments that save lives can harm normal growth and development.
- Effects may not appear until years later, sometimes only when the child reaches puberty or adulthood.

# How adult-designed treatments harm children

Chemotherapy drugs, such as Methotrexate, corticosteroids and anthracyclines designed for adult bodies can damage:[3]

**Reproductive organs** – Alkylating agents and certain chemotherapies cause infertility by damaging eggs and sperm before they've even fully developed.

**Growth plates in bones** – Methotrexate and corticosteroids can cause weak or thin bones (osteoporosis) and growth problems, particularly in children treated before age 5.

**Heart tissue** – Anthracyclines (doxorubicin, daunorubicin) were designed for adult hearts; in growing hearts they cause damage that may not appear until decades later, leading to heart failure in young adulthood.

**Brain development** – High-dose methotrexate and cranial radiotherapy affect developing brains differently than mature adult brains, causing cognitive impairment, learning disabilities and memory problems.

**Lungs** – Bleomycin and other drugs cause pulmonary fibrosis (lung scarring). Because most lung development occurs in infancy and childhood, the effects are more severe than in adults.

**Peripheral nerves** – Vincristine and platinum-based agents cause peripheral neuropathy (numbness, tingling, pain) that can be permanent in growing children.

**Endocrine system** – Treatment disrupts hormones controlling growth, development and puberty.

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[3] Sources: Children with Cancer UK, Learn about Children's Chemotherapy, British Medical Journal Anticancer chemotherapy in teenagers and young adults: managing long term side effects

# The dosing problem

**A critical issue:** Many chemotherapy doses are calculated for adult body weight (typically 70kg) and then scaled down for children.

**But children aren't just "small adults"** because their:

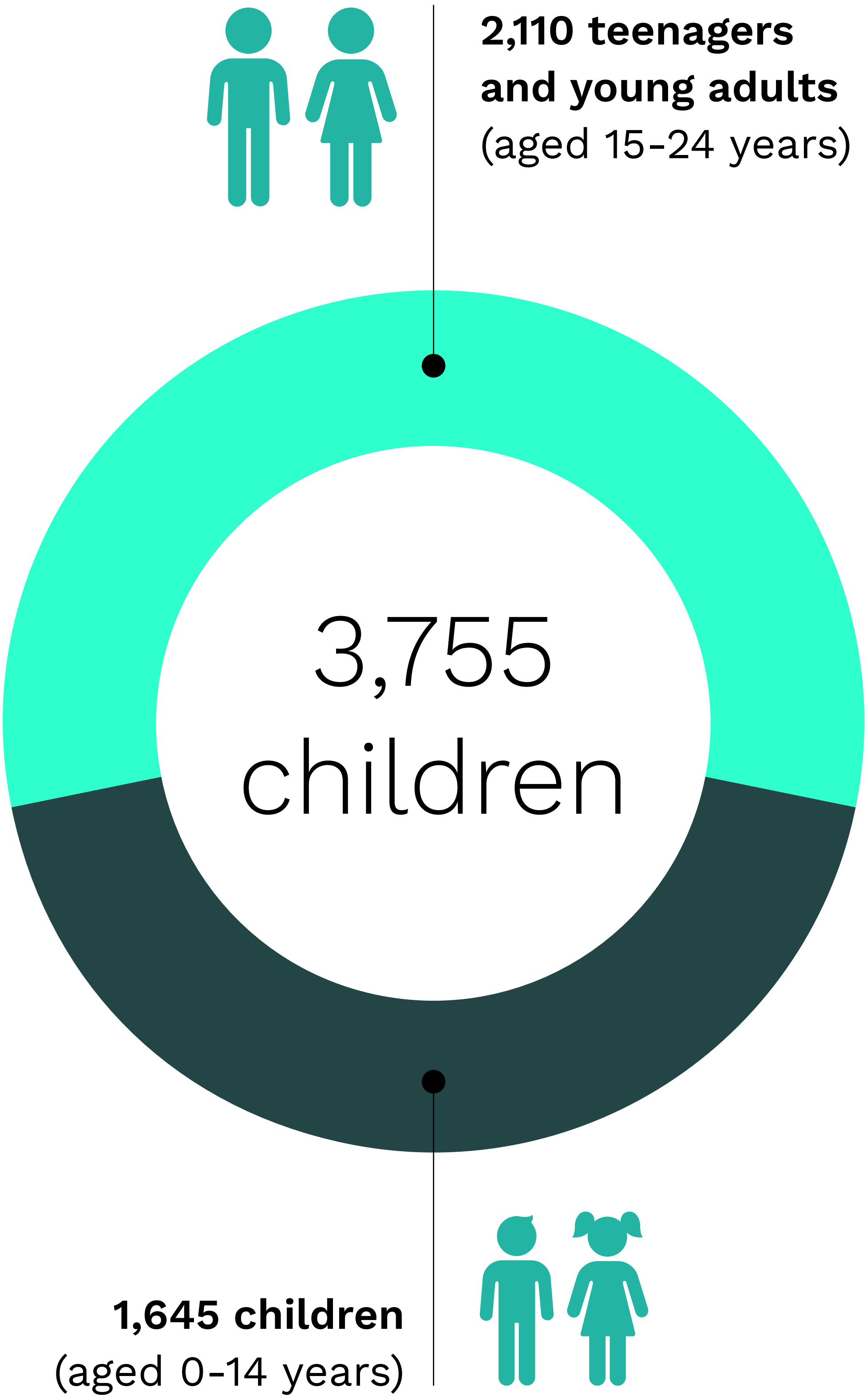
- Metabolism processes drugs differently
- Organs are still forming
- Cells divide more rapidly (which makes them more vulnerable)
- Bodies have decades ahead to develop complications

