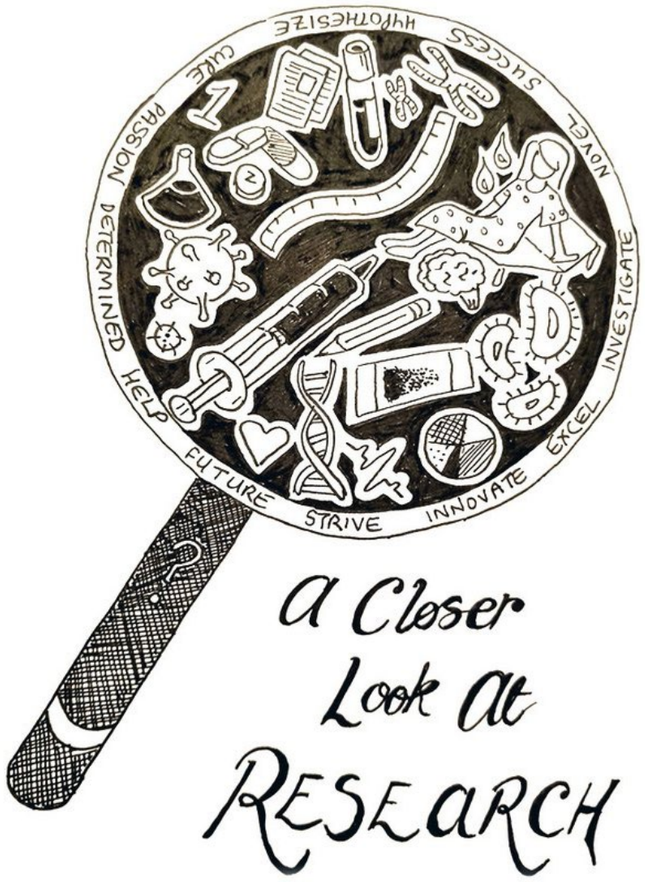
GREAT ORMOND STREET HOSPITAL CHARITY


# Research Strategy 2023–28

## Great Ormond Street Hospital Children's Charity

**Transforming the lives of seriously ill children through research-led care**




**Cover artwork collaboration:**
GOSH senior clinical research nurse, Jade Sugars, and GOSH Charity

**Inspired by the original artwork above,**
GOSH senior clinical research nurse, Jade Sugars

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# Foreword

Many of the children we see at Great Ormond Street Hospital (GOSH) have rare or complex conditions that are life-threatening or life-limiting. These diseases can be difficult to diagnose, treat and live with – and can have a negative impact far beyond the medical symptoms, affecting the quality of life for both patients and families. Many rare diseases still have no treatment, and 5% have only one treatment option. Research is therefore vital to offering new solutions and treatment approaches to patients and their families, while at the same time enhancing their everyday experience so that they can live as fulfilling lives as possible without being constantly restricted and defined by their disease.

At GOSH, we have outstanding people who conduct world-leading research into childhood diseases. These people require the infrastructure to support their work, including state-of-the-art buildings, technology, clinical and non-clinical teams, and adequate funding, as well as support for new ideas. Thanks to the generous contributions of Great Ormond Street Hospital Children's Charity (GOSH Charity) supporters, we're able to invest in these areas to further research and make significant advances in the diagnosis, treatment and care of seriously ill children. The benefits of such investment will extend far beyond GOSH patients, to children and families nationally and around the world living with a rare or complex disease.

We've worked together to set out a new research strategy, with the charity committing £70 million over the next five years. We will build upon the strengths and successes of the previous £50 million strategy and will prioritise:

- Understanding the origins and biology of disease
- Using research to advance towards treatments, tests and cures
- Improving the everyday experience of children living with a rare and complex disease
- Creating an environment where research can thrive

Our research aims are ambitious, and we cannot achieve them alone. Together, GOSH and the UCL Great Ormond Street Institute of Child Health (UCL GOS ICH) form the largest concentration of paediatric research expertise outside of North America and host the only National Institute of Health Research (NIHR) Biomedical Research Centre (BRC) in the UK focusing solely on paediatric research. We have always aligned our strategies, and now we plan an even greater focus on working together so that, collectively, we can identify and capitalise on more opportunities for further research and ensure our combined efforts achieve greater impact. Looking beyond our local campus, we fund research nationally and have close collaborations with other UK hospitals and academic institutions. These partnerships offer an unrivalled opportunity to tackle some of the biggest challenges faced by children and their families with rare or complex diseases and to make a global impact on child health.

GOSH Charity is the UK's largest charity dedicated to funding paediatric research. This, coupled with the unique patient population at GOSH, means that we have a responsibility to fund research into rare or complex diseases. We are committed to accelerating child health research and transforming the lives of seriously ill children through research-led care.

**Louise Parkes**
Chief Executive,
GOSH Charity

**Matthew Shaw**
Chief Executive,
GOSH

**Professor Helen Cross**
Director,
UCL GOS ICH

# Parent foreword

Living with a rare or complex disease can have a profound impact on children and their families. Delayed diagnoses, lack of available treatments and long-lasting side effects of current interventions can severely limit a child's ability to lead an ordinary life. There is an urgent need to do more to provide a brighter future for seriously ill children.

Research offers hope. When our son was diagnosed with a rare metabolic disease at 15 months old, we were devastated. Likened to a form of childhood dementia, his condition is progressive and life-limiting. Sadly, there are no approved treatments. We struggled to comprehend how the happy and seemingly healthy toddler in front of us could be given such a terrible prognosis. Research became a lifeline – something we could focus on and strive towards.

With the benefit of an early diagnosis, our son was able to undergo a pioneering gene therapy. The novel treatment that he received has essentially corrected his faulty gene defect. The early results are encouraging not only for our son, but many others with the same and related conditions. Learning from our experience, I have become a passionate advocate for the importance of early diagnosis and the urgent need to accelerate the journey from the 'bench to bedside' for promising therapies. My experience was the motivation for joining GOSH Charity as an Associate Trustee.

The ecosystem created by GOSH Charity's partnerships with the hospital, academic institutions across the UK and other funders was critical in enabling my family to access innovative and groundbreaking treatment. The investment areas set out in the new strategy are key to strengthening these collaborations and expanding the dissemination of knowledge, which is so vital to advancing child health research and providing hope to so many more families at GOSH and beyond.

