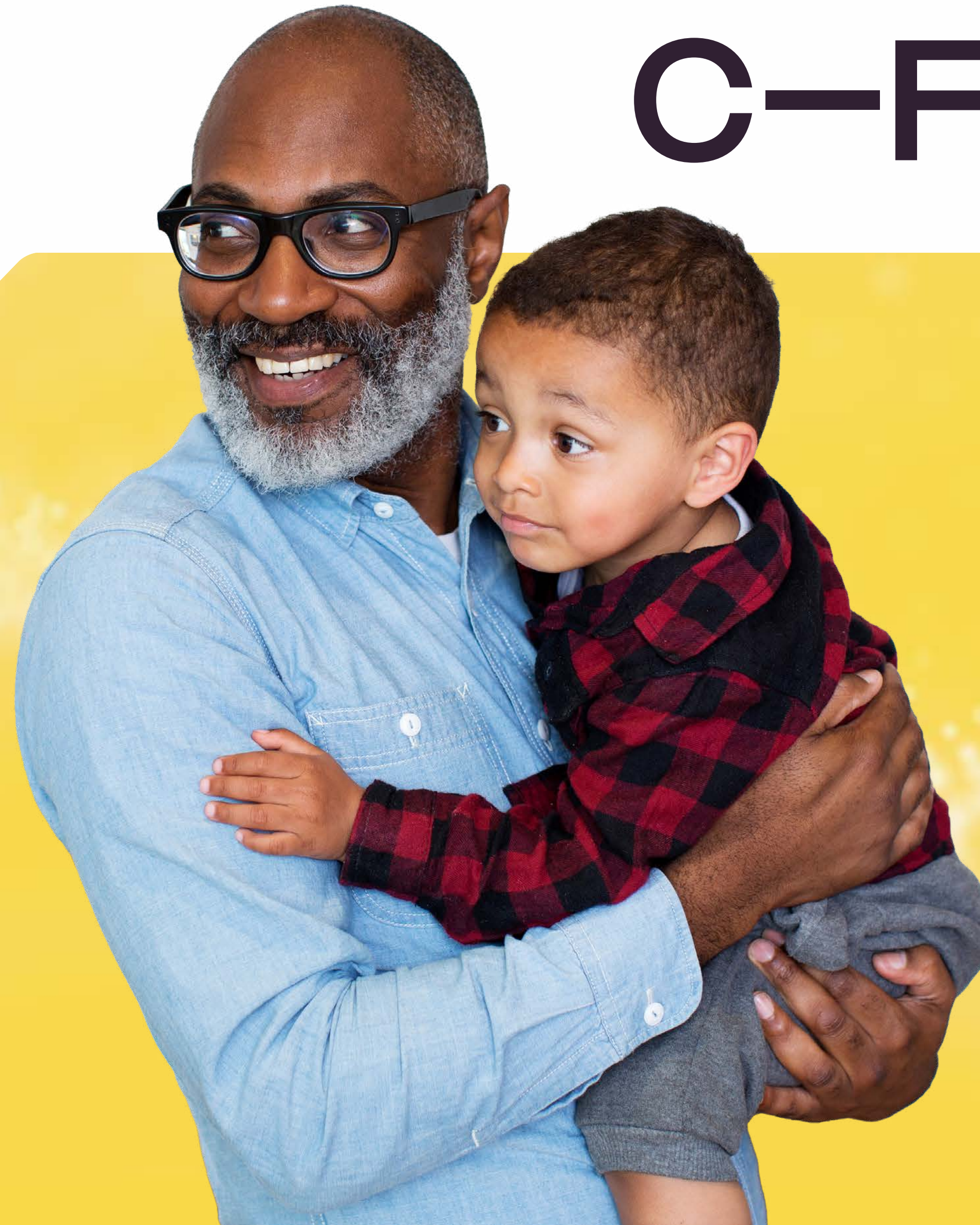


C-FURTHER



Annual review

2025/26



Executive summary

As we come to the end of C-Further's second year, we are encouraged by the progress being made across the children's and young people's cancer landscape. From policy developments such as the reauthorisation of the US Priority Review Voucher programme to promising results from early clinical trials, momentum continues to build.



James Pickett, PhD,
Co-lead, Cancer Research Horizons

One moment showed the challenges we still face, and the solutions still needed. In June 2026, the non-profit Blood Cancer United stepped in to acquire the remaining drug supply of Luvelta. The therapy had been developed for adult cancers, but had also become a lifeline for children with an ultra-rare, often-fatal leukaemia, until its manufacturer discontinued the programme entirely. Even indirect, secondary access for children, it turns out, isn't immune to the adult-oncology dynamics that too often leave young patients behind, underscoring the sector's growing determination to change this.



