



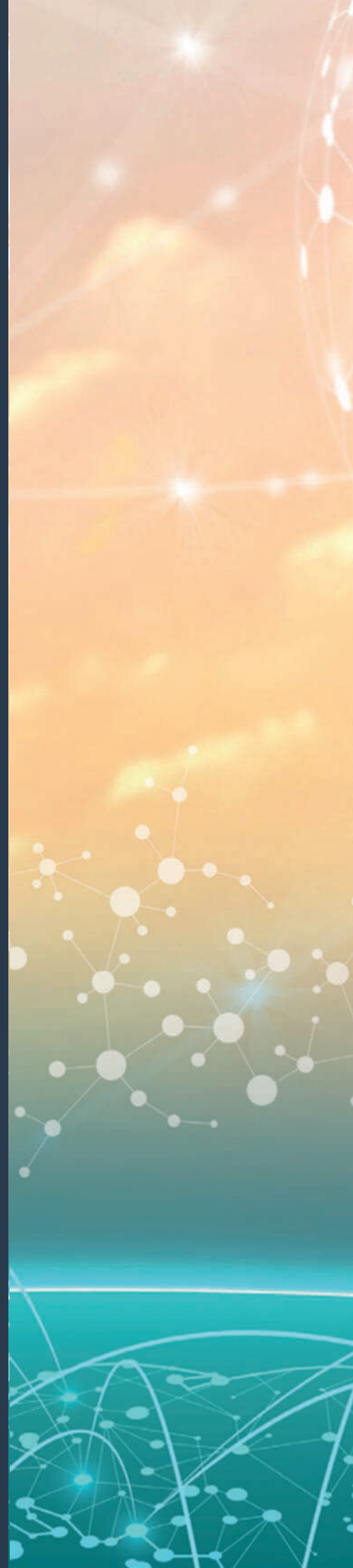
Children's
Cancer and
Leukaemia
Group


the EXPERTS
in CHILDHOOD
CANCER

45
YEAR
ANNIVERSARY

2016-2021 | Finding a cure for
childhood cancer

Celebrating CCLG's research impact





Every child with cancer deserves a future, no matter how rare their cancer is.

For 45 years, we have been at the forefront of children's cancer. We are leaders and experts in this specialist field and have a unique position as both a research funder and professional voice.

Our research drives innovation, inspires change and delivers results for all children with cancer.

We are proud of everything we have achieved in this time, and we invite you to join us as we celebrate CCLG's role in children's cancer research today.

WE ARE **CHANGING**
CHILD **CANCER**



Our CEO's message

We are proud to have funded and supported research for many years, contributing to the huge advances we have seen over the past four decades. Back in 2015, we published our first research strategy to clearly state our vision. We then became members of the prestigious Association of Medical Research Charities (AMRC), a hallmark of excellence for funders of research, and our research journey began.



Ashley Ball-Gamble
Chief Executive

Our unique role as both a charity and professional association gives us a strong position to grow important collaborations, share expertise and offer our established grant management infrastructure to other charities, enabling them to invest in pioneering research alongside us. This avoids duplication of resources and ultimately means more funds can be spent directly on research. We are now working on developing the CCLG Research Funding Network, working with more charities than ever before to drive change in childhood cancer.

Over the last five years, we have funded research through our charity partnerships and Special Named Funds, who raise vital funds for specific areas of research. Our collaborations with Bethany's Wish, Grace Kelly Childhood Cancer Trust, Neuroblastoma UK, The Harley Staples Cancer Trust and Blood Cancer UK mean that more high-quality childhood cancer research is funded. Our biggest partnership is with The Little Princess Trust, who sought out our support in 2016 to begin investing in research. We now have an ongoing, thriving collaboration which benefits the research community and, ultimately, children with cancer. Working with them and supporting them has been a highlight of our research journey.

We thank our supporters, researchers, healthcare professionals and charity partners for their commitment and dedication to research. I am hugely proud of what we have achieved over the past five years, however, some childhood cancers have not seen the same improvements in outcomes as others. There are still significant gaps in our understanding of childhood cancers and many treatments still result in significant, lifelong side effects.

This report is a celebration not just of research that CCLG has helped to fund, but the impact of our collaborations and partnerships on the childhood cancer research landscape. It reflects on our progress towards finding a cure for children's cancer.

We did not come this far to only come this far. We will not stop until all children with cancer survive and live healthy, independent lives.