I am particularly excited to see the new priority area for the charity which aims to improve the everyday experience of children living with a rare or complex disease. Often, the burden of disease is so much more than the medical condition itself. When researchers take a holistic approach and listen to patients and their families, they gain a more in-depth understanding of living day-to-day with a rare or complex disease. This is crucial to ensure that research addresses the issues and problems that are most important to the patients and families affected.

Research can change lives – it changed ours. I'm looking forward to the new strategy helping to accelerate progress in child health research to ensure more children can live as fulfilling lives as possible without being restricted and defined by their disease.

**Louise Sherwin,**
Associate Trustee
GOSH Charity and GOSH parent

# Our strategic framework

Over the next **five years**, we will build on our previous investment by committing over **£70 million** towards **research** into rare and complex childhood diseases and improving the everyday experience for children and their families.

**This framework outlines our approach to accelerating progress in child health research.**

| | |
|---|---|
| **Ambition** | Transform the lives of children through research-led care |
| **Priorities** | Understanding the origins and biology of disease<br>Using research to advance towards treatments, tests and cures<br>Improving the everyday experience of children living with a rare or complex disease<br>Creating an environment where research can thrive |
| **Harnessing the power of** | Genomic medicine<br>Data science<br>Partnerships |
| **Investing in** | Outstanding people<br>Strategic centres<br>National child health priorities<br>Research platforms and infrastructure<br>New ideas |


# Our achievements

## Our previous five-year research strategy resulted in:

- Over £50 million invested in research
- Collaborations with 16 research partners
- Over £16 million leveraged for additional research through partnerships

## Investment included:

- Appointment of five surgical scientists
- Two new research leaders – Professors in Neuro-oncology and Haematology

## The Zayed Centre for Research into Rare Disease in Children

**The doors to the Zayed Centre for Research into Rare Disease in Children, one of Europe's largest research centres focused on rare disease, opened in 2019. The centre, which was made possible thanks to a transformative £60 million gift from Her Highness Sheikha Fatima bint Mubarak, wife of the late Sheikh Zayed bin Sultan Al Nahyan, founding father of the United Arab Emirates, brings together hundreds of clinicians and scientists in a state-of-the-art facility. As well as a laboratory for early development work, the Zayed Centre for Research houses a manufacturing suite that can produce cell and gene therapies, and a child-friendly outpatient clinic. Having clinicians, researchers and patients under one roof is a great help in accelerating the bench-to-bedside progress of new diagnoses, treatments and cures.**

- **There are currently 33 independent research groups led by researchers at the Zayed Centre for Research**
- **Since opening, Zayed Centre for Research researchers have:**
  - Led 30 clinical research projects
  - Published over 200 clinical research papers
  - Trialled 17 new gene and cell therapies

## Thanks in part to our support:

- 48 out of 50 children given gene therapy as part of a trial for a rare and fatal immunodeficiency condition called ADA-SCID had their immune system restored
- More than 100 children have been treated with gene therapy at GOSH
- The use of gene therapy at GOSH has expanded from treating one to now dozens of different diseases
- More than 30 babies have benefited from surgery in the womb
- 1 in 4 patients with an undiagnosed rare disease received a diagnosis for the first time through the 100,000 Genomes Project
- More than 50 patients have been treated with thymus transplants
- 11 new life-changing treatments for children have been approved for use in Europe and the US
- Researchers discovered the cause of the fatal movement disorder dopamine transporter deficiency syndrome (DTDS) and have developed the first potential treatment that shows promise in the lab
- 12 out of 14 children with a previously incurable leukaemia were cleared of their disease after 3 months following treatment with a new type of immunotherapy. 5 of these patients remain leukaemia-free over 2 years later
- GOSH delivered the world's first use of base-edited CAR T-cells to treat resistant leukaemia

Great Ormond Street Hospital Children's Charity

# Elouise's story

Babies born with severe spina bifida have a gap in their spine. They are often unable to walk, control their bowel or bladder and need operations to drain fluid from their brain. Surgery to close the gap can be carried out soon after birth, but this has its limitations.

GOSH Charity, together with University College London Hospital (UCLH) Charity, funded the Centre for Prenatal Therapy, where the first UK prenatal surgeries for spina bifida were performed by a 30-strong team from UCLH and GOSH. The operation involves opening the womb to repair the defect in the spine and then closing the womb without delivering the baby. The surgery has demonstrated great benefits compared with neonatal procedures, including improved mobility and improved chances of a child being able to walk independently.

This innovative procedure has now been carried out in the NHS on more than 30 babies. One of those is Elouise from Burnham-on-Crouch, Essex, who was born in 2019 to mum Beth.

Beth says: "She arrived kicking and screaming, with her bladder working. Considering we were warned that she may not have any movement in her legs and that she may have bladder complications, this was a surprise. And a lovely one!

"Elouise is living proof of what this kind of pioneering surgery can do. As a family, it gave us another choice. We've been incredibly lucky."

Elouise with mum, Beth, and dad, Kieron.

Elouise at birth.

# Our research priorities

**Many of the children who pass through the doors of GOSH have rare or complex conditions that are life-limiting or life-threatening. It is often a place of last hope – and with that comes a huge responsibility to make life-changing differences for the children and their families.**

The hospital has some of the largest groups of patients with rare diseases anywhere in the world, which provides an unparalleled opportunity to study these diseases and to identify and address the needs of the children and families affected. This insight allows us to focus our research funding not just on the illness but on the whole child, their family and their lifelong health.

By dedicating our attention and funding to our four priority areas, we aim to improve care for the children who need it now, as well as delivering better health for the children of the future. It is through discovery research and its translation into pioneering clinical research trials that we will find the cures of tomorrow and help future generations of children.

## 1 Understanding the origins and biology of disease

Discovery research aims to transform our fundamental understanding of disease. By unravelling the origins of disease, researchers can uncover new opportunities to diagnose, treat and even prevent illness. This knowledge is vital to achieving the breakthroughs of tomorrow, for example, discovering new biomarkers for earlier diagnosis or new targets for better treatments and cures.

We will continue to support a broad portfolio of research, including in existing areas of strength (cancer, neuroscience, inflammation and immunity, cardiovascular, endocrine and metabolic, tissue engineering and regenerative medicine) as well as interventions such as gene, stem and cell therapies and surgical innovation.