Lone Friis, PhD,
Co-lead, LifeArc

It is precisely this challenge that sits at the heart of why C-Further was created. Our mission is clear: to develop targeted, child-first therapeutics, beyond adult drug repurposing, while helping engineer the commercial frameworks required to keep viable medicines from being deprioritised. Two years in, we report early, but meaningful, strides across several fronts.



Scientific pipeline: We unveiled our first two therapeutic projects, CF-012 and CF-033, co-developed with leading global academic and industry partners.

Systemic frameworks: We've started a large multi-stakeholder analysis to dissect macro and microeconomic bottlenecks in paediatric oncology and build better models.

Community & engagement: We held our inaugural in-person session with our Patient Advocate Panel, while expanding institutional presence and engagement with the scientific community worldwide.

12 months, what changed for C-Further:

- Launch of dedicated pipeline of child-first therapeutics
- Continuous identification and progression of promising science caught in the gap between discovery and commercial investment
- Bring commercial expertise into projects earlier to maximise chances of progression
- Give patient perspective a direct role into our decision-making



Behind the scenes

Since launch, C-Further has:

EVALUATED

100+

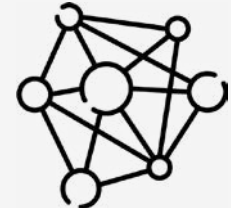
collaboration proposals



SPANNING

9

modalities



16

countries



12+

indications



In-person meeting with C-Further's panel of patient advocates. Brussels, February 2026.



For children and young people with cancer, the standard of care should be built on what science now makes possible, not on decades-old agents whose devastating consequences can last a lifetime.

This was also Great Ormond Street Hospital Charity's first year as a founding member of C-Further. Their £10m investment, the largest research partnership commitment in the charity's history, is already helping drive that mission forward. As we continue to write the C-Further story, we would like to thank all the members of our founding organisations, networks, colleagues and advocate panel, as well as everyone who contributes to the progress of this ambitious venture.

We are excited to share with you the advancements made over the past 12 months. If you share our purpose that young patients deserve better medicines, do connect with us now:

info@c-further.org



C-Further's delegation visiting Victoria's Children's Cancer CoLab team. Australia, April 2026.



Co-organised by C-Further and AACR, the panel "Rewriting the rules of childhood cancer translation" regrouped world-leading investigators in paediatric oncology. Boston, September 2025.



Grants fund the research; we fuel the pipeline:

Addressing the challenges of children and young people's cancer, whether scientific or commercial, requires more than investment. To give the community the best chance of bridging the translational gap, we shaped C-Further as an independent strategic venture, operating under an industry-style model.

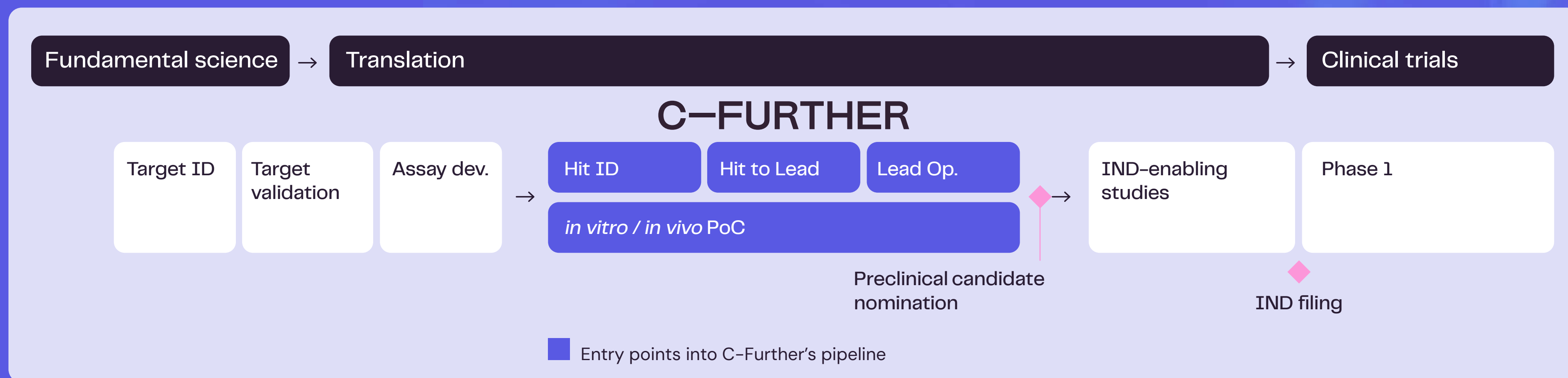
Unlike traditional grant-awarding bodies, we actively partner with academic researchers and industry innovators, with an approach defined by three distinct pillars:



Promising science for children and young people with cancer is not the limiting factor but transitioning it to the next phase requires a completely different biotech-grade engine.

We apply active portfolio management, strict milestone governance, and commercial expertise throughout to ensure every asset in our pipeline is built for clinical impact and sector adoption.

