

Doing better for children with cancer

LifeArc Childhood Cancer
Translational Challenge Strategy



Foreword

Cancer remains a leading cause of death by disease in children around the world. Although recent decades have seen a steady improvement in survival for some cancers, rates have plateaued, and the consequences of a 'cure' are significant: those who survive childhood cancer have an increased risk of developing a second cancer or other major health challenge, and are more likely to die prematurely.

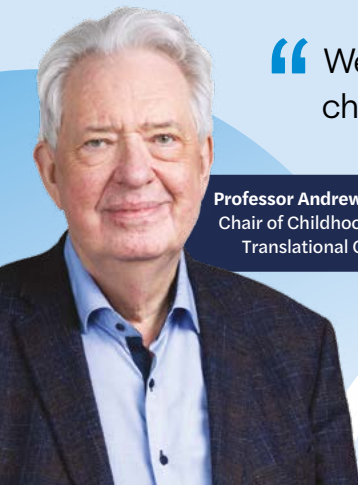
We must do better for children with cancer. Childhood cancers are fundamentally different to adult cancers, and we need bespoke treatments that target the unique features of their disease; we need novel tests to diagnose more quickly, monitor more accurately and less invasively, and rapidly match to the best available treatment. Most importantly, we must accelerate the speed with which new innovations reach the clinic.

We need a paradigm shift: to give more children the chance of a cure, and to reduce the risk of adverse effects for survivors.


At LifeArc, we're uniquely positioned to drive this change and tackle some of the challenges associated with slow progress. As a self-funded charity with a focus on helping promising discoveries reach their potential, we can take on high-risk projects that may be overlooked by others. We have the capabilities and expertise, resource and collaborative, convening power to optimise new innovations and bring them closer to the patients who need them.

We made childhood cancer one of our 7 Translational Challenges in 2023 – priority areas of unmet healthcare need where the science is ripe for translation. We're delighted to share our bold new research strategy with you. A strategy that has collaboration and partnership at its heart, and which we hope will catalyse sustainable change for children with cancer. We look forward to connecting and working with you.

“ We need a paradigm shift in the way children with cancer are treated.”



Professor Andrew Pearson
Chair of Childhood Cancer
Translational Challenge



Dr David Jenkinson
Head of Childhood Cancer
Translational Challenge

**400,000
children**

(up to age 19) are
diagnosed with cancer
each year, globally*

9x

increased chance of
**premature
death**

in childhood
cancer survivors**

In high-income
countries, around
**80% of
children
with cancer
survive***

Childhood cancer
survivors have a
**higher risk of
developing a
new cancer*****

*World Health Organization (2019) | **Fidler et al. BMJ (2016) 354:i4351

***Bhatia et al. JAMA (2023) 330:12

Shaping our strategy: informed by patients, guided by experts

We're very grateful for the expertise and insight shared with us by members of the childhood cancer community, including clinicians, opinion leaders, charities and people with lived experience.

Together, we've explored the barriers that continue to stifle progress and identified areas where we need to collaborate to break them down.

This consultation was invaluable in shaping our strategy, which will address the main barriers to translation and approval of new tests and treatments — so that children with cancer are diagnosed quickly, monitored more efficiently and less invasively and treated with bespoke therapies.

Challenges we hope to address through our Childhood Cancer Translational Challenge

Limited treatment options

Too often, children are reliant on adult cancer therapies that are inefficient or cause adverse effects, immediately or later in life. We need bespoke therapeutics that target the unique drivers of childhood cancer, and we need to ensure they have efficient routes to market.