Discoveries in rare and complex conditions, and the sharing of this knowledge, can then also progress understanding of more prevalent diseases that have a wider medical burden, such as obesity and diabetes. Unlocking this invaluable information could help more children and even adults.

### We will:

- **Expand funding for pioneering discovery research through our existing National Call funding scheme, and launch new discovery research funding schemes**
- **Support discovery research in a broader range of childhood diseases, particularly in underfunded areas**
- **Encourage collaboration and sharing of knowledge through open access publications, and increase our funding of conferences and workshops**

## 2 Using research to advance towards treatments, tests and cures

Discovery science provides new ideas and opportunities for tackling rare or complex diseases in children. We also want to enable the translation of new knowledge about child health into more precise and faster diagnosis, improvements to existing treatments and new precision treatments and cures.

By supporting research from bench to bedside, we will deliver improvements for children in the future, as well as ensuring those being treated right now can benefit from the latest trials, treatments and interventions.

We are in the privileged position of being able to facilitate the translation of discovery research into the clinic by taking advantage of the co-location of the UCL GOS ICH and the hospital. We will continue to promote the collaboration of scientists and clinicians to help save lives and improve patients' health and quality of life.

In the last five years, GOSH researchers have contributed to the development of 11 new life-changing treatments that have been approved for use in Europe and the US. We will continue to fund high-quality research to develop the tests and treatments that give hope of a brighter future for seriously ill children.

### We will:

- **Support the translation of scientific discoveries into clinical research by continuing to fund the Translational Research Accelerator Grants**
- **Launch new translational research and clinical research funding schemes to support the development of new drugs, tests and interventions, and early-stage clinical trials**
- **Launch new patient-focused research funding schemes to facilitate the translation of scientific discoveries into everyday clinical care**

**Rhys, at age 21**

# Rhys' story

Rhys was born in 2000 in Caerphilly, Wales, with severe combined immunodeficiency (SCID), a rare disease that left him vulnerable to even the most minor infection. At the time, the only treatment for patients with SCID was a bone marrow transplant.

"Before Rhys' treatment, he was cut off from the outside world in a very clinical room where he could only be visited under strict supervision. He was so weak he couldn't even hold his head up," Rhys' mum, Marie, says.

In 2001 at GOSH, Rhys became the first child in the UK to receive gene therapy, effectively curing him of his disease. Rhys is now 23 years old and is a healthy young adult in his third year at university.

"I feel that I can live as normal a life as anyone else, the only difference being some daily medication and my weekly antibiotic injections for my immune system," Rhys says.

Since that historic moment at GOSH, more than 100 children have been treated with gene therapy for dozens of different diseases. GOSH Charity has supported a number of researchers to develop gene therapy for SCID, as well as other serious conditions, such as spinal muscular atrophy.

# 3 Improving the everyday experience of children living with a rare or complex disease

Living with a rare or complex disease can have a huge effect on a child's quality of life, as well as wider implications for their family. Many struggle to have their diseases diagnosed and, even following a diagnosis, a cure may not be possible. Of the children who survive, current interventions can have long-lasting and permanent effects into adulthood, and many may require continued support and treatment – limiting their ability to lead an ordinary life.

We understand that other conditions may also be caused by living with disease, for example, issues in children's mental health. Children with chronic health conditions are almost twice as likely to present with a mental health disorder compared with healthy children, and this association only increases with age. Despite this, research on children's mental health has not progressed as rapidly as many areas of children's physical health. Children's and young people's mental health is now a national priority in the UK and a priority for GOSH.

In addition to prioritising research that will improve the everyday experience of children living with a rare or complex disease, we want to advocate for seriously ill children and their families, ensuring their voices are amplified when discussing the issues that most affect them. Advocacy maximises the impact and value of delivering outstanding care and cutting-edge research.

Through specific communication programmes and activities, advocacy provides us with an opportunity to raise awareness, influence decision-making and help improve outcomes on issues of particular interest and benefit to GOSH patients and families, and in the broader child health landscape. Our advocacy platform of 'child health equity' ensures we are focusing on how we can drive and deliver meaningful change around the larger issues impacting children living with serious disease, with the goal of ensuring there is equitable access to good care and research to improve outcomes.

## We will:

- **Launch new, targeted funding schemes for projects that are likely to improve the everyday experience of children living with a rare or complex disease**
- **Increase the amount of funding for research that will improve children's everyday experience and support their healing process**
- **Address issues of most importance and benefit to children and families at GOSH through advocacy**
- **Involve patients and their families in research and advocacy activities to understand what's important to them**

# 4 Creating an environment where research can thrive

Making research part of everyday practice for all clinicians and stakeholders across the hospital leads to better patient outcomes.

We will continue to support GOSH to build on its existing strengths to offer the best research-led care and to flourish as an intelligent research hospital driven by curiosity and innovation. The potential of the existing campus (which includes state-of-the-art research facilities at the Zayed Centre for Research and the NIHR BRC) will be optimised by ensuring researchers have access to cutting-edge equipment, facilities and research platforms and the clinical capacity and infrastructure to deliver their research plans.

We want GOSH to be a place where every research opportunity is maximised, and every patient is part of a research journey. We will work with GOSH to help embed a strong research culture across the hospital. We strongly believe that we can learn something from every patient that will improve diagnoses and treatments in the future.

## We will:

- **Increase access to equipment and expertise by funding infrastructure such as core facilities, digital technology and research platforms**
- **Support 'team science', bringing experts together to address important research questions and challenges through networking events, mentoring and seminars**
- **Expand funding for research at the point of care, so that research outputs can quickly translate to improvements in clinical practice**

"Thanks to research, we've seen major improvements in the lives of children being treated for rare or complex disease – but there remains an urgent need to invest more into child health research. I am delighted that the charity is launching a research strategy that aligns with the needs of the hospital, and national and international research. Together, we can accelerate child health research. I look forward to seeing the difference this strategy will make over the coming years."

