



**GREAT ORMOND STREET
HOSPITAL CHARITY**

Cancer Research Strategy

2024–2029



**Until no childhood is lost
to cancer**



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Louise Parkes

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Saint-Hallie was diagnosed with retinoblastoma,
aged 18 months



Foreword from our CEO

At Great Ormond Street Hospital Charity (GOSH Charity), we stop at nothing to help give seriously ill children the best care and the best childhood possible. We have built a phenomenal legacy by funding groundbreaking research, and patient and family support, alongside our investment in technology, cutting-edge medical equipment and the building of patient-centred facilities. This to help ensure Great Ormond Street Hospital (GOSH) can deliver life-changing and life-saving care.

Every week, around five children in the UK lose their lives to cancer. That's five families who are faced with the worst possible news. Young patients and their families have to deal with cancer and the profound impact it brings to their lives – disruptions that can rob them of the simple joys of childhood. We believe that no childhood should be lost to illness.

GOSH cares for children from across the UK with the rarest and hardest to treat cancers. Cancer remains the leading cause of death among children aged one to 14 in the UK – a stark reminder of the urgency of our work.

We know that research is the only way to find better treatments and cures. Experts are already pushing boundaries, and pioneering research is underway. We believe that through targeted and dedicated research for children and young people, we can really make a difference. As the nation's largest charitable research funder dedicated to paediatrics, it is our role to help accelerate research and provide hope for a better future.

We are committing at least £15 million to this cancer research strategy over the next five years. Importantly, it is not only those treated in the hospital itself who will benefit. The world-class research that takes place at GOSH, alongside its academic partner the UCL Great Ormond Street Institute of Child Health, helps seriously ill children across the UK and the world.

In addition, GOSH has set out to create a new world-leading Children's Cancer Centre, which the charity is supporting through the Built It. Beat It. campaign. We have pledged to raise £300 million to help build it, but it is only through research and pioneering treatments that we will be able to help beat cancer.

We cannot do this alone, nor do we want to. Working together with others will be crucial to help overcome some of the biggest challenges faced by children and families affected by cancer. We won't stop until no childhood is lost to cancer. We want to give more children the chance to lead happy, healthy lives, thriving into adulthood.

Louise Parkes
Chief Executive, GOSH Charity



Foreword from Alyssa

I'm Alyssa. I live in Leicester with my mum, my dad, my brother and my dog, Holly, who's a Labrador. She's very cute.

In 2021, when I was 12 years old, I was diagnosed with T-cell acute lymphoblastic leukaemia (T-ALL). My treatment started with chemotherapy, but when we found out that wasn't working, I was put on more intensive chemo.

I had been told there was a 95% chance of a cure for my cancer, but when the chemo didn't work as it should, I wondered if I would ever be cured. Next the doctors tried a bone marrow transplant and radiotherapy, which made me really poorly, I had never felt so sick, I could not do anything, I just slept all day. Despite the side effects, the treatment had no effect on my cancer. At that point, I was going to die.

One of the things I remember that I said to my doctor was, "Please don't give up on me." I was thinking, "I can't die yet, I haven't done anything with my life."

Then our doctor found this trial at Great Ormond Street Hospital, led by researchers based at the hospital. It had just opened, and I would be the first person to go on it. My parents gave me the choice, they didn't want to make the decision for me because I had already been through so much. I could either stay at home and live out my last few weeks in peace or go into hospital for the trial. I said I wanted to do the trial, because I thought if it didn't help me, it would help other children. I was scared but also excited to be the first person to do a clinical trial that might save so many lives.

After 28 days I was in remission, and I am cancer free now. I am so grateful for everyone who worked so hard on this trial, as without this, I might not be here today. It's so amazing what research can do. When GOSH Charity told me that finding new treatments is a priority for their new Cancer Research Strategy, I was so happy and hopeful that there will be more research and support for trials like the one I was on.

I spent 16 weeks in GOSH, and there are too many good memories to talk about. As soon as you go in, you are welcomed by everyone. It was a very colourful place, a very cheerful place, not like you think of a hospital to be. It seemed like the nurses and support staff weren't just there to treat us; they were there to be our friends as well. They cared about me, as a person, so much. Learning about GOSH Charity's new Cancer Research Strategy and its focus on improving the experience for children and families is so important and will make the world of difference to families like mine, who may at times feel anxious, scared and alone.

Two years on from treatment, a lot of people think everything has gone back to normal, whereas nothing will go back to normal. People are left with physical and mental effects afterwards. It's not the end when your treatment has finished, it's still very hard. The illness has definitely changed me as a person. I am proud of what I have been through, and it is not something that I will be able to forget. I can look back and say, "Wow, I was able to do this."

In the future, my hopes for the treatment of childhood cancers are that there are more research trials. That is really important, not just for my diagnosis, but also for other people with other cancers. We need people to try new ideas and treatments, without this so many lives would be lost.

My hopes for me, now I'm cancer free, are that I get to live a normal life, grow up, be a mum, a wife and grandmother. I am excited to finish school and get a job, hopefully in the NHS.



Our cancer research strategy at a glance

Vision	Until no childhood is lost to cancer		
Mission	Transform the outcomes and experiences of children and young people with the rarest and hardest to treat cancers		
Priorities	Develop new treatments and cures that are more effective and targeted		
	Improve the experiences and quality of life of patients and their families through research		
	Rapidly translate research discoveries into the NHS clinic		
To achieve our aims, we need a strong research culture			
Our funding principles	Equity and fairness	Partnerships and collaboration	Funding highest quality research for biggest impact

How we built our cancer research strategy

We developed this strategy over 18 months with extensive consultations across the cancer community, including:

- Hospital staff and researchers at GOSH and our partner organisations
- Children's cancer experts across the UK
- International children's cancer centres and hospitals
- Research funders and charities
- Patients and families

This has helped us identify the unique role GOSH Charity can play in cancer research for children, young people and their families.