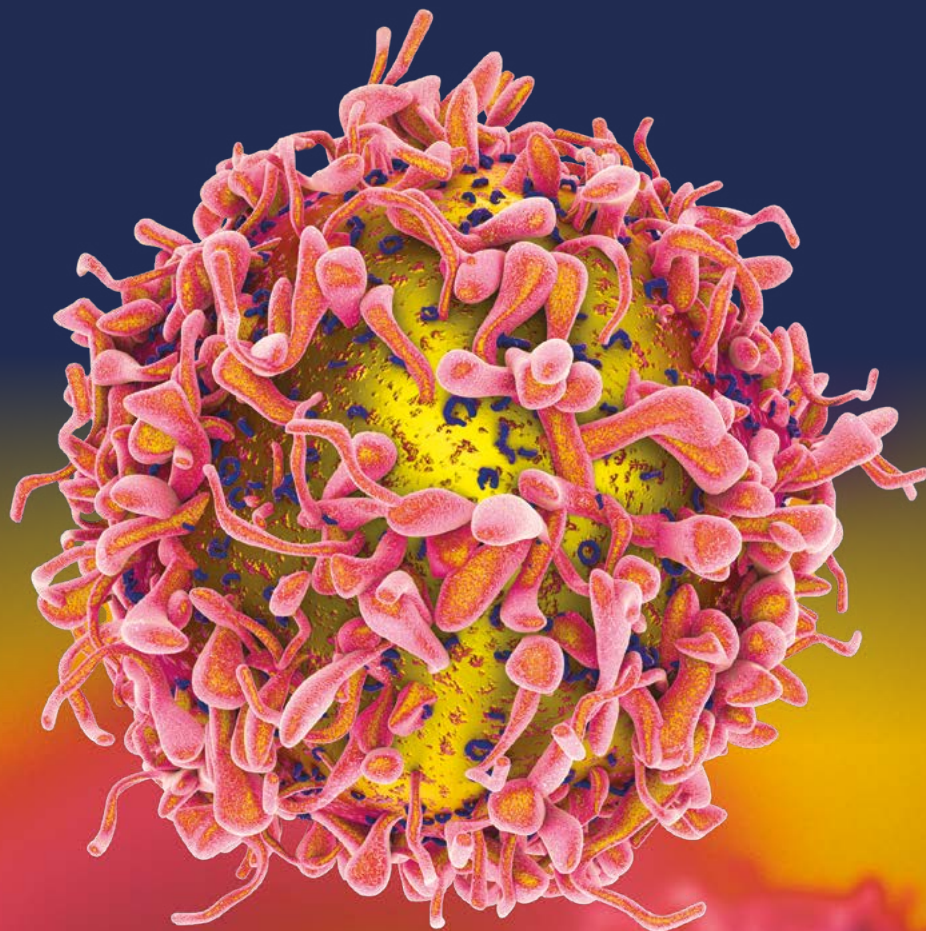
Inefficient clinical trials

Fragmented trials lead to duplication of efforts, and complex approval processes delay innovations from progressing to market.

Delayed diagnosis and invasive monitoring

Inadequate, invasive tests make it difficult to match patients to the best therapy and right dose for them, and to monitor how their treatment progresses.

Driving life changing innovations for children with cancer



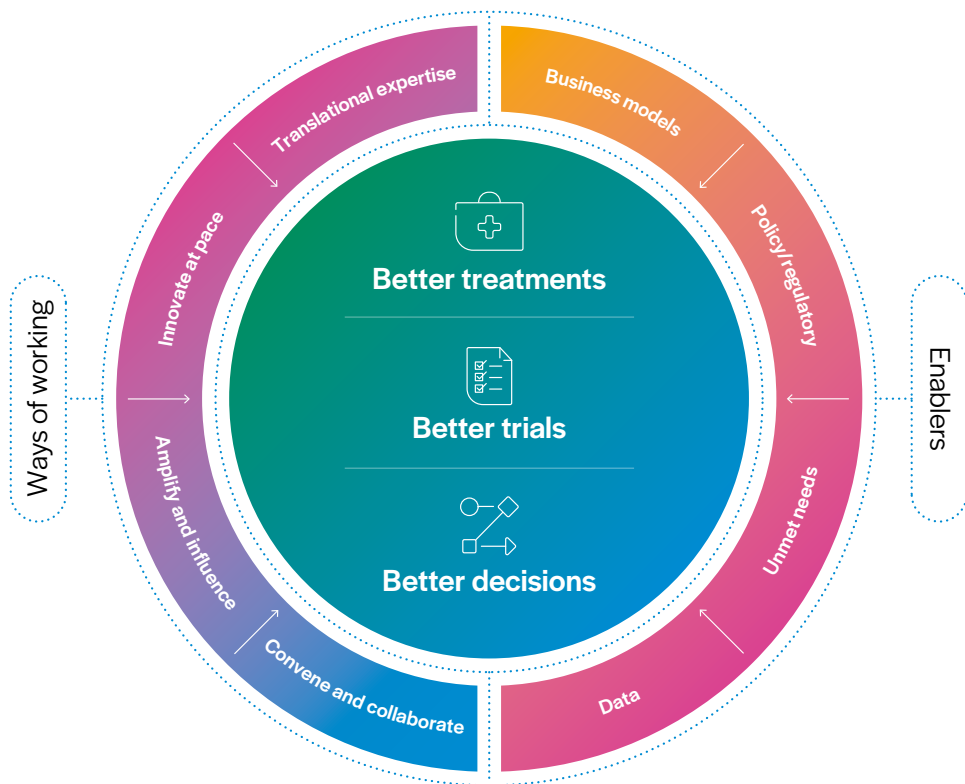
Our Childhood Cancer Translational Challenge strategy

We want children with cancer to be diagnosed quickly and treated with therapies that are targeted and tailored to their individual needs.