— **Dr Kiki Syrad, Director of Research and Innovation at GOSH, Former Director of Impact and Charitable Programmes at GOSH Charity**

# Lucrezia's story

The 100,000 Genomes Project was launched by NHS England in 2014 to sequence and study the role our genes play in health and disease. GOSH formed one of 11 centres set up to recruit patients with genetic disorders, with Professor Dame Lyn Chitty leading this process. GOSH recruited around 20,000 children in total to the project.

Thanks to this study, one in four patients with a previously undiagnosed rare disease received a diagnosis for the first time.

One of these was ten-year-old Lucrezia from Birmingham, who developed difficulties walking when she was around two years old. Despite various tests, Lucrezia had no definite diagnosis.

At GOSH, Lucrezia and her family met Professor Francesco Muntoni, who offered them the chance to join the 100,000 Genomes Project. They discovered Lucrezia had a rare alteration in a gene that was responsible for a problem with her nerves. The study also provided good news for Lucrezia – patients with this condition typically deteriorate slowly and retain their ability to walk.

Lucrezia's mum, Francesca, says: "The mutation was so rare that we wouldn't have been able to discover it without the research.

"People think research today is generating findings that will take 10 years to be developed in medicine, and that can be true. But in this case the research and resulting diagnosis had an immediate effect on our quality of life."

With charity support, Professor Dame Chitty has also pioneered research into rapid and safer prenatal diagnosis. Her services to medicine were recognised in 2022 when she was made Dame Commander of the Order of the British Empire.

**Lucrezia, at age seven**

# We will harness the power of...

**Our research goals are ambitious, but by working in partnership and building on our previous funding successes in patient data and genomic medicine, we can meet the challenge. Together, these three approaches will enable the most important and highest-quality research to thrive. This will ultimately deliver better outcomes for children and their families at GOSH and around the world.**

## Genomic medicine

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. The UK is a world leader in this field, and it is a major strength of GOSH and the UCL GOS ICH.

Our Zayed Centre for Research houses state-of-the-art genomics technology that can analyse patients' genomes to support the development of diagnostic tests and cutting-edge research.

The hospital already provides safe and accurate pre-natal genomic and genetic diagnosis to families throughout England via the NHS and internationally. And, as one of only seven NHS Genomic Laboratory Hubs in England, GOSH leads the North London genomic service that delivers diagnosis, risk assessment and genetic counselling to a population of 4.5 million.

GOSH also recruited around 20,000 children to Genomics England's 100,000 Genomes Project, helping uncover the genetic causes of rare diseases and cancer. As a result, patients are receiving more precise diagnoses and more refined treatments.

## We will:

- **Support researchers to collect, curate and analyse the vast amount of data generated from genomic medicine and link it to patient records, utilising data science tools**
- **Help researchers translate the genomic analysis into clinical practice, including for earlier diagnosis, better monitoring of patients' conditions and new approaches to treatment**

# Data science

Data science is vital for understanding rare and complex diseases. Using data science, researchers will be able to link results from blood and biopsy testing with clinical information, such as which treatments are used and how patients respond. This will make it possible to distinguish patterns in how treatments work for different patients that can be used to guide future research and help tailor treatments to individual children.

We have already made transformative investments in this area by supporting the hospital to make all patient records digital. Implementing the electronic patient record system in 2019 led to GOSH achieving Healthcare Information and Management Systems Society Level 7, the highest attainable international digital maturity benchmark. The hospital was the first trust in the UK to achieve this status, which demonstrates its advanced use of data and analytics to improve patient care. Through this investment in infrastructure, GOSH developed its Digital Research, Informatics and Virtual Environment (DRIVE) unit, which was recently highlighted as an exemplar for effective NHS data partnerships in guidance published by the NHS Transformation Directorate. We continue to support DRIVE through its Clinical Informatics Research Programme, focussing on developing new digital technologies for child health. For example, by conducting, analysing and reporting on interviews with healthcare professionals and co-design workshops with young people being treated for cancer, one project is hoping to improve our understanding of how virtual reality can be implemented in the NHS, and in particular to support young people through cancer treatment.

The NHS securely stores patients' hospital data and enables it to be linked with their GP records. This infrastructure is unique to the NHS and is not in place anywhere else in the world. This, combined with the hospital's unique patient population, means GOSH is world-leading in terms of maximising the use of data to understand and improve treatment and care for children with rare or complex diseases.

## We will:

- **Build on our outstanding digital capabilities and data collection, so that real-world data can inform and improve care, for example by supporting the next generation of data scientists through GOSH's Clinical Informatics Research Programme**
- **Help develop innovative technology, such as artificial intelligence and wearable devices for research and patient care**

# Partnerships

We cannot achieve our ambitious research goals alone. By working in partnership at all levels – local, national and international – we can bring together knowledge, facilities and funding to speed up improvements in diagnosis, treatment and care.

Patients, families and the public are naturally some of our most important partners. We regularly consult with them to ensure their voices are represented in all the charity's work and decisions and encourage all of our funded researchers to do the same.

As the home of the UCL GOS ICH, UCL is one of one of the charity's and GOSH's closest partners, and they also work with other world-class universities, hospitals, and research institutes in London, and around the globe. The close proximity of UCL and GOSH to the Francis Crick Institute and hospitals such as UCLH and Moorfields Eye Hospital, is just one example of partnerships creating a wider community of world-class expertise.

We have already developed strong partnerships with other funders who share our purpose and currently have 16 active partnerships. During our previous five-year research strategy, the partnerships we made resulted in over £16 million in additional funding towards paediatric research. One example is our exciting partnership with LifeArc, which is vital to helping us move research and new technology forward into clinical trials.

## We will:

- **Support our researchers to embed patient and public involvement and engagement in their research plans and delivery**
- **Develop a new approach for patients and their families to be involved in every stage of our research funding and decision making**
- **Maintain our existing research partnerships and develop new ones, both nationally and internationally, to amplify our impact for patients and their families**

# We will invest in...

## Outstanding people

Outstanding research requires outstanding people. We want to retain and recruit world-class researchers and inspirational leaders, and give them the time, funding and support they need to find better treatments for seriously ill children. Over the next five years **we will provide up to £10 million to support outstanding researchers** at GOSH and the UCL GOS ICH.

We want to help build a community of diverse and talented people who can dedicate their research efforts to improving the lives of children with rare or complex health needs. We know there are already many incredible people working towards this aim, but we need to grow and nurture this community.