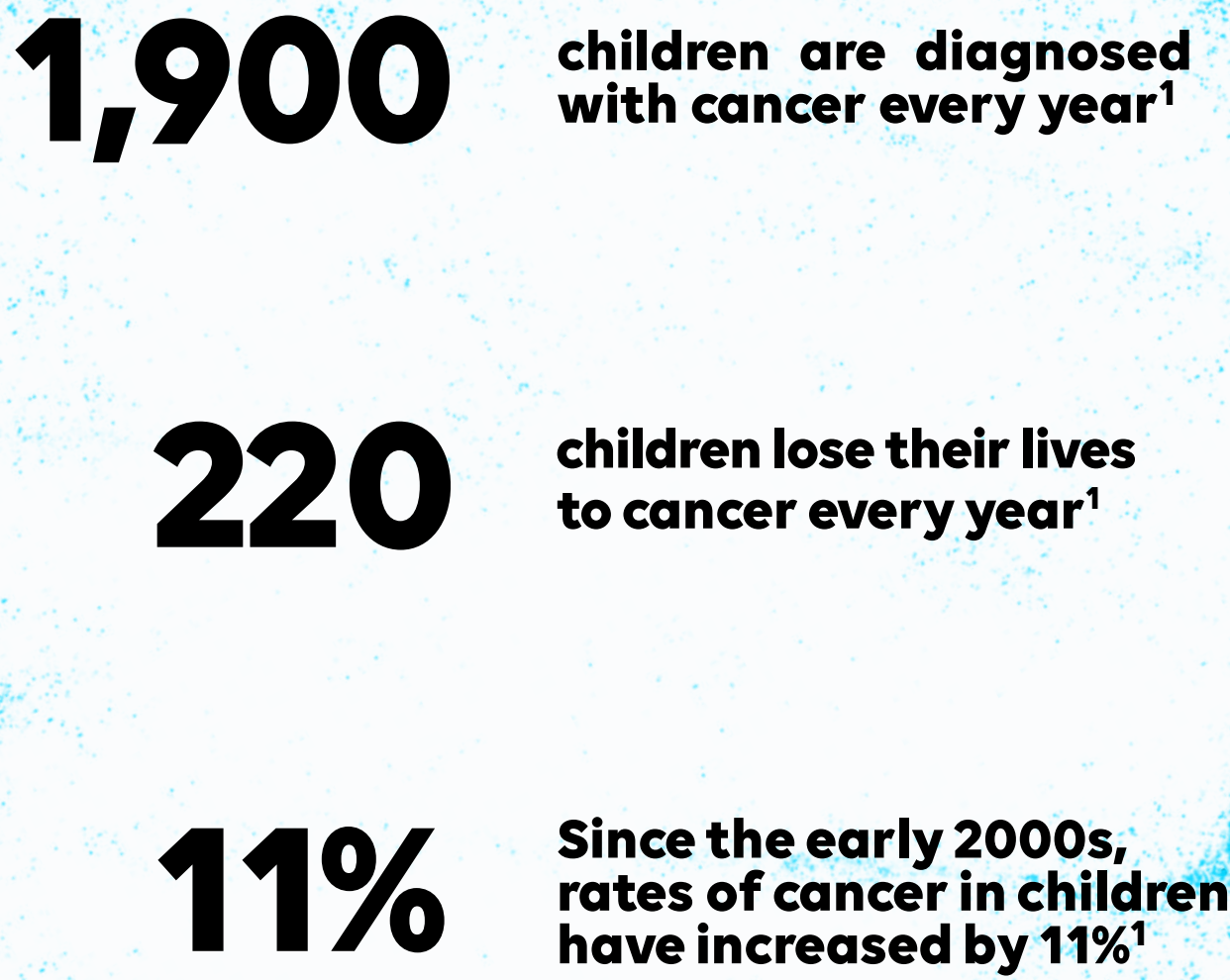
We also aligned our strategy to the James Lind Alliance Children's Cancer Top 10 Priorities. These are questions in most need of answering, according to children, their families and the professionals that care for them. This work was funded by the Children's Cancer and Leukaemia Group (CCLG) and The Little Princess Trust (LPT).



"Children's Cancer and Leukaemia Group and The Little Princess Trust are proud to have jointly funded the development of the research priorities in children's cancer report. We are happy to see that GOSH Charity's first cancer research strategy has taken into account these priorities as well as insights from many charities and funders in this space. It is only through collective effort, commitment and investment into childhood cancer research that we will shape a better and brighter future for young patients and families."

Ashley Ball-Gamble, CEO of CCLG,
Phil Brace, CEO of LPT and Wendy Tarplee-Morris,
LTP Co-Founder and Director of Service and Impact

In the UK:



¹ Source: cancerresearchuk.org/health-professional/cancer-statistics/childrens-cancers#heading
CRUK Children Statistics

Cancer in children and young people

Cancer is rare in children and young people, compared to cancer in adults. However, there are still around 1,800 new cancer cases in children in the UK every year – that's around five every day.¹

"My son died because of the lack of money available into research, as his is deemed a rare cancer. Obviously to us as a family and ultimately my son, that is not good enough. It's only rare until it happens to you."

Parent who took part in our consultation

Thanks to research and better treatments, survival rates have improved. In the 1970s, four out of 10 children diagnosed with cancer in the UK survived beyond five years, now it's more than eight in ten.¹

However, there are disparities in survival rates for different types of cancer in children and young people, with some types showing little or no improvement in survival. For example, with some types of brain tumour, five-year survival rates are as low as 20%, compared to retinoblastoma, a rare type of eye cancer, which has almost 100% five-year survival.¹

More research is needed to improve survival rates, especially for children with the rarest or hard to treat cancers.

"A cancer diagnosis is devastating. Even for those children who survive the disease, their childhood will be interrupted by treatment and monitoring, and they may also suffer with long-term impacts of treatment. Over the last fifty years, research has saved the lives of many children and young people. Only with more research can we help those children who are diagnosed with rare or hard-to-treat cancers, and only with research can we improve the quality of life for children who survive cancer."



Pamela Kearns
Chair of GOSH Charity's Research Assessment Panel

Even when children and young people are successfully treated for cancer, most will live with long-term health problems.

We need to research to make treatments more effective and targeted to give children a better quality of life and to reduce long-term side-effects.

"We have had to help families and patients understand the long-term effects of their treatments. They've had to have conversations about future fertility, increased risk of osteoporosis, and work through the mental health side effects of their experiences, potentially of intensive care, at such a young age."

"The dial has shifted from treatment at all costs, to a more balanced approach between treatment and quality of life. Targeted modernised treatments, like CAR-T, will pave the way to achieving this."



Mary Foo Caballero
Matron at GOSH

¹ Source: cancerresearchuk.org/health-professional/cancer-statistics/childrens-cancers#heading
CRUK Children Statistics

Compared to adult cancers, research on children's cancers has not progressed quickly enough and has been hampered by lower levels of funding.

Only 12 anti-cancer medicines

In the last ten years, only 12 anti-cancer medicines have been authorised for a specific paediatric cancer indication, compared to over 150 for adult cancers.²

6.5 years longer

When compared to adult studies, clinical trials testing new treatments in children take 6.5 years longer on average to reach approval stage.³

22 years after

For some medicines, approval for use in children has only been awarded 22 years after its approval for adults.⁴

² Source: European Society for Paediatric Oncology
³ Source: European Journal of Cancer
⁴ Source: World Health Organisation