# The scale of the challenge in the UK

**Approximately 3,755 children and young people under 25** are diagnosed with cancer in the UK annually:[4]

- **1,645 children** (aged 0-14 years)
- **2,110 teenagers and young adults** (aged 15-24 years)

That breaks down to 10 children and young people diagnosed every single day.



**2,110 teenagers and young adults** (aged 15-24 years)

**1,645 children** (aged 0-14 years)

[4] Sources: Children with Cancer UK, Childhood Cancer Facts & Statistics

# Beyond age 14

Whilst much focus is on younger children, teenagers and young adults (TYAs) face unique challenges:

- **Different cancer types** – TYAs get different cancers (more carcinomas, melanomas, germ cell tumours, lymphomas).
- **Different life stage** – Treatment interrupts education, career planning, relationships, independence.
- **Treatment gaps** – Many treatments are designed for either children OR adults, leaving TYAs in treatment limbo.
- **Lower survival rates** – For some cancers (bone tumours, soft tissue sarcomas), TYAs have worse outcomes than younger children.[4]

**Cancer remains the most common cause of death in children and young people beyond infancy.**[5]

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[5] House of Commons Library, Childhood Cancer Report

# What research can change: The gap Children with Cancer UK is trying to fill

Current research into childhood and young adult cancer is working to:

1. **Reduce Treatment Toxicity:** Develop treatments that kill cancer cells whilst protecting healthy, growing tissues.
2. **Preserve Fertility:** Create protocols that cure cancer without destroying reproductive capability – particularly critical for teenagers and young adults.
3. **Protect Growth and Development:** Design treatments that don't damage growth plates, hormone systems or organ development.
4. **Minimise Cognitive Impact:** Reduce effects on learning, memory and brain development – especially important for young children whose brains are still forming.
5. **Prevent Cardiac Damage:** Develop treatments that don't cause heart problems that appear 20-30 years later.
6. **Eliminate Pulmonary Toxicity:** Create treatments that cure without causing lung scarring and breathing problems.
7. **Prevent Secondary Cancers:** Lower the risk that treatment itself causes future cancers.
8. **Improve Quality of Life:** Ensure children and young adults don't just survive, but thrive after treatment.