We also know there are factors that make it harder for talented people to pursue their research, and we will use this knowledge to guide our funding and offer support where it is needed most. For instance, we want clinical staff to be able to carry out research alongside their work caring for patients, but we know this can be difficult. This is a shared challenge for staff across healthcare, including doctors, nurses, allied health professionals, pharmacists, psychologists and more.

We will **support early and mid-career** researchers, particularly those making their first step into independence, and **support research-protected time for clinicians and healthcare professionals.** This will help build research capacity at GOSH and the UCL GOS ICH and develop the next generation of research leaders.

We will also **establish a future leaders fund** to recruit and support talented research leaders, at senior lecturer or associate professor level or above, in key areas where there is a need to build capacity or expertise. In turn, they will attract, develop and mentor a talented pool of up-and-coming researchers, increase research activity and secure further funding for child health research.

We will achieve this by growing our partnerships with established funding schemes, for example with the NIHR-funded GOSH BRC and ORCHID (the Centre for Outcomes and Experience Research in Children's Health, Illness and Disability), which focuses on research capacity building by providing structured support to nurses, allied health professionals, clinical scientists and any other associate clinical, non-medical professionals across the Trust.

These collaborations will expand the funding available to surgical scientists and open up more fellowships, projects and internship schemes for staff throughout GOSH, the UCL GOS ICH, and GOSH BRC's national partner hospitals.

Spotlight on:

# Dr Polly Livermore

## Senior Paediatric Rheumatology Nurse at GOSH

"In 2015, I applied for a GOSH Biomedical Research Centre research internship with GOSH's Centre for Outcomes and Experience Research in Children's Health Illness and Disability, or ORCHID.

This helped me gain funding for a National Institute for Health and Care Research PhD, where I used mixed methods research to study the thoughts, feelings and experiences of children with juvenile dermatomyositis, which is a life-threatening autoimmune condition. I was able to publish my research and present my findings at conferences in Atlanta, Toronto and Berlin.

Towards the end of my PhD, I applied to become the clinical academic programme lead in ORCHID. I now run the internship programme that helped launch my own research career and I'm supporting others to do the same.

Most recently, I am delighted to have been awarded an NIHR post-doctoral fellowship. I am the first and only nurse in the country to have so far received this award since the programme began, and I am so grateful for all the support I have had towards this achievement. I'll be working with families to co-design and co-develop an artificial intelligence tool to help parents and children with rheumatic disease to manage their treatment plans more easily.

I hope my research journey will inspire the next generation of nurses to want to lead their own research endeavours.

I am excited that GOSH Charity is working with the BRC to expand their schemes and give more opportunities to nurses and allied health professionals across GOSH."

**—Dr Polly Livermore**

# Strategic centres

We want to establish a select group of strategic centres to focus on important areas of child health research. These will be areas where significant research is urgently needed and where GOSH could provide an ideal home for that research.

The centres will have an ambitious vision for change in their focus area, and will bring together networks of researchers, clinicians and experts from UCL and the GOSH community. They will collaborate nationally and internationally and join forces with research organisations, industry and other research funders.

**We want to invest up to £20 million** to develop the breadth and depth of research at these centres. In particular, significant funding will be provided to develop a strategic centre focused on **paediatric cancer research.**

## Cancer in children and young people

Cancer survival rates for children and young people have improved over time. However, some types of cancers remain hard to treat and those who survive may live with long-term side effects. Our ambition is to develop a strategic Paediatric Cancer Research Centre to **support research into the highest risk and hardest-to-treat cancers.** A £300 million Children's Cancer Centre is being established to care for cancer patients at GOSH and we want to maximise its impact by increasing research activity, improving the communication and sharing of research findings among patients and clinical staff, and providing ample opportunities for patients to take part in cutting-edge clinical trials.

We will also explore the development of other strategic centres that will support research in areas of most strategic importance to GOSH and the UCL GOS ICH. As well as cancer, we may explore bioethics and clinical informatics, along with continued support for the hospital's existing areas of strength, which include: inflammation and immunity; neuroscience; cardiovascular; endocrine and metabolic; tissue engineering and regenerative medicine.

## Research platforms and infrastructure

The hospital and the UCL GOS ICH, combined with the existing facilities at the Zayed Centre for Research and the NIHR funding for the BRC and Clinical Research Facility, already offer an environment where research can flourish. We want to help GOSH build on its reputation as a world-class hospital to become an 'intelligent research hospital' where research and innovation are embedded into everything it does.

To achieve this, we will support GOSH to enhance its research culture, bringing together multidisciplinary teams where expertise and knowledge can be shared, and the importance of research is emphasised. We will also support the hospital with the aim of offering every child the opportunity to participate in research, or for research to be offered as standard as part of every patient journey. We know patients treated at hospitals that carry out research have better outcomes – they have more confidence in their care, feel more informed and tend to survive longer.

Creating an environment that is 'research ready' is also vital to maximising research opportunities. We can help GOSH achieve this by ensuring that the cutting-edge tools and infrastructure needed for research are available and accessible to everyone.

To support this aim, we will work in partnership with GOSH and UCL, and provide around **£7 million of funding for world-class research tools and infrastructure.**

### Increasing clinical research capacity

We will support the **growth of hospital-based research.** We will focus on increasing research capabilities and activity in departments that have been less research active than others in recent years.

### Understanding the specialised facilities and technology researchers need

Allowing researchers access to the right platforms, technologies and expertise will maximise our investment in research. We will therefore carry out a review to help us fully understand the existing provision and **see where and how investment could have the greatest impact.** We will work closely with the hospital and UCL GOS ICH to do this.

### Bringing researchers together

We want to promote the idea that research is for everyone and **create a culture of research-led care across the hospital.** We can achieve this by publicising opportunities to join in with research and by closer collaboration between GOSH and the UCL GOS ICH. We will provide funding for healthcare professionals and scientists to run specialist meetings, workshops or networking events to bring expert teams together, with a particular focus on supporting closer collaboration between clinical and academic researchers.