Neil Jones, PhD,
Science Delivery, Cancer Research Horizons



1 Drug discovery capabilities & active project and portfolio management

We function as an active drug discovery partner. Drawing on our founding organisations' track record – which contributed to over 20 marketed oncology and rare disease drugs – we provide scientific, translational and commercial expertise throughout the drug discovery journey.

Projects enter our portfolio at an early stage, typically between hit identification and lead optimisation. Each programme is advanced through a structured, milestone-driven framework, managed by C-Further with guidance from boards and external experts.

2 Specialist networks required to move science forward

Scientific breakthroughs alone are not enough. Successful translation requires clinical expertise, commercial insight, intellectual property and onward development strategies tailored to the unique specificities of small-cohort indications. We bring together specialists from these areas to navigate the complex challenges in childhood cancer drug development and design viable pathways for child-first innovation.

Backed by three of the UK's leading medical research organisations, we also carry the collective credibility and leadership to contribute to the conversations that can change the system for the better.

3 Non-profit mission with commercial rigour

Backed by \$50m (£37m), we focus on bespoke paediatric targets across diverse modalities, absorbing early translational risk to convert promising science into investable, industry-ready assets. By combining a non-profit mission with commercial discipline, we look to create viable routes for advancing therapies that might otherwise be left behind.

[Our operating model](#)

[What makes a strong collaboration](#)



Our first research projects

Project CF-012:

a potential first-in-class ETV6 inhibitor for Ewing sarcoma

Project ID: CF-012
Target: ETV6
Modality: Small molecule inhibitor
Stage: Early drug discovery
Indication: Ewing sarcoma

Research Collaborators:



Project CF-012 aims to target a critical unmet need in relapsed and metastatic Ewing sarcoma, an aggressive bone and soft tissue cancer primarily affecting adolescents and young adults.

The proposed approach

The disease is driven by the EWS-FLI1 fusion oncogene, a transcriptional driver long considered "undruggable". The approach for project CF-012 is to bypass this bottleneck by targeting ETV6, a newly identified dependency that competes with EWS-FLI1 for chromatin binding.

By inhibiting ETV6, project CF-012 aims to disrupt the EWS-FLI1 transcriptional activity and downstream regulatory elements that promote tumour growth and metastasis.



The strength of our pipeline relies on a powerful synergy: combining the deep biological insights of our research partners with our proven drug discovery capabilities and translation expertise. This allows us to progress at speed for young patients.