# Our approach to research

## What we fund

Children with Cancer UK funds research across four strategic priorities:[6]

1. **Understand and Discover:** Support discovery and translational cancer research through project awards, which helps scientists learn new things about childhood cancer in the lab and then turn those discoveries into actual treatments for patients.
2. **Early Detection, Diagnosis and Prevention:** Fund research enabling detection at the earliest point so that intervention might be made.
3. **Develop Treatment:** Fund pre-clinical projects aiming to progress onto future clinical trials – specifically treatments designed FOR children and young adults, not adapted FROM adult treatments.
4. **Impact;** Maximise impact of research findings and provide follow-on funding.

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[6] Sources: Children with Cancer UK, We Fund Research

## A unique investment in research

Since 1988, **Children with Cancer UK has raised over £300 million** to fund childhood cancer research.[7]

**We primarily fund:**

- UK universities and research institutions
- Medical and scientific research centres
- Hospitals conducting clinical trials
- Research fellowships and professional development
- Infrastructure for long-term research programmes

**Research must be:**

- Peer-reviewed by our Scientific Advisory Panel
- Based at UK research organisations
- Aligned with our strategic priorities
- Focused on improving outcomes for children and young adults (0-25 years) with cancer

## Our research partners

We collaborate with many of the UKs leading institutions and charities on research, including:

- **UCL Cancer Institute**
- **Great Ormond Street Hospital**
- **Institute of Cancer Research**
- **Royal Marsden Hospital**
- **Northern Institute for Cancer Research**
- **Children's Brain Tumour Drug Delivery Consortium**
- **Tessa Jowell Brain Cancer Mission and many others.**

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[7] Sources: Children with Cancer UK, We Fund Research

**We're also members of:**

- **Association of Medical Research Charities** (AMRC)
- **National Cancer Research Institute** (NCRI)
- **The Children and Youngs People Association** (CCLG)

# Some breakthroughs we have funded

**Minimal Residual Disease (MRD) Test for Leukaemia**
In the early 2000s, we co-funded a major clinical trial that led to development of the MRD test, which measures how much leukaemia remains after treatment and predicts relapse risk. Today this test is used by the NHS and hospitals worldwide.[8]

**Precision Medicine Programme** In 2017, we invested £3.7 million into development of a new Precision Medicine programme involving detailed genetic profiling of children with cancer and their tumours – helping match treatments to individual biology rather than using one-size-fits-all approaches.[8]

**Research Infrastructure** Since our founding, we've provided funding for five research centres in England and Scotland, ensuring the infrastructure exists for breakthrough research into child-specific treatments.[8]

**CAR-T Therapy** We fund and support research into CAR-T cell therapy – a cutting edge immunotherapy that genetically modifies and attacks cancer cells. The hope is to develop treatments for hard-to-treat childhood cancers like acute myeloid leukaemia and neuroblastoma.

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[8] Source: Children with Cancer UK, Our Impact and Success

# Research into childhood cancer faces significant funding challenges

Despite childhood cancer being devastating for families and affecting thousands of children and young adults annually:

**Research funding is disproportionately low** compared to adult cancers

**Why this matters for children and young adults specifically:**

**Commercial pharmaceutical companies invest less** because:

- The patient population is smaller (though 3,755 annually is significant)
- Development costs are similar to adult drugs
- Commercial returns are lower
- Regulatory requirements are complex
- Clinical trials in children require special ethical approvals
- Each type of childhood cancer is relatively rare
- Research requires international collaboration
- Effects may not appear for decades, making research slow and expensive

**This means charities like Children with Cancer UK play a crucial role** in funding the research that commercial entities won't pursue – research specifically designed for growing bodies, not adapted from adult protocols.

# Kira's story: living with Neuroblastoma

**Kira was diagnosed with neuroblastoma in 2014 when she was just 11 years old. She and her mum, Aud, share their journey of hope and resilience.**

## An unexpected discovery

When Kira was 11, doctors found a mass the size of a watermelon in her abdomen. It was neuroblastoma, a rare cancer more commonly seen in children under five. The diagnosis changed everything.