Our mission and priorities

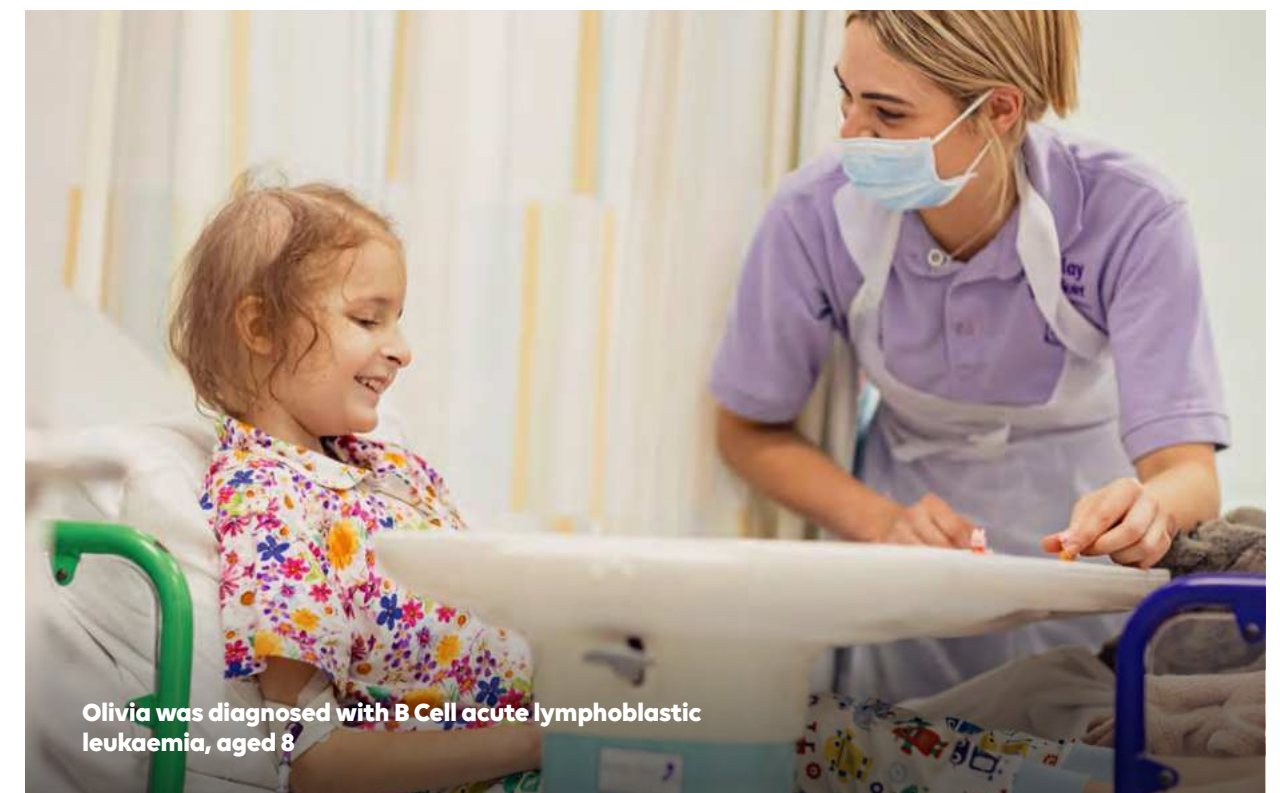
Our vision

Until no childhood is lost to cancer

Our mission

Transform the outcomes and experiences of children and young people with the rarest and hardest to treat cancers

We are committing at least £15 million to this cancer research strategy over the next five years. Our approach is to fund strategically across three inter-related priorities, that will have the greatest impact for children, young people and families affected by cancer.



Olivia was diagnosed with B Cell acute lymphoblastic leukaemia, aged 8

Oliver was diagnosed with Burkitt lymphoma, aged 6

Our research priorities

1.

Develop new treatments and cures that are more effective and targeted

Our close relationship with GOSH presents unique opportunities. We want to use our resources to speed up the progress of cancer research at GOSH and beyond, so that more effective and targeted treatments can become available on the NHS as quickly as possible. We want to see more progress in the rarest types of cancers, cancers that do not respond to current treatments, cancers that return, cancers that have low survival rates and cancers that have few or no treatment options available – many of these patients are seen at GOSH.

2.

Improve the experiences and quality of life of patients and their families through research

We want to work with the incredible patients and families at GOSH to support research that aims to improve patient and family experience and overall quality of life, during and after treatment.

There are many challenges in conducting research into cancers affecting children and young people, but GOSH Charity is uniquely positioned to help tackle these challenges.

3.

Rapidly translate research discoveries into the NHS clinic



An artist's impression of a patient room in the Children's Cancer Centre at GOSH

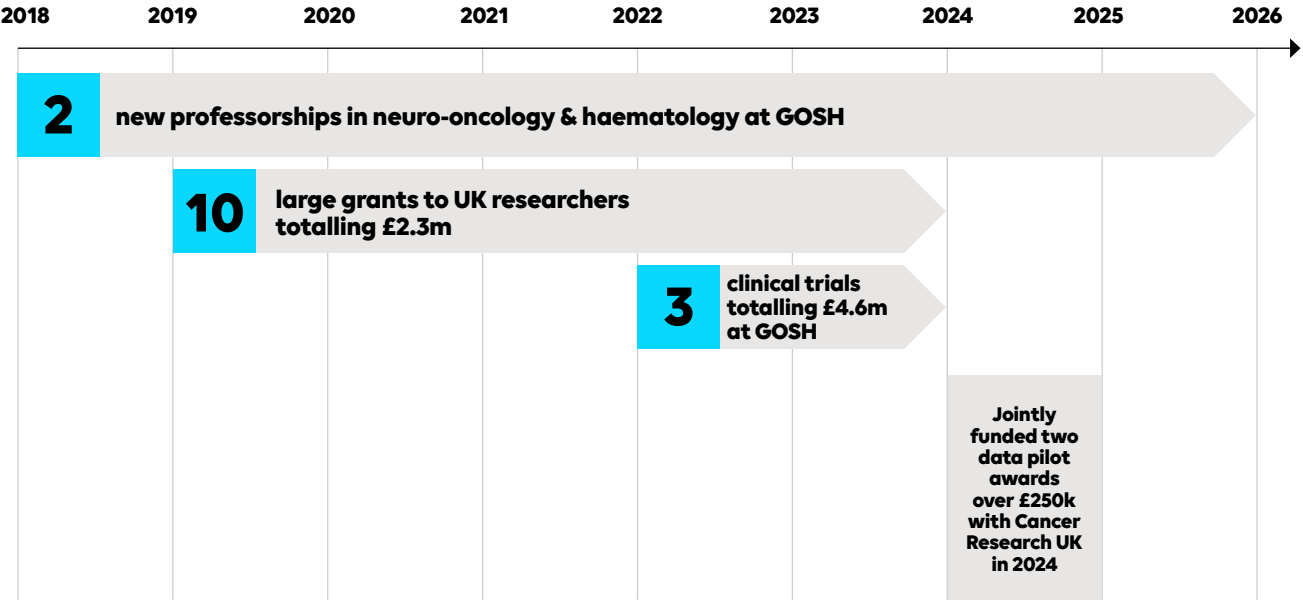
GOSH Charity and cancer

GOSH Charity is the largest charitable funder of medical research dedicated to children's health research in the UK. Alongside this, it provides funding to the hospital above and beyond the day-to-day running costs of the NHS to continue providing extraordinary care, expertise and facilities. GOSH Charity funds cutting-edge equipment, child-centred spaces, and patient, family and staff support.

GOSH, together with University College London Hospital (UCLH), forms one of the largest centres in Europe for children with cancer. GOSH treats cancer patients from across the UK. Many of these children are referred to GOSH because they have a very rare cancer, where little is known about treatment options, or a hard to treat cancer, where survival rates are low, or treatment options are very limited.

Together, the hospital and charity have a strong connection to groundbreaking cancer research.

Over the past five years (2019–2024), GOSH Charity has contributed over £10 million towards cancer research, including:



Children's Cancer Centre

The GOSH Children's Cancer Centre is due to open in 2027 with a £300 million investment from GOSH Charity. It will transform the care that patients and their families receive. It will also enable cutting-edge research that we need to transform outcomes and experiences for children and young people with cancer.

This world-class centre will bring together the different services needed for specialist cancer care. The centre has been designed with children at its heart, reflecting their needs and preferences. Rooms will have enough space for parents and carers to stay and for children to play and do normal activities. It will house a new school for all children at GOSH, ensuring that education will be a part of everyday life and children can easily receive treatment while at school. There will be an accessible roof terrace so children and families can play and relax, and balconies will provide much-needed breakout space for staff and families. We want to use these amazing facilities to improve health outcomes for patients through areas such as physiotherapy and nutrition to help children eat well and be more active during and after treatment.

"The new centre needs to happen, it will be fantastic. Having everything consolidated, alongside a safe green space the children can access when they're vulnerable, will be so important. When things were dark, having a bit of beauty around you, it really helped. It helps you to keep going. Then the research... There is still so much to do. It is not just curing the cancer, it is also making sure it is done in the best possible way [with as few side-effects of the treatment as possible]."