Caroline Barton, PhD,
Science Delivery, LifeArc

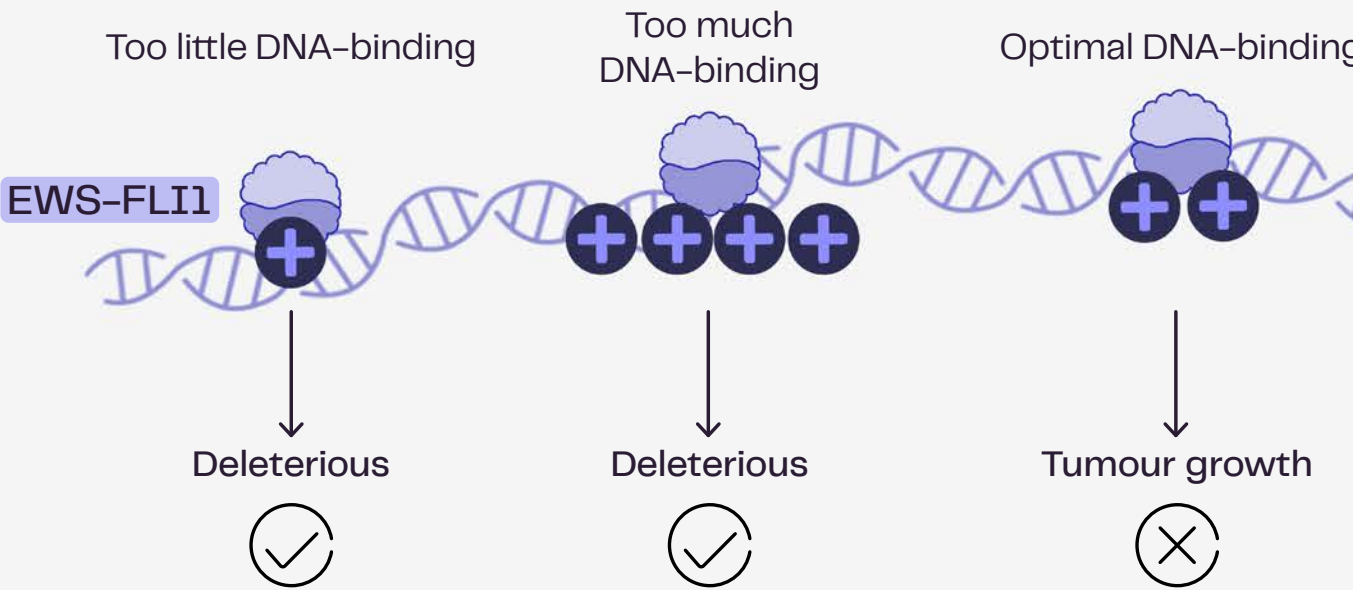




The proposed mechanism of action

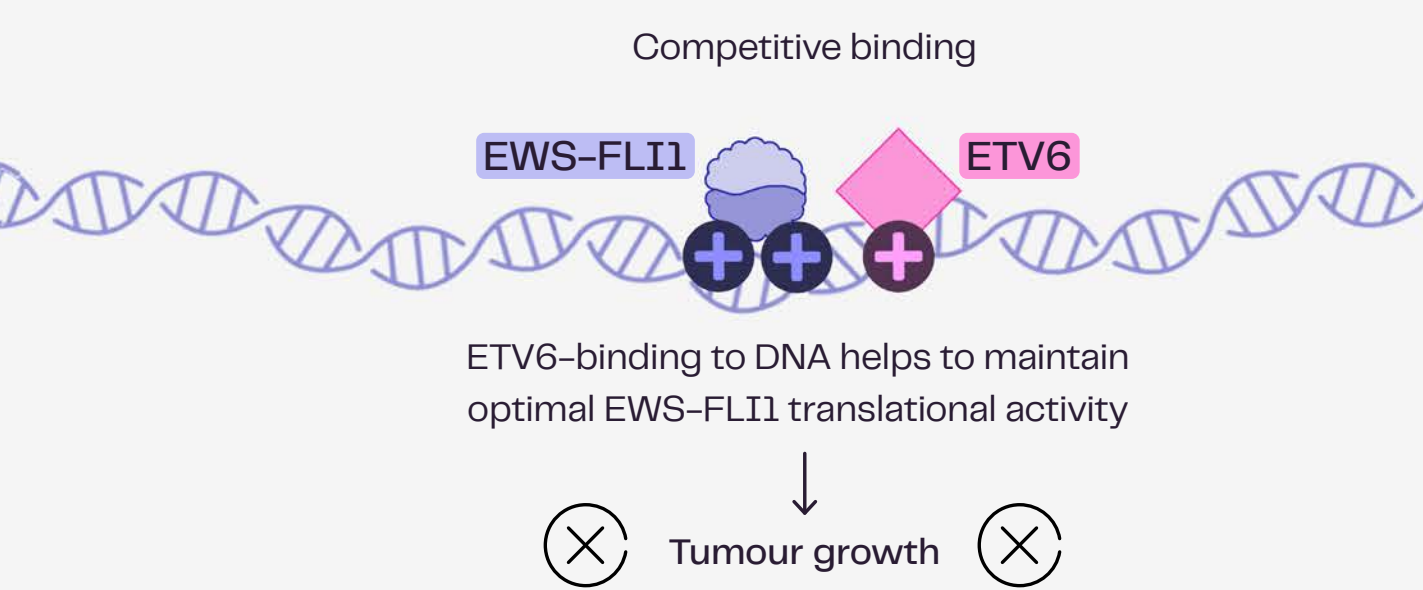
1. The challenge

Tumours growth is driven by **EWS-FLI1**, an aberrant transcription factor fusion, and depends on its finely tuned transcriptional state.