## 11 years of fighting back

Over the next 11 years, Kira faced treatments including chemotherapy, surgery, radiotherapy, and a stem cell transplant. Her cancer returned eight times. When doctors said a growing tumour wrapped around major blood vessels was too dangerous to remove, her family refused to give up. Through community support and donations, they travelled to a specialist surgeon in New York who successfully removed it.

## Finding hope through innovation

After treatment in America in 2018, scans showed Kira’s cancer had spread to multiple locations within her abdomen. But Kira was determined. She became one of the first UK children to try a new targeted therapy called Lorlatinib, combined with cutting-edge blood testing that tracked her cancer in real-time.

> **Proton beam therapy had devastatingly not worked and Kira’s cancer continued to grow. Not only had it not worked, but the tumours had multiplied and spread.**
>
> Aud, Kira’s Mum

## Additional treatment

Kira’s cancer further relapsed at the end of 2021 and her liquid biopsy showed that the same ALK mutation remained but had increased in percentage in her bloodstream. It was crucial that Kira remained on the ALK inhibitor and add in an additional therapy such as chemoimmunotherapy.

## Living life to the fullest

After six months of treatment, the tumour became inactive, with no evidence of active disease on a scan. Kira has remained that way to date. Her blood tests continue to show her to be in sustained molecular remission since the beginning of 2022, which means that there are no detectable mutations in her circulating DNA. Over the past six years, Kira's tracking of her ctDNA in her blood has correlated perfectly with her clinical course and imaging.

Now 21, Kira lives life on her own terms.
Having faced cancer nine times has shaped her, but it hasn't defined her.

> **I always squeeze the joy out of life.**
>
> Kira

## Stratified Medicine Paediatrics (SMPaeds1) programme

Thanks to funding Children with Cancer UK, Kira enrolled on SMPaeds Serial Blood Testing, which tracked her response to treatment alongside targeted cancer treatment. She was one of the first children in the UK to receive this serial blood testing alongside Lorlatinib.

SMPaeds1 aims to advance precision medicine for children and young people whose cancer has returned. This treatment focuses on analysing childhood cancer tumours at diagnosis and relapse stages of the cancer journey, to help better understand how the tumours evolve.

The research team from **The Institute of Cancer Research, London** introduced the use of ctDNA, DNA that is released into the bloodstream circulation by cancer cells, as a potential tool for tracking cancer progression and identifying new treatment targets. The ctDNA test offers a less invasive way to monitor how tumour mutations change over time and can add additional information that is complimentary to tissue biopsy.

> **We are hugely grateful to Children with Cancer UK for investing invaluable funds into SMPaeds Serial Blood Testing for all children with relapsed cancers and believe it will make a positive difference to outcomes for children living with cancer.**
>
> Kira and Aud, March 2025

# Some key takeaways

**More than 8 in 10 children and young people now survive cancer** – remarkable progress thanks to research.

**But more than 50% of survivors face long-term side effects** – including infertility, stunted growth, heart damage, cognitive problems, organ damage.

**Many treatments were designed for adult bodies** – children's growing bodies are more vulnerable to toxicity and long-term damage.

**The dosing and drug selection problem is real** – what works for a 70kg adult affects a 20kg child or developing teenager very differently.

**3,755 children and young adults (0-25) are diagnosed annually in UK** – that's 10 every day.

**Research can change this** – developing age-appropriate treatments that preserve both life and quality of life is vital.

**Funding is the barrier** – commercial entities under-invest in paediatric cancer research due to smaller market size.

**Charities fill the gap** – organisations like Children with Cancer UK fund research specifically designed for children and young adults, not adapted from adult treatments.

**If you are able to make a donation, please click the button below. Thank you.**

**Donate Now**

## Contact us

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Our offices are open 9am-5pm, Monday-Friday.

This guide is provided free of charge to help increase understanding of childhood cancer research and the specific challenges faced by children and young adults with cancer. No email address or personal information is required to access this information.