Sandrine Heutz
GOSH mum

The centre will enable:

- 20% increase in capacity offering life-changing and life-saving care for more children.
- More capacity for research giving the hope of better treatments for families faced with few treatment options. We will partner with other research funders to co-fund research in areas of shared strategic priority.
- An increased focus on nutrition, physical activity and wellbeing for children with cancer, supporting their recovery and improving outcomes.
- New imaging technology to reveal what's happening inside cancer cells and improve GOSH's diagnostic capability.



An artist's impression of the Children's Cancer Centre entrance



We want to speed up the pace of research at every stage to provide more treatment options to patients. We want these to be more targeted, to reduce the side effects during and post treatment.

We want to see more trials being run to test new cancer treatments for children and young people. We know that cancer patients who are treated as part of a clinical trial generally have better outcomes.

What we currently do

We already fund discovery and translational research, including cancer research, through our four response-mode funding schemes. We currently fund a number of clinical trials testing targeted therapies. We will continue to do so as well as looking at further support to accelerate the set up and delivery of clinical trials at GOSH. This will mean more children can be offered more treatment options sooner.

Name of scheme	Total annual budget	Aim of scheme
Lift-Off Grants	£800k	Support the essential first step in a new research question
National Call	£2.5 million	UK's largest regular funding scheme dedicated to research into rare or complex childhood disease
Translational Research Accelerator Grant in partnership with LifeArc	£1 million	Supporting bench-to-bedside research at GOSH and UCL GOS Institute of Child Health
Programme Grants	£5 million	Create a step-change in an area of rare and complex childhood disease

Apply for our research funding here:
gosh.org/apply-for-funding/research/research-funding-schemes/

Research Priority 1

We will develop new treatment and cures that are more effective and targeted

CAR-T therapy

Chimeric antigen receptor T-cell (CAR-T) therapies have revolutionized the treatment of certain blood cancers in children and adults. In this treatment, immune cells, called T-cells are taken from the blood and are then altered in the lab so that when they are given back to a patient they can target and destroy cancer cells. This type of treatment is currently available in the NHS for some children with leukaemia.

GOSH Charity has supported GOSH and the UCL Institute of Child Health (UCL GOS ICH) to be at the forefront of developing these treatments for children. As the stories below show, these treatments have the potential to be truly transformative, but challenges remain. Some cancers return after initial treatment, and CAR-T therapy has yet to be shown to be effective in solid tumours. Through this strategy, we will support research that addresses these challenges.

In 2019, GOSH patient Yuvan became the first person to receive Kymriah – a type of CAR-T therapy – in the NHS, when he was just 11 and other treatments for his leukaemia had failed. His parents Sapna and Vinay said: "This therapy was our last hope. We are so glad that we at least had this option. If he had relapsed a year earlier, it would have been a different story." With ongoing weekly treatments at home to boost his immune system, Yuvan remains in remission.

Next generation CAR-T therapies

In 2015, GOSH Charity funded the CARPALL trial where a team led by Professor Persis Amrolia and Dr Sara Ghorashian at GOSH, used CAR-T therapy to treat a type of blood cancer called B-cell acute lymphoblastic leukaemia (B-ALL).

The T cells were taken from patients' own blood and adapted to target a protein called CD19. This had an unprecedented response in some children with otherwise incurable B-ALL.

Although CAR-T therapy has revolutionised treatment for B-ALL, some patients still relapse, so more research is urgently needed. Professor Amrolia's team discovered that the main causes of relapse are that cancer cells stop expressing CD19 and so avoid detection by CAR-T cells, or the CAR-T cells do not last long enough in the body to fully eradicate all cancer cells.

Now, thanks to Cris Cancer Foundation, we can support a continuation of the CARPALL trial to address these issues. The research team are evaluating a new CAR-T therapy where CAR T-cells are designed to have an extended lifespan and can recognise multiple protein targets on cancer cells. They plan to treat several patients with relapsed B-ALL with these next-generation CAR-T-cells. They hope the treatment will further improve the survival rates and ultimately mean more children can be treated with CAR-T therapy rather than more toxic treatments.



"This therapy was our last hope. We are so glad that we at least had this option. If he had relapsed a year earlier, it would have been a different story."



Yuvan became the first person to receive Kymriah – a type of CAR-T therapy on the NHS, aged 11



Austin's story

Austin was initially diagnosed with ALL aged two. By the age of eight he had dealt with four relapses and exhausted all traditional treatment options including radiotherapy, chemotherapy and a bone marrow transplant.

Austin's dad, Scott, says: "When Austin relapsed for a fourth time, our world came crashing down. We thought we had exhausted every last option. Then we found out there was a trial available for Austin at GOSH and had a life-changing meeting with Persis Amrolia. I remember that day so clearly. He was talking about potential hope, and suddenly we could see a future both for Austin and also for medicine. The CD19 trial would offer a completely different form of treatment.

"The side effects from the first four years of Austin's treatment were incredibly tough; they caused Austin significant long-term damage and led to stints in intensive care from associated infections. In comparison, CAR-T therapy has had the least side effects of all his treatments. Thanks to advances in ground-breaking research, this targeted approach to fighting cancer tipped the balance in our favour when conventional treatment failed.

"Prioritising fresh approaches to fighting cancer will be the key to improving patient survival rates. The disease is brutal, and it damages so many aspects of life that we take for granted. The damaging strain of undertaking prolonged and invasive treatment regimens takes its toll on all family members over such prolonged periods. So our experience of witnessing the least intrusive treatment achieve the result we feared impossible, gave a crystal-clear appreciation of the value that such complex research studies provide.

"Austin is now more than seven years post treatment and the first child in the UK to meet this milestone. We are so grateful to Persis and the team who have given Austin another chance of life. Our wish is that immunotherapy continues to develop, offering more children with cancer new hope."

"Our experience of witnessing the least intrusive treatment achieve the result we feared impossible, gave a crystal-clear appreciation of the value that such complex research studies provide."

Targeting aggressive leukaemias

T-cell acute lymphoblastic leukaemia (T-ALL) is an aggressive form of blood cancer. Conventional treatments for T-ALL cause serious side effects for children in the short- and long-term, and they are not always effective. There is currently no standard of care for children whose T-ALL returns or is resistant to treatment, and so long-term survival for these children can be as low as 30-50%.

So far, there are no CAR-T therapies licensed for treating T-ALL, in part because both healthy and cancerous T-cells could be damaged by this type of treatment. However, researchers at UCL, led by Dr Paul Maciocia and Professor Marc Mansour, have recently identified a protein found specifically on cancerous T-cells, called CCR9, which could be a target for CAR-T therapy for T-ALL.

Excitingly, the team at GOSH and UCL GOS ICH, led by Dr Sara Ghorashian, have secured funding from GOSH Charity to bring this therapy to children with T-ALL. Dr Ghorashian and her team will run a clinical trial for 12 children with T-ALL to test the effectiveness of CAR-T therapy directed specifically at CCR9.

The study will run alongside a trial already being implemented in adults meaning researchers can combine data from both studies, saving time and money. This format of trial could initiate an entirely new approach in clinical research, where paediatric and adult patients are treated in a single early phase study as a matter of course, rather than separately. Running joint trials should make it possible to define the safety and correct doses of a new treatment for children and adults, with fewer patients needed overall. If the trials are successful, this should ensure both children and adults with T-ALL will get access to the new CAR-T therapy in the NHS as soon as possible.

Dr Sara Ghorashian says: "Funding is so competitive that cost saving in delivery of complex and early phase clinical studies such as FRACTALL will mean that it is more likely joint adult-paediatric studies can be funded in the future.

"This means that therapies that may be equally effective in children as in adults will be more readily available to the former without a need to run a dedicated paediatric study, improving access to novel therapies for children."