## Alyssa's story

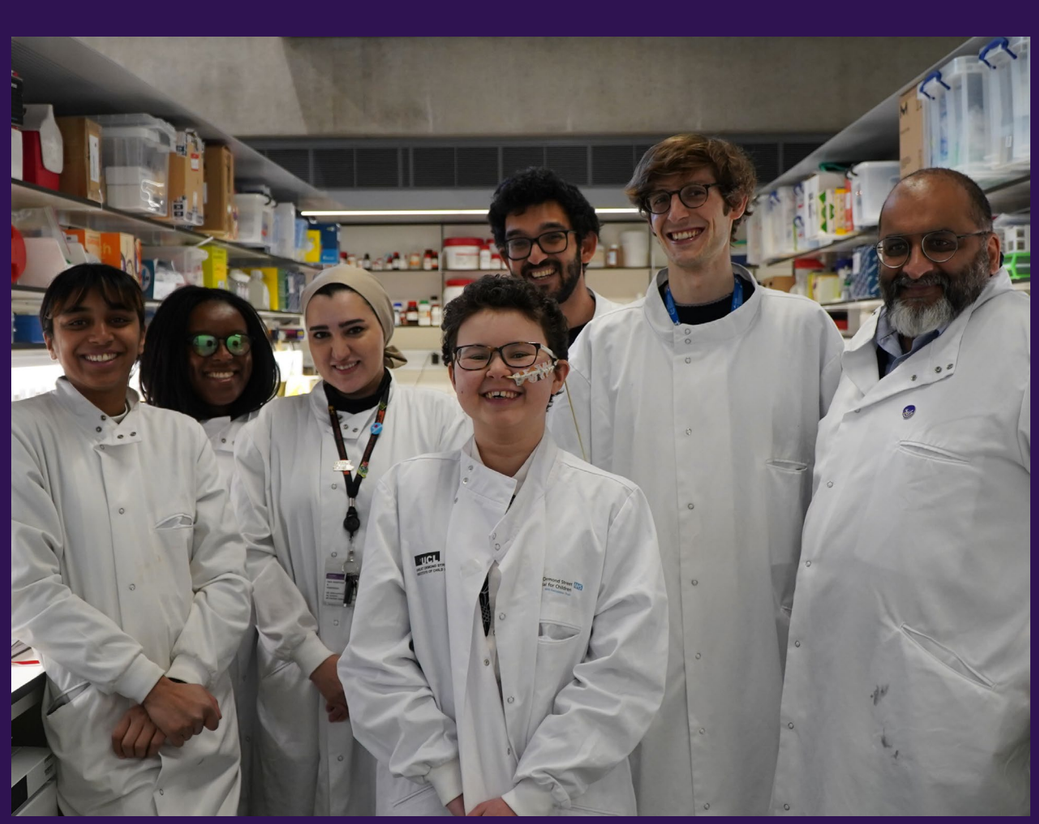
Alyssa from Leicester was just 12 years old when she was diagnosed with T-cell acute lymphoblastic leukaemia in 2021. She was treated with all the current therapies, but these did not cure her cancer.

As part of a trial at GOSH, Alyssa was treated with genetically modified immune cells that were created using a new technique called base editing. Without this experimental treatment, the only remaining option for Alyssa would have been palliative care. Alyssa's cancer is now in remission, and she is back at home recovering. "We're on a strange cloud nine to be honest – it's amazing," Alyssa's mum, Kiona, says.

Research on the base editing technique was led by Professor Waseem Qasim and his team based at GOSH and UCL, thanks in part to early funding from GOSH Charity.

"This is a great demonstration of how, with expert teams and infrastructure, we can link cutting-edge technologies in the lab with real results in the hospital for patients," Professor Qasim says. "It's our most sophisticated cell engineering so far and paves the way for other new treatments and ultimately better futures for sick children.

"We have a unique and special environment here at GOSH that allows us to rapidly scale up new technologies and we're looking forward to continuing our research and bringing it to the patients who need it most."



**Alyssa (middle), age 13, with the team of researchers behind her cure, led by Professor Waseem Qasim (far right).**

## Funding new research ideas

We aim to fund research on child health at all stages, from basic biology in the lab to patient trials, and onwards to creating new tests and treatments for patients. This funding will support researchers and healthcare professionals at GOSH and the UCL GOS ICH to investigate research questions that could help patients both in the near future and in the longer term. We also want to provide greater support for qualitative research, where researchers can ask more open-ended questions, for example about a patient's treatment experience or quality of life.

We will provide up to **£15 million to support the highest-quality research ideas.** This will be offered through competitive grant schemes that complement the funding already provided by our partners and other national funders.

### Research programme funding

We want to provide **long-term support for the most outstanding research programmes** that will produce the biggest impact for seriously ill children. We will provide competitive funding for long-term, large-scale research in areas where GOSH is considered world-leading or where we believe there is an opportunity to become world-leading. We will also encourage researchers to win grants from other national funders, and reward this by providing additional, complementary funding.

### Starter grants for research

In the past, we have offered Clinical Research Starter Grants. These supported pilot studies, often done by early career researchers, that were geared towards improving healthcare at GOSH. The grants **helped get research to a point where it could secure further funding to grow and thrive**. With just £3 million spent on starter grants between 2013 and 2018, researchers went on to achieve more than £37 million in additional funding. Based on this success, we will re-open this scheme, ensuring our eligibility criteria allow for applications from clinicians and allied health professionals, as well as expanding the opportunity to non-clinical researchers. We will encourage applications that address our new priority area, to improve the everyday experience of seriously ill children.

### Moving research into the clinic

Early translational research, where promising work from the lab first enters patient trials, is often a bottleneck in the progress of medical innovations, especially for rare diseases. That is why we have partnered with research charity LifeArc to jointly fund Translational Research Accelerator Grants at GOSH and the UCL GOS ICH. Ten studies have been funded through this scheme since it was established in 2019, including research on children's brain tumours and an inherited immune disorder.

We will continue this collaboration to **help more researchers translate their work into patient trials** and better understand how to move new technology into mainstream healthcare.