To achieve this, we'll concentrate on 3 strategic priorities: better treatments, better trials and better decisions.

Powering our success are our enablers. For example, sharable, high-quality data will boost the efficiency of clinical trials, while new business models will encourage engagement by industry. We'll prioritise areas of unmet need and work with policy makers to overcome regulatory hurdles.

We know we won't get there alone, and collaboration underpins everything we do. This, combined with our translational expertise and resource will fuel us to innovate at pace.





The strategic priorities of our Childhood Cancer Translational Challenge strategy

Better treatments

Too often, children are reliant on adult cancer therapies, designed for diseases of fundamentally different biology.

We urgently need therapeutics targeted to the unique features of childhood cancers, but a major challenge remains the translation of new discoveries into the clinic. Additionally, traditional commercial models, combined with small population sizes and low returns on investment, can make commercialisation a challenge.

Our multi-faceted approach to driving change includes:

- collaborating with partners to augment our expertise, resource and capabilities in translation and early drug development
- exploring how we could target 'undruggable' drivers of childhood cancers
- rescuing drugs that have stalled during development for commercial reasons and exploring their potential in combination with other treatments

By addressing funding and infrastructure gaps in the translational development pathway, we hope to progress promising treatments to children who need them.

Delivering a new roadmap for therapeutic innovation

Collaboration is key to achieving sustainable change for children with cancer. That's why we've joined forces with Cancer Research Horizons to establish C-Further, an international consortium that will focus on developing bespoke cancer therapies for children and young people.

Working with a network of partners, we'll build a pipeline of potential therapeutics that could benefit from our collective expertise and capabilities to move to the next stage of development. We'll lay the foundations for a long-term, sustainable framework for the translation of new treatments for childhood cancers.



Better trials

All children with cancer should have access to clinical trials if they need it. Trials are not only a vital step in the development of new therapeutics, but they can also provide access to potentially life-saving treatments.

We're broadening our translational research focus to include early phase trials and beyond, de-risking novel drugs for further downstream development.

We aim to:

- reduce trial fragmentation, with collaborative networks that provide oversight of therapies in development
- support disease-specific platform trials that are adaptive, meet the data threshold required for regulatory approval, and are accessible to children and young people when they need them
- develop technologies to aid individualised dosing, maximising the chance of successful treatment with minimum side effects

Improving clinical trials will ensure the most promising new treatments reach the children who need them more quickly.

Better decisions

Effective biomarkers could make a huge difference to a child's journey with cancer, guiding clinicians to make informed decisions at diagnosis and throughout treatment.

Initially, we will focus on:

- diagnosis, providing primary care practitioners with the tools they need to refer children with concerning symptoms to specialist clinicians
- making monitoring more efficient and less invasive, with the development of accurate tests that could replace scans and bone marrow biopsies
- guiding treatment decisions by predicting an individual's risk of life-limiting side effects now, and later in life

Our translational expertise and validation capabilities will help drive promising biomarkers closer to the clinic – leading to more effective, less invasive treatment decisions and reducing the risk of adverse late effects for children with cancer.

What will success look like?

While some areas of healthcare research have clear paths of translation, the same can't currently be said for childhood cancer.

This needs to change. Through our Childhood Cancer Translational Challenge, we will build a portfolio of partnerships and initiatives, creating a lasting roadmap to guide the bench-to-bedside translation of new treatments and tests for children.

Our work, in collaboration with like-minded partners, will ensure more children with cancer are diagnosed quickly, treated with more effective, targeted therapies and suffer fewer long-term effects. We look forward to the day when more children survive their diagnosis and lead quality, independent lives, long into adulthood.



We will do better for children with cancer.



**Childhood Cancer
Translational
Challenge**

**Find out more and
get in touch**

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