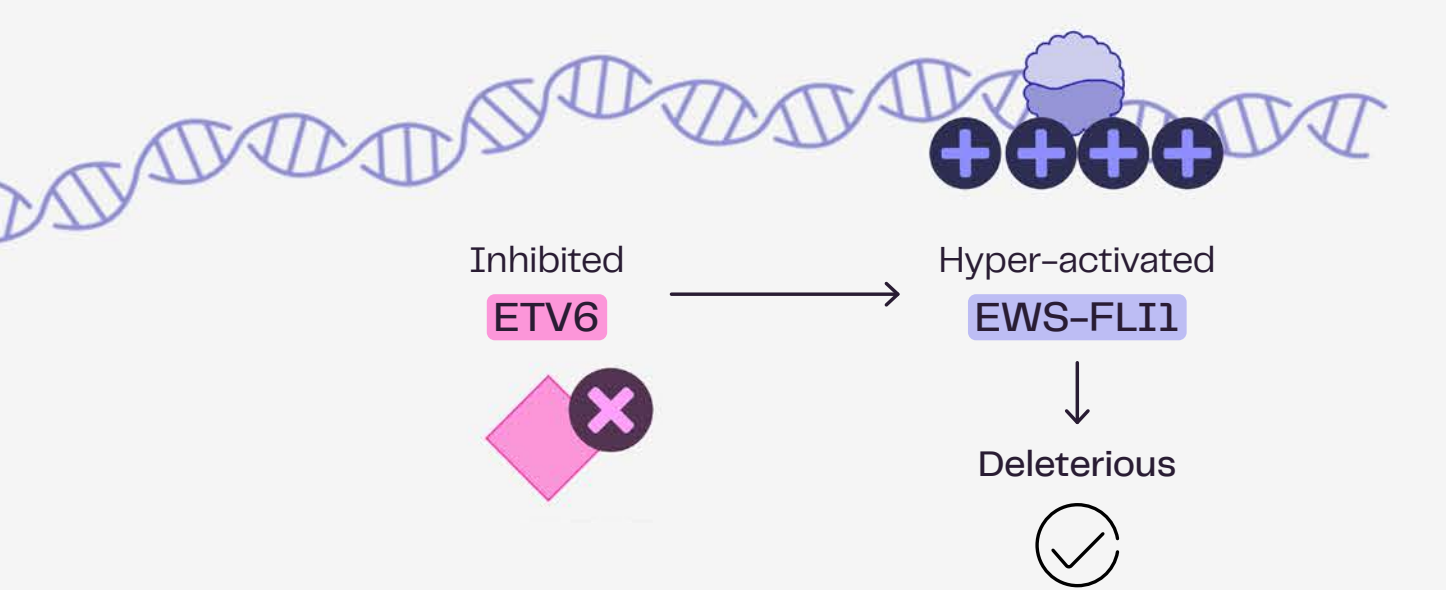
2. The vulnerability

ETV6 is a transcriptional repressor that competes with **EWS-FLI1** for chromatin-binding.



3. The disruptive approach

Inhibiting **ETV6** shifts this balance: **EWS-FLI1** overtakes and hyperactivates regulatory elements that disrupt tumour growth and metastasis.



Why it matters

The standard of care for Ewing sarcoma patients whose disease remains localised comprises surgery, chemotherapy and radiation. While this helps survival rates exceed 70%, the short-, medium- and long-term side effects are devastating to many vital organs such as the kidneys and the heart.

However, for 25% of patients, outcomes are much less favourable. Once the disease has relapsed or spread beyond its original site, the five-year survival rate drops below 30%, often closer to 13-20%. New treatment options are urgently needed.

The project is being developed through a collaboration between C-Further and leading academic partners, bringing together expertise in cancer biology, drug discovery and translational research.

Research partner leads



John Bushweller, PhD, Professor of Molecular Physiology and Biological Physics University of Virginia



Kimberly Stegmaier, MD, Chair of the Department of Pediatric Oncology Dana-Farber Cancer Institute



Miguel Rivera, MD, Associate Professor of Pathology Massachusetts General Hospital



Louise Tonkin, PhD, Group Leader Cancer Research Horizons

C-Further project lead



Project CF-033:

a potential first-in-class allogeneic PRAME-targeting iNKT cell therapy

Project ID: CF-033
Target: PRAME
Modality: Cell Therapy
Stage: Early drug discovery
Indication: Multiple

Research Collaborators:



Project CF-033 is a potential cell therapy applicable to multiple high-risk childhood cancers, often characterised by treatment resistance and a weakened immunity that cannot sustain tumour control.

The proposed approach

A Dual-targeting anti-cancer activity: Project CF-033 combines an engineered PRAME-specific TCR iNKT cell with native NK-cell receptors, to specifically target cancer cells. Simultaneously, CF-033 aims to modulate immune cells in the tumour microenvironment, to enhance immune effector function and reduce immunosuppression.

B An “off-the-shelf” format designed with children in mind: Project CF-033 aims to replace long patient-cell extraction, engineering and expansion, a delay critically ill children cannot afford, with pre-made donor cells available for immediate use. Crucially, it aims to bypass toxic lymphodepleting pre-treatments, offering a significantly safer profile for fragile patients.