Applying the science to solid tumours

Solid tumours have so far proved much harder to target with CAR-T therapy than blood cancers. This is partly because CAR-T cells seem to find it more difficult to distinguish solid tumour cells from healthy cells and partly because CAR-T cells can become exhausted trying to reach all the cells in a solid tumour.

With funding from GOSH Charity and self-funded medical research charity LifeArc, Professor John Anderson and his team at UCL are attempting to adapt CAR-T therapy to target high-risk solid tumours in children, including brain tumours. They are using drugs called immunomodulatory drugs (IMiDs) that act on the immune system to control CAR-T cells. They hope to use these drugs to switch the CAR-T cells on and off within the patient's body. This will allow the CAR-T cells to rest and recover, then re-activate so they can target and destroy more tumour cells without becoming exhausted.

Another team at UCL, led by Dr Karin Straathof and Professor Darren Hargrave, are trialling a new type of CAR-T therapy for children with an aggressive type of brain tumour called diffuse midline glioma (DMG). DMG develops in important parts of the brain that control bodily function, so removing the cancer via surgery is impossible. Most children with DMG die within two years of diagnosis. With a £1.2 million grant from a partnership including GOSH Charity, The Lyla Nsouli Foundation for Children's Brain Cancer Research, Funding Neuro, Abbie's Army, CRIS Cancer Foundation and The Azaylia Foundation, the team will use CAR T-cells in a trial for up to 12 patients with DMG at GOSH. The CAR T-cells will be adapted to target a protein found on the surface of DMG cancer cells. This work could be a crucial first step in developing effective CAR T-cell treatments for DMG and other high-risk brain tumours.

Dr Karin Straathof, who is leading the research, said:

"DMG is the most difficult and challenging childhood cancer we face, and can be devastating for families. We need novel approaches to tackle it, and we hope this trial – the first in the UK – will bring us one step closer to developing an effective treatment."

"With personal experiences of our own, we are really proud to be coming together to fund much-needed research into one of the most devastating childhood cancers. DMG has a median survival rate of less than a year, with only very limited treatment options. The speed of this disease is devastating for families to witness and come to terms with. We need to change this, and can only do so through trials like CARMIGO. We are glad to see GOSH Charity will continue to promote and support vital research like this through their new Cancer Research Strategy."

The Lyla Nsouli Foundation for Children's Brain Cancer Research, Funding Neuro, Abbie's Army, CRIS Cancer Foundation and the Azaylia Foundation



What more we will do

- We will invest in staff and infrastructure at GOSH to speed up the time it takes to set up clinical trials and to increase the number of clinical trials that can be supported.
- We will invest in areas of existing strength in clinical research such as next generation CAR-T and other therapies for rare and hard to treat cancers.
- We will look at how we can partner with industry and international forums to help accelerate the development of new treatments for cancers in children and young people, especially rare cancers.
- We will partner with other research funders to co-fund research in areas of shared strategic priority.

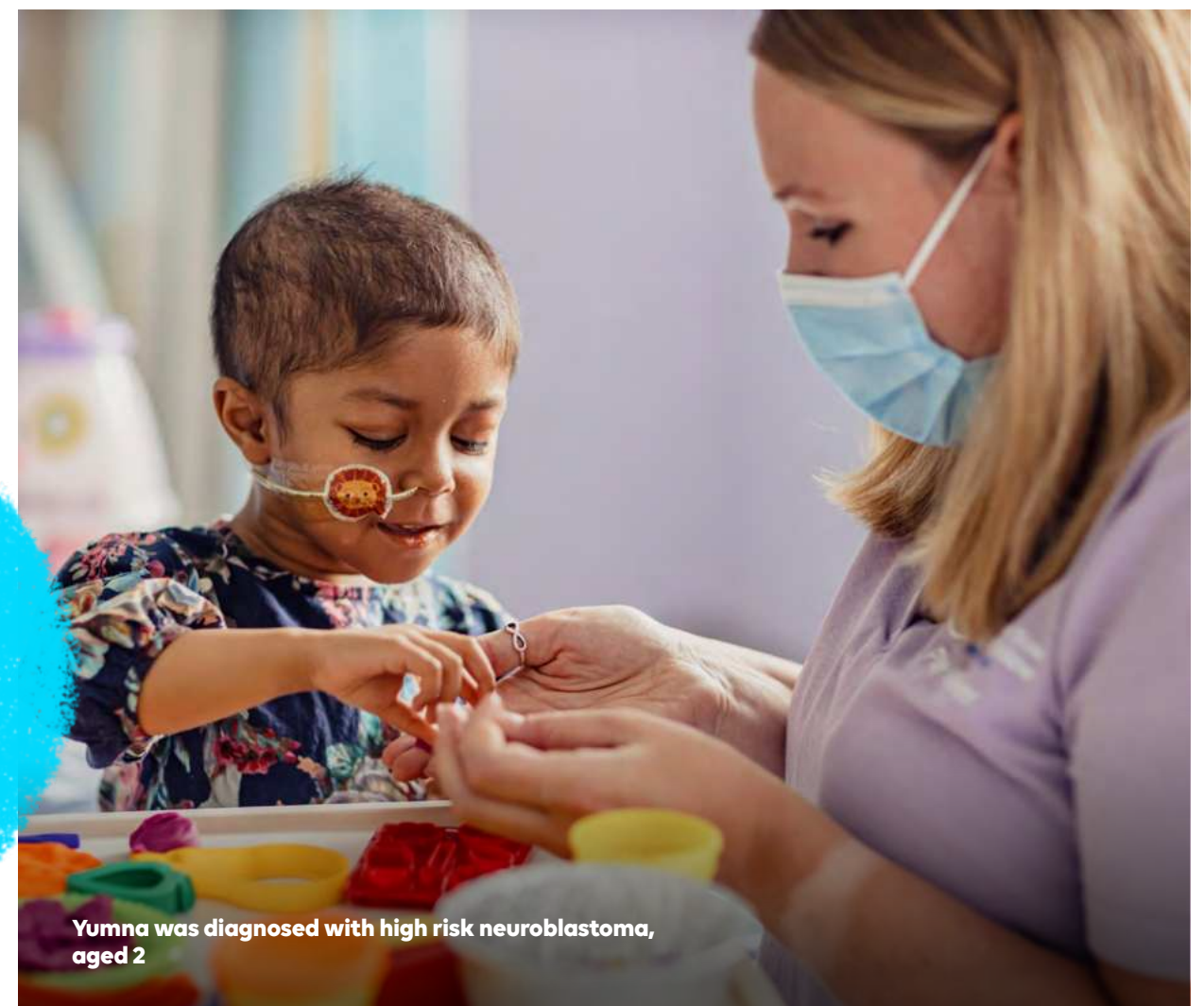
Ilan was diagnosed with acute
myeloid leukaemia, aged 11

Living with cancer and spending time in hospital can have a deep and long-lasting impact on patients and their families. It's important that children's individual personalities, needs and interests are considered, and children don't feel defined by their condition.

We want to explore supporting research that helps patients enjoy their childhood, enhances the hospital experiences for them and their families, and improves their overall quality of life.

Research Priority 2

We will improve the experiences and quality of life of patients and their families through research



Yumna was diagnosed with high risk neuroblastoma,
aged 2

What we currently do

GOSH Charity provides essential support for services that aim to improve patient and family experience whilst being under the care of GOSH, such as play, music therapy, arts and entertainment programmes, family accommodation, a multifaith chaplaincy and family support services. We are also exploring how holistic patient care, such as physiotherapy and nutrition, can improve hospital experience and health outcomes for children being treated for cancer.