## Spotlight on:

# Professor Manju Kurian

### Consultant Paediatric Neurologist at GOSH and NIHR Research Professor at UCL GOS ICH

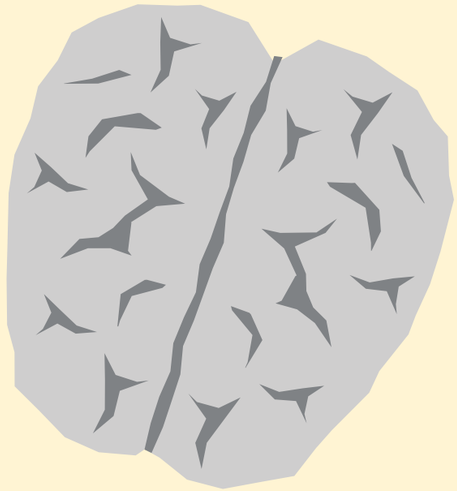
**Professor Manju Kurian discovered a rare condition called dopamine transporter deficiency syndrome (DTDS) when she was working on her PhD in Birmingham. Previously, doctors thought that patients with DTDS had cerebral palsy from birth injury, but her work showed it was a different condition caused by a single faulty gene.**

Children with DTDS are rarely able to learn to walk or speak, and there are currently no effective treatments or a cure. Most children with DTDS die before reaching adulthood.

In 2016, GOSH Charity supported Professor Kurian with a clinical starter grant of around £90,000 and, in 2021, she and her colleagues at the UCL GOS ICH were able to 'cure' the condition in mice and in human brain cells in the lab.

This seed funding helped Professor Kurian to secure a further £3.5 million of funding from the NIHR and Sir Jules Thorn Charitable Trust. Now, she plans to work towards a clinical trial that will offer hope of a treatment for patients with DTDS and other similar brain disorders.

She says: "It can be very hard to find funding for early-stage research. The support from GOSH Charity helped me build research capacity and was key to progressing my work towards patient trials."



## National child health priorities

We want to support researchers to carry out the most outstanding work, wherever they are in the UK. National funding not only benefits children across the country who take part in the research, but it can also have widespread impact as many research results are used to improve clinical care nationally. We are already the largest charity specifically funding child health research in the UK and we want to increase our role as a national research funder even further.

**We will provide up to £15 million to support national research** over the next five years. We also plan to expand our national funding into priority areas for child health research, such as mental health and wellbeing; health inequalities; obesity; data and informatics; and supporting the next generation of paediatric researchers.

### Nationwide funding for research

We began providing national funding for research in 2011 and this expanded following our partnership and subsequent merger with Sparks Charity in 2017. Through the GOSH Charity National Funding Call, **we will continue to offer at least £2 million in national funding per year for the next five years** to support high-quality research on the most pressing issues in child health. We will also continue to work with smaller rare disease charities to fund projects in their area of focus. These partnerships have already provided over £1 million investment in national paediatric research.

### Bringing the best UK researchers together

The National Paediatric Research Excellence Initiative is a collaboration between GOSH, Alder Hey Children's Hospital in Liverpool, Sheffield Children's Hospital, Birmingham Women's and Children's Hospital. We will provide support for the initiative in partnership with the GOSH BRC. Through this initiative, we will encourage collaboration to **build on our shared expertise** and develop innovative research projects in priority areas. We can also give more patients the opportunity to take part in cutting-edge research.

### Open access policy

We want our researchers to **publish their findings where they can be read and used by others**, ideally via open access journals and platforms. However, this costs money, so we plan to develop a policy on open access publication, including funding for the researchers we support.

## Spotlight on:
# Dr Heidi Fuller
### Senior Lecturer at Keele University

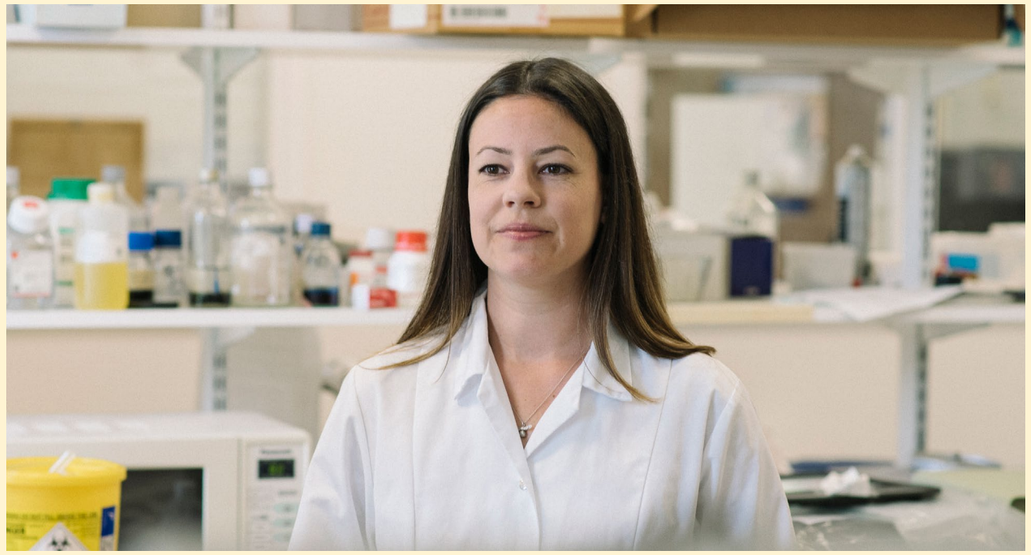
**Dr Heidi Fuller researches the childhood form of motor neuron disease (called spinal muscular atrophy, or SMA) at Keele University. With funding from GOSH Charity, Dr Fuller and her colleagues have been studying how this disease develops and looking for new ways to treat it.**

"Funding from GOSH Charity's National Call allowed us to characterise the molecular defects in cells from children with different severities of SMA. In doing so, we identified key molecular differences between SMA severities, some of which may be useful as biomarkers for monitoring treatment efficacy, and for the identification of severity-specific treatments.

"Beyond the impact of the work on advancing SMA research, the grant has benefited my research group enormously. We employed a postdoctoral researcher new to SMA research who has since gone on to secure fellowship funding to continue her work. The award also facilitated several new national and international collaborations.

"From a personal perspective, the award was instrumental in my promotion to senior lecturer at Keele University and recognition at national and international levels. We're so grateful to GOSH Charity for the work it does to support research across the country and for its support throughout our project."

**—Dr Heidi Fuller**



# Our research culture

## Holding the highest standards

To support the very best quality research in child health, we need to be well-organised and accountable. We already have an established Research Strategy Advisory Board, made up of external members who help bring a national perspective, as well as stakeholders from across GOSH, the UCL GOS ICH and GOSH Charity. This group was created to monitor the success of our previous research strategy. It also provides ongoing strategic evaluation alongside our Research Assessment Panel – the group of independent experts who review applications and provide recommendations for our research funding.