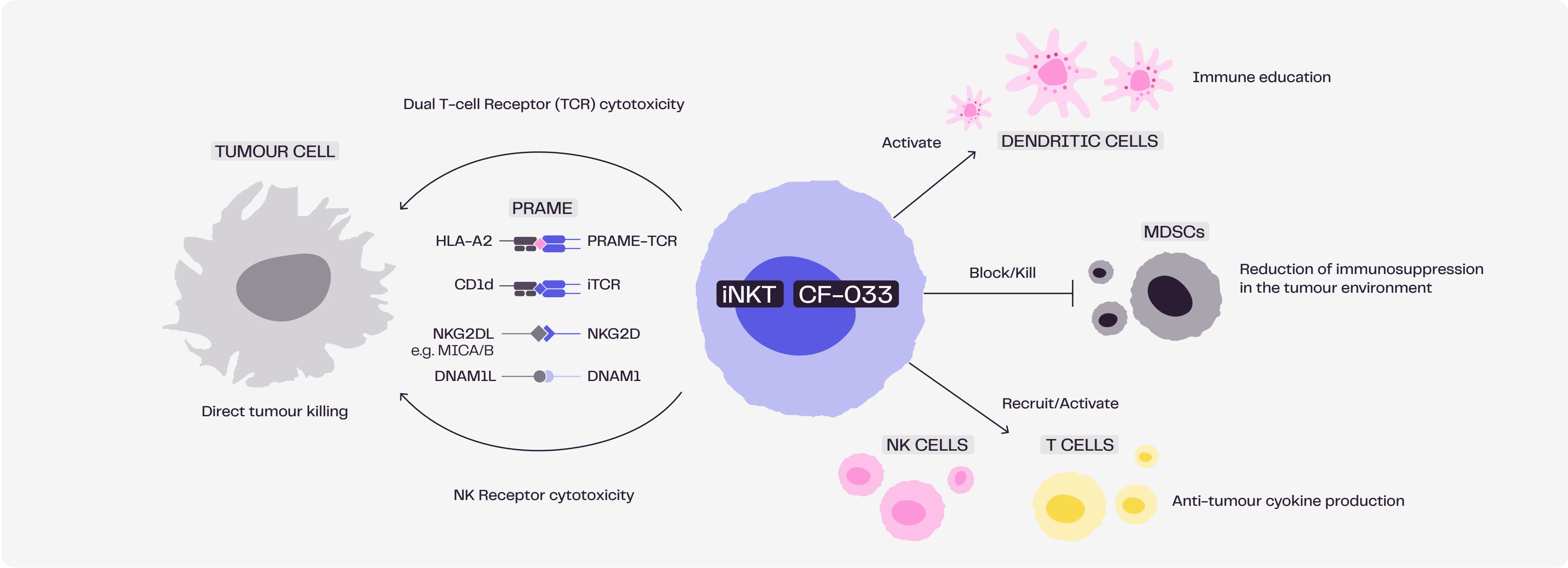
CF-033 sits where three strengths meet: MiNK’s iNKT discovery and clinical experience, Southampton’s expertise in cellular therapies and advanced preclinical models, and C-Further’s translational leadership and network to take it to paediatric patients.

Claire Reader, PhD,
Principal Scientist, LifeArc





The proposed mechanism of action



Why it matters

Children with relapsed or treatment-resistant cancers often have limited therapeutic options. By combining a broadly relevant cancer target such as PRAME with an off-the-shelf iNKT cell therapy platform, project CF-033 aims to address some of the key scientific and practical barriers that have limited access to advanced cell therapies in children and young people’s cancer.

The ability to deliver therapy without the delays, variability and logistical complexity of autologous manufacturing directly addresses a critical limitation in paediatric care, where time to treatment is often decisive. When combined with the favourable safety profile observed to date, including the absence of graft-versus-host disease and the potential to avoid lymphodepletion, this begins to define a modality that is not only biologically differentiated, but also practical and scalable for children.

Clinical validation

In August 2026, a case report from teams at KiTZ Heidelberg, NCT Heidelberg and Immatix described an exceptional clinical response to PRAME-directed T-cell therapy in children with solid tumours, providing validation of PRAME as a paediatric oncology target, which CF-033 pursues through its own, off-the-shelf approach.

N Engl J Med 2026;395:721-724

The programme combines expertise from across academia and industry to advance a novel cell therapy approach for children and young people with high-risk cancers.

Research partner leads



Head of Translational Medicine
MiNK Therapeutics



Associate Professor
University of Southampton

C-Further project lead



Principal Scientist
LifeArc



Embedding lived experience

People affected by children and young people’s cancer hold knowledge no scientific publication, market analysis or development plan can replicate: a direct experience of where the system is failing. That knowledge informs us beyond science; it shapes how we weigh risk against benefit across our decisions and approaches. That is why we embedded patient and family perspectives early within C-Further’s development via our patient advocate panel.

Established shortly after C-Further’s launch, this highly experienced panel operates as an active strategic advisor that guides our thinking and helps shape our portfolio priorities, investment, and translation plans. Embedding lived experience directly into our industry-style model helps ensure capital allocation targets genuine unmet need, not just scientific opportunity.

Our wider advocate community also contributes to reviews and consultations, while helping us to build patient insight into how we prioritise and select projects. Thanks to them, we offer non-profit partners and philanthropists something more than a cause to support: a high-impact, risk-managed vehicle for their capital.

*As of October 2026, Nick has transitioned off the panel.



Keith Desserich,
Chairman & Co-Founder,
The Cure Starts Now
Foundation

Sara Wakeling,
CEO & Co-Founder,
Alice’s Arc

John Rainsbury,
Co-Founder & Chair,
Little Hero Brain Tumour Charity

Nick Bird, MBE,*
Chair of Trustees,
Solving Kids Cancer UK

Ann Graham,
Founder & Executive Director,
MIB Agents Osteosarcoma Alliance

On World Cancer Day,
we held our first in-person
session with C-Further’s
patient advocate panel,
in Brussels.