We have heard very clearly from families just what a difference these services make to them at a hugely challenging time in their lives. Through this strategy, we want to expand our research remit to include studies designed to optimise the services we support at GOSH, in addition to supporting research that aims to improve the quality of life and long-term impact of cancer on patients and their families. Improving the experience and quality of life for patients and their families is a new area of research focus for us and we believe our close links to the hospital, its patients and families, make us well-placed to fund this research and deliver change.

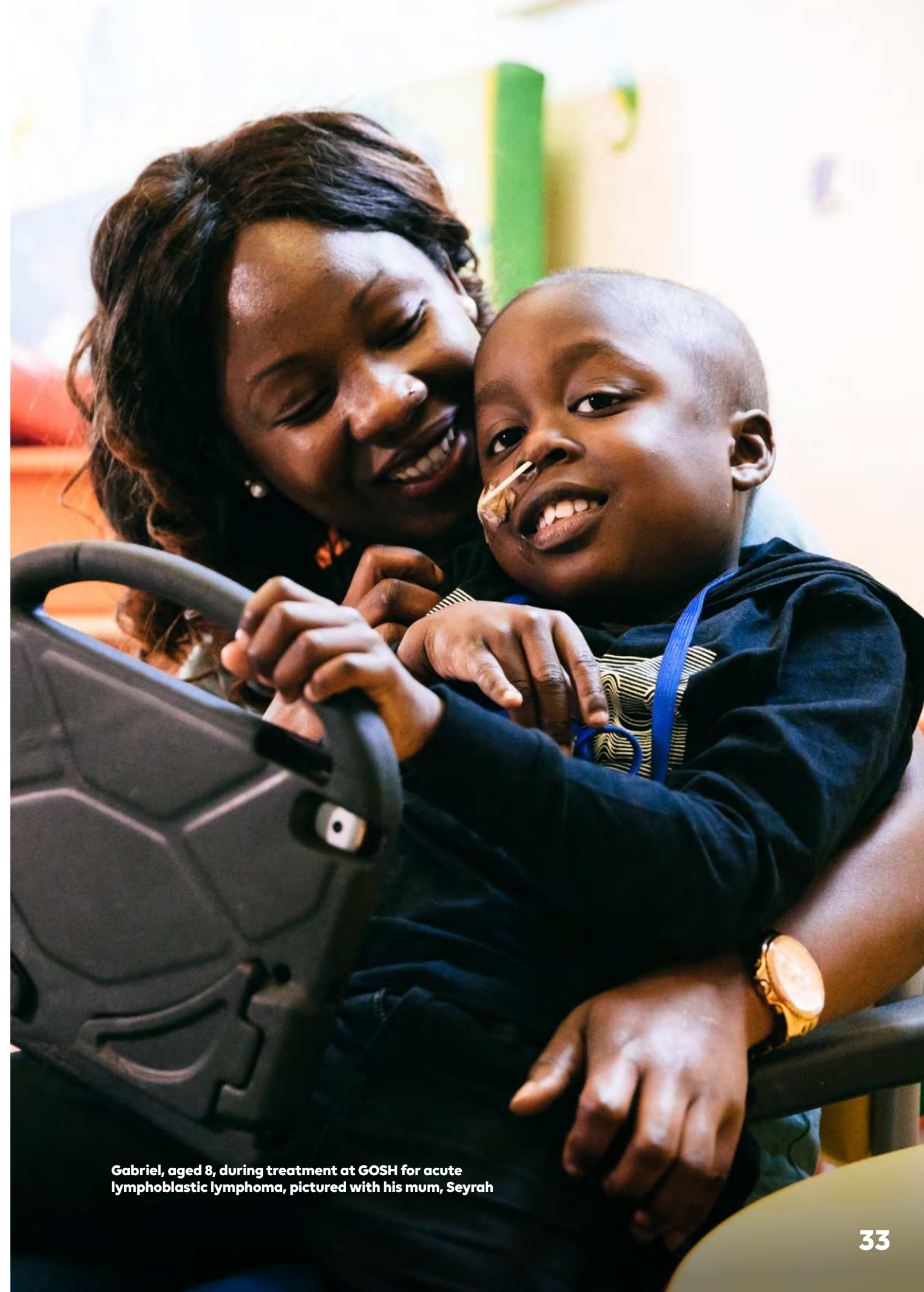
Tailoring cancer care for children with additional needs

The experience of being treated for cancer can be even more challenging for children with learning disabilities or autism spectrum condition and their families. With funding from GOSH Charity, Consultant Nurse, Dr Kate Oulton, is leading a study of how children with autism and/or those with a learning disability experience cancer care and treatment.

Dr Oulton and her team hope their study will pave the way for increased awareness and understanding among clinical staff of what it means for children with additional needs to undergo cancer treatment and lead to better support that is individualised to their needs. This would not only improve the experience of being in hospital for these children and their families but could bring improvements to their health and well-being in the longer term.

Gabriel, who has autism and ADHD, was diagnosed with acute lymphoblastic lymphoma when he was eight years old. His mum, Seyrah said: "GOSH gave us hope, nothing was too challenging for them, they adapted Gabriel's treatment in response to behavioural changes, and ensured we had a quiet space ahead of procedures as due to Gabriel's autism, he does not like loud noises like crying.

"All the changes they made to accommodate Gabriel really eased our fears and helped Gabriel cope better with his anxieties. Also, the play specialist was always there to sing, play and cuddle Gabriel when he was scared of treatment or when the waiting time was too long, and Gabriel would get dysregulated. They even brought a music therapist on the ward to make Gabriel's stay at the hospital easier, as he loves music. All these really gave us a positive experience on the ward. It wasn't just about chemotherapy treatments, but an experience of a bit of normality."



Gabriel, aged 8, during treatment at GOSH for acute lymphoblastic lymphoma, pictured with his mum, Seyrah

The Play team

GOSH Charity funds a team of 52 play workers and specialists who help children feel comfortable and relaxed, while preparing them for life-saving treatment and surgery. In 2023/24 the Play team helped reduce anxiety for around 6,500 patients and provided emotional support to around 900 patients. We will explore how we can develop new routes for research funding to gather evidence on how services, such as play, improve experiences for children with cancer and their families at GOSH and impact health outcomes. Craig had a kidney transplant at GOSH when he was eight years old as part of his treatment for Wilms tumour.

Craig’s mum, Lisa, said:

“His recovery was supported by play specialists. It makes things easier as a parent when you’re going through everything and you know there’s someone there to brighten their day. Just the positivity they have around them is amazing and it’s so helpful.”

“There was a playworker who did these parcels, and every time a child hit a post-transplant milestone, they get to open one of these presents. That was a huge incentive to him to want to get up and start doing things like normal.”



Providing mental health support to more children and families

Many children and young people with cancer and their families experience difficulties with their mental health and wellbeing.

Psychologist, Dr Sophie Bennett believes that more children and families could be helped by offering ‘low intensity interventions’ delivered by specially trained children’s wellbeing practitioners. This means offering children the most effective, but least intrusive, time-consuming and expensive interventions.

Thanks to GOSH Charity funding, Dr Bennett has trialled these interventions at GOSH. This work has highlighted the need for further exploration and development of low intensity psychosocial interventions that would be suitable for cancer patients, as well as the need for further understanding of which patient groups are most likely to benefit.

Helping children to stay active during treatment

Research shows that increasing physical activity is one of the most effective ways of reducing treatment side effects and improving physical and psychological wellbeing during treatment and beyond.

Lucy Waller, Clinical Specialist Physiotherapist at GOSH is carrying out research with patients, their families and their clinical teams to help understand what is needed to enable children to be physically active while in hospital and during survivorship.