We are also a member of the Association of Medical Research Charities, adding quality, accountability, transparency and openness to our research funding. In line with this, all research applications will undergo a competitive peer review process, ensuring only the highest quality of research is funded.

We will continue to seek input from national and international experts, and from the patients and families at GOSH. We will also ensure consistency of governance with our closest partners, with cross-representation on the equivalent advisory and review bodies.

We will give greater consideration to intellectual property in our grant-making. This will encourage innovation, reduce delays in moving new technologies into the clinic and provide more benefit for patients.

## Equality, diversity and inclusion

We know that diversity of thought creates a thriving research environment. The research we fund must be diverse and inclusive to all, if we are to achieve our ambitious goal of transforming the lives of children with rare or complex disease.

We have already made progress and will do even more to understand the diversity of the applicants and beneficiaries of our research funding. This information will help us monitor, report on and improve our funding practice as required, to ensure our research portfolio is representative of the researchers we serve.

GOSH, the UCL GOS ICH and the GOSH BRC have appointed an Equality, Diversity and Inclusion manager. They will lead a review of existing processes to challenge hidden inequalities, monitor exclusivity and champion diversity. We will work closely with our partners and this key role to ensure the researchers we fund have equal opportunities.

## Involving patients, families and the public

Our research must be relevant to patients and their families, so we are committed to embedding patient, family and public involvement in our research governance. We also encourage patient and public involvement (PPI) across all the research that we fund.

To achieve this, we will create a PPI strategy in close alignment with our partners. This will ensure we continue to involve patients and families in the development and delivery of our research projects. It will also ensure there is patient, family and public input in decision making for all funding schemes that we run.

Through our existing partnership with Genetic Alliance UK, we will continue to provide support and guidance to facilitate our grant holders to embed patient views in their research methods and goals, to help shape and improve the impact of their research.

Spotlight on:

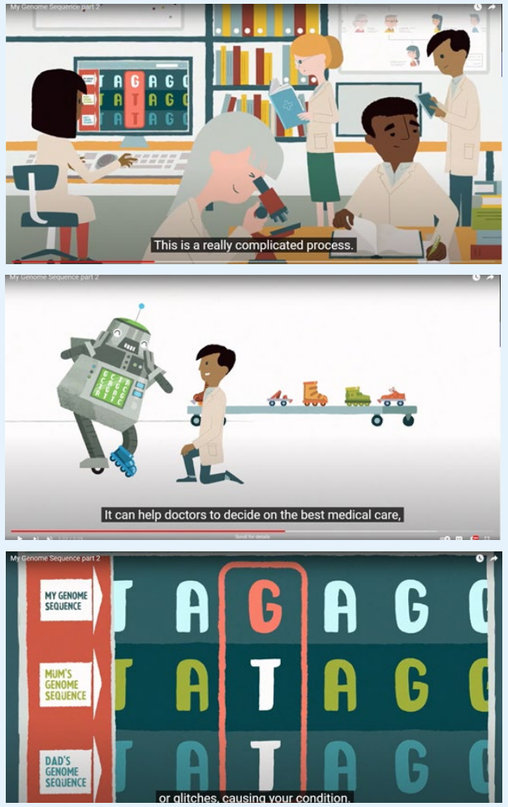
# Young people's perspectives

**Professor Dame Lyn Chitty wanted to find a way to help children and young people understand why they might be offered genome sequencing. With support from GOSH Charity, she and her team worked with the GOSH Young Persons' Advisory Group for Research to develop short videos to explain what a genome is and why genome sequencing can help children with rare or complex health conditions.**

Professor Dame Chitty says: "We spoke with this group twice. The first time to discuss how information about genome sequencing should be delivered and what it should include, and the second time to test out the script for the animation – to ensure it is clear, addresses issues of importance and is entertaining."

The team received vital feedback from young people about how to improve the videos, such as: "I found it was easy to understand. However, I didn't understand the risks of genome sequencing because it wasn't mentioned, maybe there could be a further animation showing this."

The videos they produced have been viewed thousands of times and been translated into Turkish, Chinese and Bengali. They have also become an important resource for young people who will be having genome sequencing in the new NHS Genomic Medicine Service.




**A selection of stills from genome sequencing videos**

# Evaluating our impact

It is vital that we can demonstrate the impact of our research funding now and for the future, particularly how our investments in research lead to longer, healthier and better lives for seriously ill children.

Our research objectives are aspirational, and we know we will not achieve them alone. We are part of a unique partnership with GOSH, the UCL GOS ICH and the GOSH BRC, and our impact should be viewed as a contribution towards our joint achievements.

We are currently developing a framework to help us judge our impact, alongside our partners. This will ensure our grant holders share our objectives and understand how they can contribute to achieving our goals. We will measure both short-term research outputs and longer-term outcomes of previously funded work. We will assess our progress using a combination of measures, with monitoring throughout the next decade to identify and overcome barriers along the way. We have also developed a monitoring framework to measure the success of our research strategy and its activities. Success metrics will be both scheme and priority specific, to clearly demonstrate the difference that our charitable funding is making.

"The impact of research on children and their families living with a rare disease is huge. We've seen major advances in diagnoses and treatments over recent years, but there remains more to be done. We want to expand our funding to meet this need and provide hope of a brighter future for seriously ill children. If you have a research idea that you want to pursue, then we want to hear from you."

**— Dr Alexandra Bonner, Head of Grant Funding at GOSH Charity**

"This strategy has the potential to change lives. It provides clear direction and an organised approach to ensuring that the impact of the charity's research funding is maximised. I am looking forward to seeing the strategy implemented and the lives of seriously ill children being continuously transformed through research-led care."

**— Professor Sir Doug Turnbull, Chair of Research Strategy Advisory Board**


# Acknowledgements

We are hugely grateful for the time and insight of all those who contributed to the development of our strategy. This included 58 patients, families and members of the public, and more than 100 members of the paediatric research and clinical community at local, national and international levels. Our Research Strategy Advisory Board was also instrumental in shaping our strategy and ambition. By working together, we can transform the lives of seriously ill children through research-led care.

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