Science without lived experience operates in the dark; patients without science are left without options. C-Further bridges that divide. Contributing to this panel matters to me because patients and families are not just recipients of cancer care, we are essential partners in informing it. By keeping lived insight central to drug discovery, C-Further ensures that every breakthrough trial and treatment is designed with the true finish line in mind: life-saving, life-changing progress for young people.

Ann Graham,
Patient advocate



The treatment my son went through is essentially the same as I would have at his age. C-Further is creating a genuine pathway for novel paediatric centred treatments. Together, we can start replacing the ‘no’s with ‘yes’s, and create new hope for kids facing the worst that life can throw at them.

John Rainsbury,
Father of Will, patient advocate



Will Rainsbury, diagnosed with a particularly aggressive type of Medulloblastoma, age 5.



Looking ahead: accelerating our impact



Together, we have both a responsibility and an opportunity to create a future where children are treated – by default – with effective, tailored, and well-tolerated medicines.

Lone Friis, PhD,
Co-lead, LifeArc



The past two years, and specifically the last twelve months, gave C-Further a functioning portfolio and the partnerships to sustain it.

In the coming months, our aim is to build a stronger pipeline of child-first therapeutics, announcing new research collaborations as our portfolio broadens. We will also continue working to identify and create the conditions in which those therapeutic opportunities have a viable route to patients. That includes publishing the readout of our strategic market evaluation, which will capture our approach to the commercial frameworks needed to overcome market failure and keep innovations on route to young patients.

Momentum across paediatric oncology is accelerating, but no single entity can solve systemic market failure alone. Lasting change will depend on continued collaboration between researchers, clinicians, patient advocates, funders, industry partners and non-profits.

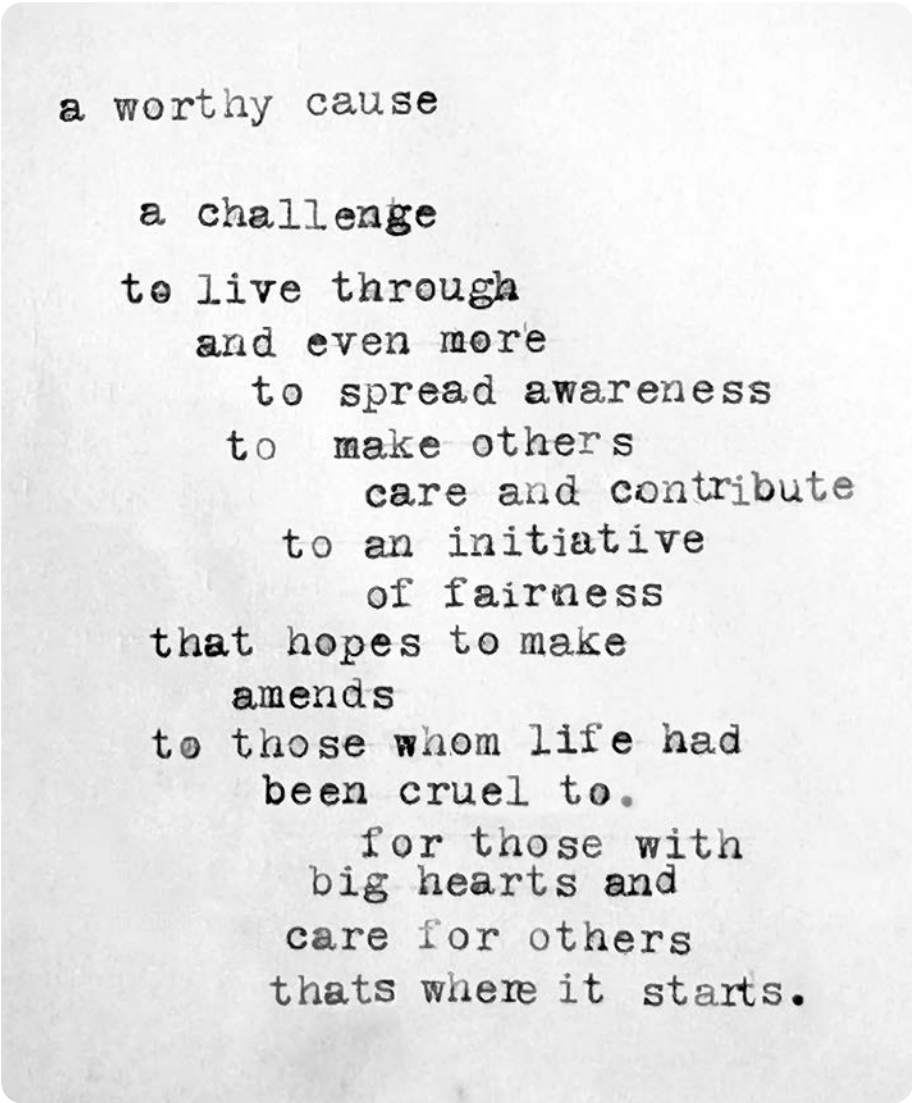
If you share this vision, get in touch with us at

info@c-further.org



This year, they talked about us

Momentum is building around childhood cancer translation, and the coverage on this page shows the world is starting to take notice.