She hopes the findings will provide the evidence required to embed physical activity into the daily routine of children and young people during and after cancer treatment and ultimately bring improvements to children’s wellbeing in the long term.

What more we will do

- We will identify opportunities in our Patient and Family Experience Strategy that will benefit cancer patients.
- We will explore how we can expand our research funding schemes to better understand how patient and family support services and holistic care improve experiences and health outcomes, including for children with cancer.
- We will continue to increase our research funding towards projects with a focus on improving the quality of life for children with cancer, including those experiencing late effects of cancer treatment.
- We will work with national research funding partners to ensure our research supports the priorities identified by patients and families through the James Lind Alliance Children’s Cancer priorities shared strategic priority.

A photograph of a man with a beard and a young boy looking at a small object together. The man is on the left, wearing a dark shirt, and the boy is on the right, wearing a red hoodie. They are both smiling and looking down at the object. The background is a blurred indoor setting.

Umar was diagnosed with
high risk neuroblastoma, aged 3

Research Priority 3

**We will rapidly
translate research
into the NHS**

We know that currently, research into rare diseases, like childhood cancer, takes far too long from initial discoveries in the laboratory to reaching a child's bedside and making an impact. GOSH Charity's partnership with GOSH and the UCL GOS ICH creates a unique environment for research, and we want to use this strength to support research to progress as quickly and efficiently as possible to ensure new discoveries reach patients without delay. This includes clinical research findings as well as more holistic research, to shape research-led care. Once breakthroughs become available on the NHS, it means many more patients can benefit.

Below are some great examples of how research has led to changes in practice and care in the NHS.

Clinical trial gives hope for children with rare gliomas

Children diagnosed with rare brain tumours, called BRAF mutated gliomas, can now be treated with a new targeted treatment in the NHS, thanks to research led by a GOSH consultant.

Professor Darren Hargrave led the trial, involving researchers from around the world, which compared a combination of targeted cancer drugs called dabrafenib and trametinib, with standard chemotherapy. The research showed that this treatment, targeted at the BRAF mutation, improved children's survival and had milder side effects, compared to chemotherapy.

As a result, the treatment has now been approved by the National Institute for Health and Care Excellence for treating children with BRAF mutated gliomas.

Professor Hargrave said:

"It has been incredible to watch research move our ability to treat specific cancers forward at such a quick pace."

GOSH Charity has supported the professorship currently held by Professor Hargrave, which enables him to build and lead a research team at the Hospital. Increasing staff research capacity is important to enable breakthroughs like this to take place.

Olivia's story

When she was two, Olivia was diagnosed with a rare brain tumour in a part of the brain where it could not be surgically removed. Despite three courses of chemotherapy, the tumour began to regrow. So, Olivia became the first child to be treated with the targeted drug dabrafenib as part of a trial at GOSH, following promising results in adults. Unlike chemotherapy that has to be given in hospital, dabrafenib is a tablet that can be taken at home.

Her mum Vicki reflects that, "as soon as we started on the new medication, all of the side effects that Olivia had experienced from chemo, that had negatively affected us as a family, had gone. It is so important for research to encompass quality of life into new treatments, and I am glad that GOSH Charity has made this a priority in their Cancer Research Strategy. Olivia has been taking dabrafenib for the past seven years, whilst the trials have progressed to approvals within the UK. In the interim, it is horrible to think new patients have had to undergo gruelling chemotherapy treatment despite kinder treatments being just around the corner. A key aim of GOSH Charity's new cancer strategy is to accelerate the time it takes for discoveries to be available on the NHS. This gives me hope and confidence that more lives of seriously ill children will be improved."



Whole genome sequencing for children and young people with cancer

Advances in technology mean it is easier than ever to analyse a patient's complete set of genetic information and use this information to understand their disease and plan the right treatment. GOSH leads the NHS North Thames Genomic Laboratory Hub – one of only seven hubs in England responsible for providing testing for the Genomic Medicine Service in the NHS.

NHS England is one of the first healthcare services in the world to offer whole genome sequencing to every child diagnosed with cancer. This technique enables doctors to understand all the information in a patient's cancer cell and means that treatment plans can be specifically tailored to each patient.

Recently, researchers at GOSH, the Wellcome Sanger Institute, the University of Cambridge and Cambridge University Hospitals NHS Trust assessed, for the first time, the impact that whole genome sequencing is having on patients and families. They found that, when compared with standard of care testing, whole genome sequencing delivered potential benefits to

children with cancer and even altered treatment in high-risk patient groups. In almost a third of patients, whole genome sequencing provided additional information such as showing which children have a higher risk of cancer in the future and could benefit from regular screening.

Dr Jack Bartram, Consultant Paediatric Haematologist at GOSH, said:

"Our research shows that whole genome sequencing delivers tangible benefits above existing tests, providing better care for our patients. We hope this research really highlights why whole genome sequencing should be delivered as part of routine clinical care to all children with suspected cancer."

Through our new strategy, we want to continue to build on the capabilities of the hospital set up and ensure that leading facilities, such as whole genome sequencing, can be used to drive research forward and provide to patients.



Improving palliative care for children and young people with cancer

Palliative care aims to improve the quality of life for patients and their families who are undergoing treatment and care associated with life-limiting illness. It focuses on relieving suffering through early identification, assessment, and treatment of pain and other issues, whether physical, psychosocial, or spiritual. Palliative care is not only for patients approaching the end of their lives; it is a fundamental part of a patient's treatment plan. This area of research-led care is a key focus for GOSH and GOSH Charity and we are working together to embed research learnings into the NHS, to offer the very best palliative care to children and families within the European Society for Paediatric Oncology.

A centre of excellence in palliative care research

Since its foundation in 2009, The Louis Dundas Centre has become a world leading centre of excellence in research, teaching and clinical practice in palliative care for children and young people at GOSH. The Centre is the vision of Ruth Kennedy and Bruce Dundas, out of the experience of the too short life and death of their son Louis Dundas. Louis was a patient at GOSH who sadly died from a brain tumour in April 2008, aged four.

The Centre has paved the way for advancements in pain management and ensuring children receive timely referrals to a palliative care team nationally through research. It has trialled the NHS England initiative to improve early referral to palliative care for cancer patients by implementing a framework of enhanced supportive care (ESC). Through this pilot, the teams worked to produce definitions of 'poor prognosis', which now enable more children to be referred to the palliative care team. Currently half of patient referrals are cancer patients.

This is the only centre for palliative care for children in the UK and it is instrumental in developing and shaping best practice for children across the UK and worldwide. The Centre shares knowledge and advances globally at conferences and by supporting the Palliative Care group within the European Society for Paediatric Oncology.



Izzie's story

Izzie was diagnosed with a germ cell tumour aged seven in 2018. Germ cell tumours have a 91% success rate of cure; however, in Izzie's case, three rounds of chemotherapy, stem cell replacement, and surgery were sadly not enough. Izzie passed away in 2019, aged eight.

Reflecting on the support provided by the GOSH palliative care team, Kelly, Izzie's mum, said: "They were amazing, they would talk us through any new medications and how they were working, taking onboard how we as a family found them. From the off, I felt really reassured by the team. They focus on medication and pain management; palliative care is not just for people who are dying, but vital throughout treatment.

"Throughout Izzie's treatment, the team were always available over the phone, any time of day. Some of the medication used is so strong, like morphine, I couldn't believe I was giving this to my child. They were able to put my mind at rest, and it made a massive difference."