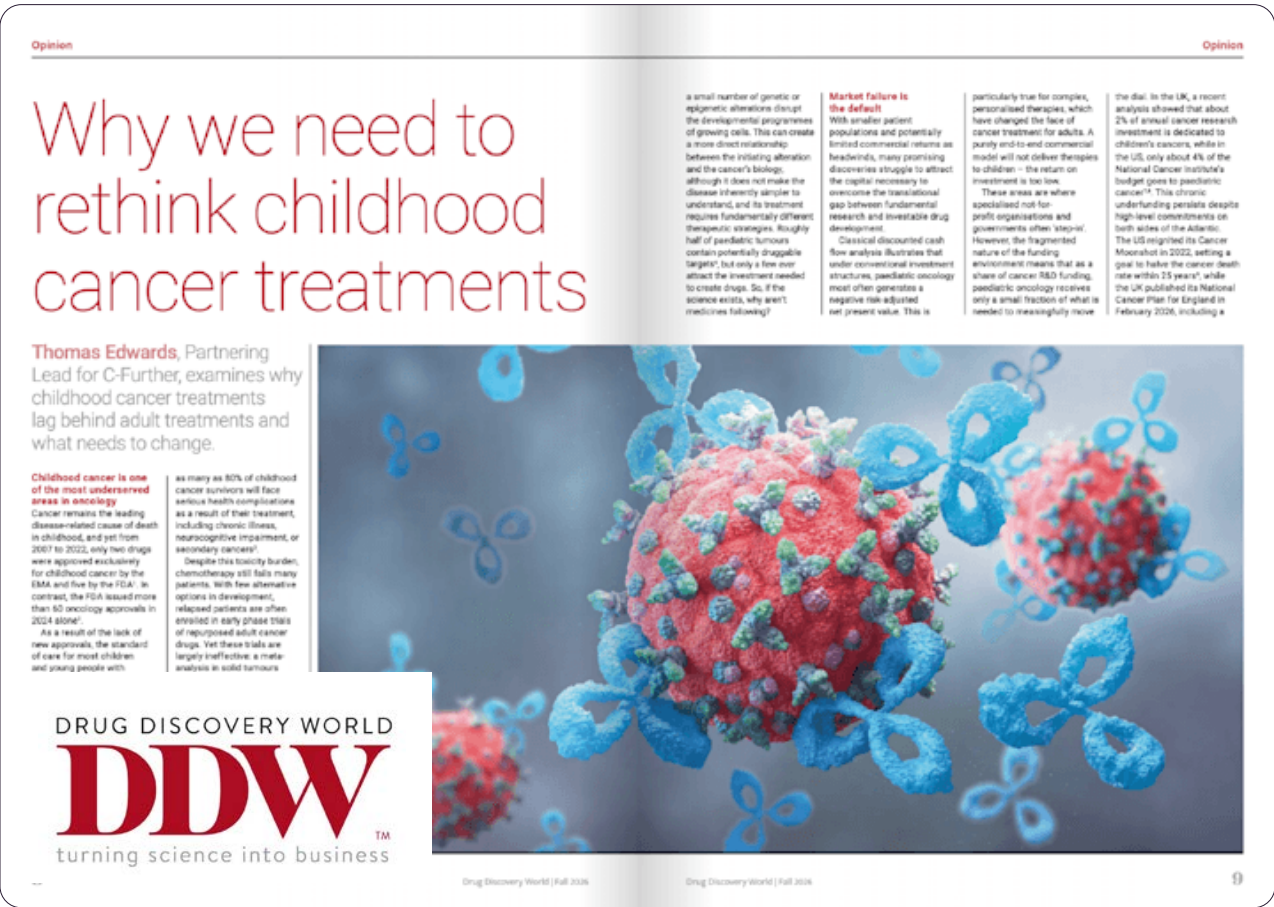


A chance meeting with a street poet in Boston, on the sidelines of AACR's special conference on pediatric cancer, September 2025. This poem is what she made of it.



There's child first and child only and C-Further will do both. But what we won't do is adult only.

Tony Hickson,
Joint Steering Committee at C-Further,
CBO at Cancer Research Horizons



C-FURTHER

We're looking for innovative research, new partners, funders and impact investors, join us:

info@c-further.org