**To achieve our
aims, we need
a strong research
culture**

We want to create an environment where all research can thrive and is embedded within the NHS. We want to support the research ambition of the Children's Cancer Centre. To do this, as well as deliver on our priorities set out in this strategy, we need to support our workforce, giving them the time, space and resources to carry out important research. We also want to share our research across the UK and around the world, so as many patients and families can benefit.

Below sets out how we're currently investing in our workforce and research infrastructure, and how we will continue to build on this.

Supporting research careers

We fund early career researchers through our partnership with Cancer Research UK (CRUK). We also fund senior leaders in neuro-oncology and haematology through funding their professorships. We are looking forward to increasing our support for mid-career researchers in this strategy.

The CRUK City of London Centre

GOSH Charity has committed to contribute £750k over 4 years towards the Cancer Research UK City of London Centre to fund PhD studentships in children's and young people's cancers. By bringing together researchers from UCL, King's College London, Queen Mary University of London and The Francis Crick Institute, this Centre is working to create new tests and treatments and helping them move from the lab bench into the clinic.

As well as receiving funding, our PhD students have access to a vast range of scientific and transferable skills training via the Centre partners, and they will broaden their experience through placements with different research groups across the Centre.



What more we will do

- We will expand our support for early career researchers, such as through mentoring and training, and support to attend research conferences.
- We will partner with other research funders to co-fund fellowship schemes at the mid-career stage.
- We will investigate how we can encourage and support new people, such as nurses and allied healthcare professionals, to get involved in children's and young people's cancer research.
- We will bring together the whole children's cancer research community through networking activities.

Supporting research careers

We have provided over £1.1 million towards clinical cancer research infrastructure funding from 2017-2024. This has supported clinical trials, general laboratory infrastructure, sample banking and funding staff.

We have also provided over £1 million over the next 3 years to enable the transformation of GOSH cancer services. The priorities for this cancer transformation programme revolve around holistic care, research and treatment and improvements in these areas will be supported by a focus on modernising the workforce, engaging with patients and families, harnessing data and digital innovation and collaborating with partners.

Additionally, we are proud to invest in research projects that maximise the use of data to help better understand cancer and treatments used.



Rajveer was diagnosed with acute lymphoblastic leukaemia, aged 3

Data pilot awards

GOSH Charity jointly funds two data science projects with Cancer Research UK. The Data for Children's and Young People's Cancer Pilot (D4CYP) Award is designed to generate new insights about children's and young people's cancers. The scheme provides seed funding of up to £250k per project over two years.

Understanding infant acute lymphoblastic leukaemia

Infant acute lymphoblastic leukaemia (ALL) – when the child is younger than one – occurs in about 12 to 15 babies in the UK each year. There are currently no national trials for babies with ALL in the UK and a lack of data on their disease, treatment and outcomes.

Through the D4CYP Award scheme, Professor Anindita Roy and Andrew Blake from the University of Oxford and co-investigator Dr Jack Bartram from GOSH hope to create a national database to collect patient information for babies with ALL. This will in turn help to better understand the disease and improve outcomes for patients and families.

She said: "Most leukaemia treatments that adults are now receiving worldwide, were pioneered in children. Childhood cancers are rare, but advances in leukaemia treatments are an exemplar of how paediatric cancer research and clinical trials benefit all."



Professor Anindita Roy
Data for Children's and Young People's Cancer Pilot Award

Understanding how recurrent cancers in children, teenagers and young adults are treated

Unfortunately, not a lot is known about the best way to treat childhood cancers that relapse. There is a lack of data and very few large-scale studies to guide treatment decisions when cancer returns. This means that the treatment of recurrent disease varies across the country.

Dr Martin McCabe and his team at the University of Manchester hope to develop new data links between diagnostic, research and treatment datasets. The team hope to better understand how recurrent cancer in children and young people is treated in the UK, how successful treatment is, what proportion of patients have access to advanced genetic testing, and the impact of this testing on determining future treatment.

They will also be able to see whether certain patient groups are routinely disadvantaged in the treatment they receive based on geography, ethnicity or deprivation.



Dr Martin McCabe
Data for Children's and Young People's Cancer Pilot Award

What more we will do

- We don't know enough about what is hampering progress in paediatric cancer research, particularly in moving promising research through to clinical studies. We will map out clinical research infrastructure to determine where investment is most needed to accelerate research. This is vital to support all three of our research priorities.
- We will continue to support expansion of data and genomic sequencing capabilities.
- We will investigate how we can work more closely with other hospitals and research institutions to share best practice.



Harry was diagnosed with Ewings Sarcoma, aged 6



Our funding principles

As we explore new opportunities and embark on new activities in cancer research, we will be guided by a set of funding principles.

Equity and fairness

We want to address the biggest unmet needs and overcome the challenges currently hampering progress in children's and young people's cancers. To do this, we will be guided by the lived experiences of patients and families.

GOSH has a young people's forum which is a group of current patients and siblings aged 10-21 who have a strong voice in helping to improve patient experience at GOSH.

In addition, the National Institute for Health and Social Care Great Ormond Street Hospital Biomedical Research Centre has a Young People's Advisory Group for research and a GOSH Parent and Carers Research Advisory Group. This is because high-quality research depends on listening to the voices of children and young people, as well as their families and carers, taking account of their experiences, priorities and perspectives.

We want to ensure that the voices of patients and families are at the heart of our work and we are representing their views and experiences. As a Charity, we are striving to embed the patient voice into all of our funding decisions, to ensure all research addresses real patient need.



Funding the highest quality research for the biggest impact

We will fund the highest quality research in order to make the biggest difference to children and young people with cancer and their families. Using our recently developed impact framework, we will monitor the research we support, and ensure the right evidence is identified, collected and analysed to really demonstrate our impact.

Partnership and collaboration

We will work together with other research funders, hospitals, universities and research institutes to build on our strengths and achieve our shared goals.

We are already seeing the impact of such partnerships, like our decision to support the Tessa Jowell Brain Cancer Mission (TJBCM). This group of over 13 organisations is working together to improve brain tumour research, treatment and care in the UK.

After a detailed review process across the UK, supported by the National Institute for Health and Care Research and GOSH Charity, the TJBCM has announced a new network of 15 brain tumour centres for children, of which 6 have been designated as excellent. GOSH and UCLH Joint Centre is one of those centres of excellence, meaning they met the high standards of excellence across all 168 areas along the patient pathway. Areas assessed include clinical care, quality of life care, and access to clinical trials. Special attention was paid to play therapy and education provision, key elements of children's cancer care.

Dr Niky Huskens, CEO of the TJBCM, said:

"The outcome of this rigorous process has been reassuring. Every centre that participated provides good care, with six providing exceptional care. Going forward we will be working with our partners in the charity, public and corporate sectors to provide bespoke centre support and launch new meaningful national initiatives to improve brain tumour treatment and care."



Alyssa and the research team who developed her leukaemia treatment



Conclusion

We have outlined our ambitious cancer research strategy, aimed at transforming the outcomes and experiences of children and young people with the rarest and hardest to treat cancer.

With a commitment to invest in research and partnerships, we envision a future where more children can enjoy their childhood and thrive into adulthood. This strategy represents a bold step forward in paediatric cancer care and research and builds on GOSH Charity's legacy of innovation and a commitment to child health.

Research can be life-changing and life-saving. We have highlighted some incredible breakthroughs already being made and the impact this is having on patients and their families. However, we know that this isn't the case for every child. More research into rare and complex cancers is urgently and desperately needed. We are committed to working together to enable a better future for children and young people with cancer.

Acknowledgement

Thank you to everyone who has contributed to the development of this cancer research strategy, especially patients and their families for sharing their experiences.





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