“My daughter Bethany was diagnosed 14 years ago. I'd like to think if she was diagnosed today, things would look a lot different. She would be able to have her tumour DNA sequenced, have access to clinical trials, and have kinder treatment that would allow her to have a child of her own one day. This is thanks to the dedicated professionals who make sure that research happens for children with cancer.

Together, we can do anything. If we work together, we can make sure that children can survive cancer for longer.”



Angela Polanco
Mum of Bethany, who raises funds for Wilms tumour research through Bethany's Wish, a CCLG Special Named Fund

Our research story so far

With our long-standing history and experience in childhood cancer, we have an established and growing network of professional experts who respond to challenges and influence positive change.

We share important information among our clinical and scientific communities for the benefit of patients through excellence of care.

Our aim is to find a cure so all children with cancer survive and live healthy, independent lives.

From small beginnings ... come great things

In 2015, we launched our first CCLG Research Strategy to help fund research into key areas of children's cancer.

We identified a broad base of research to cover all aspects of children's cancer and to fill in gaps not served by any other national funding.

Our research aims were to:

- ▶ Broaden and deepen our knowledge of childhood cancer so we can fight it more effectively
- ▶ Transfer scientific breakthrough from the laboratory direct to clinical practice
- ▶ Improve treatment and patient care across the whole cancer experience
- ▶ Investigate the long-term effects of having had treatment as a child and find out ways of reducing these

Together with our partners, our network is one of the biggest funders of children's cancer research in the UK.

Why is research into childhood cancer so important?

Childhood cancers are biologically different to adult cancers so we need to understand why and how they can be treated.

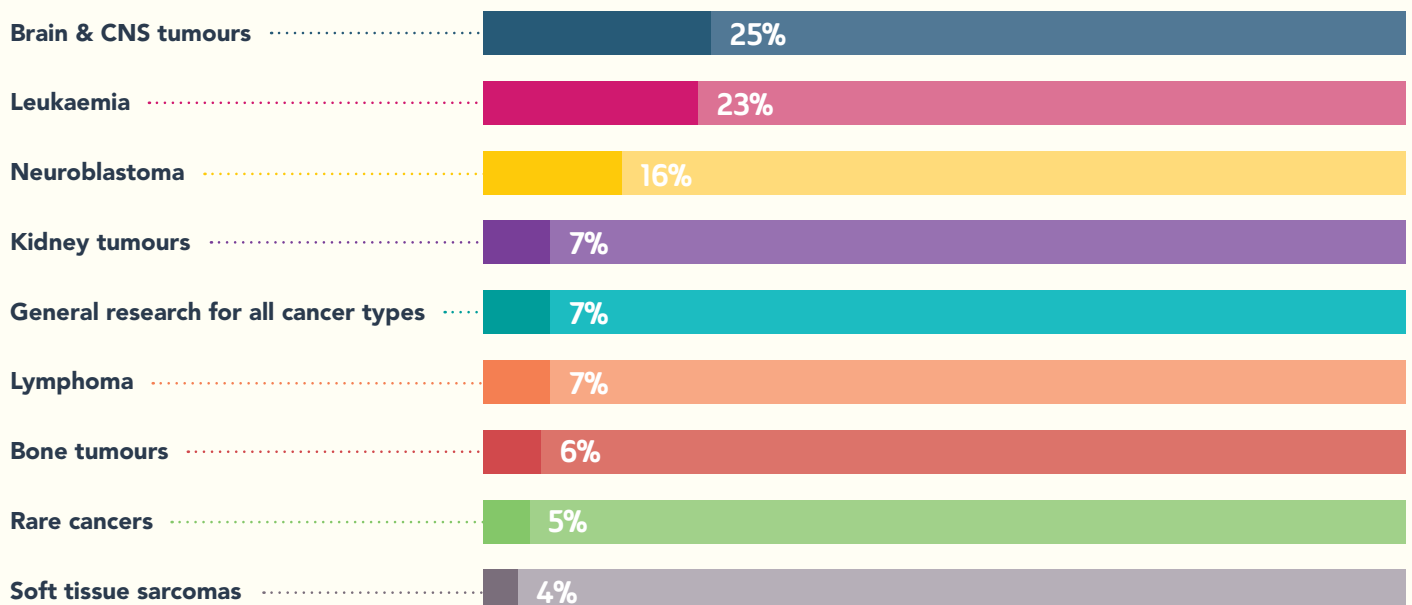
Side effects of having cancer and treatment on a child's body that is still growing and maturing can be huge and long-lasting.

Having cancer as a child can affect future health with long-term effects appearing later in life as an adult.

Number of projects and funding amounts 2016-2021



Research investment reflecting childhood cancer types



Our journey to success

2015

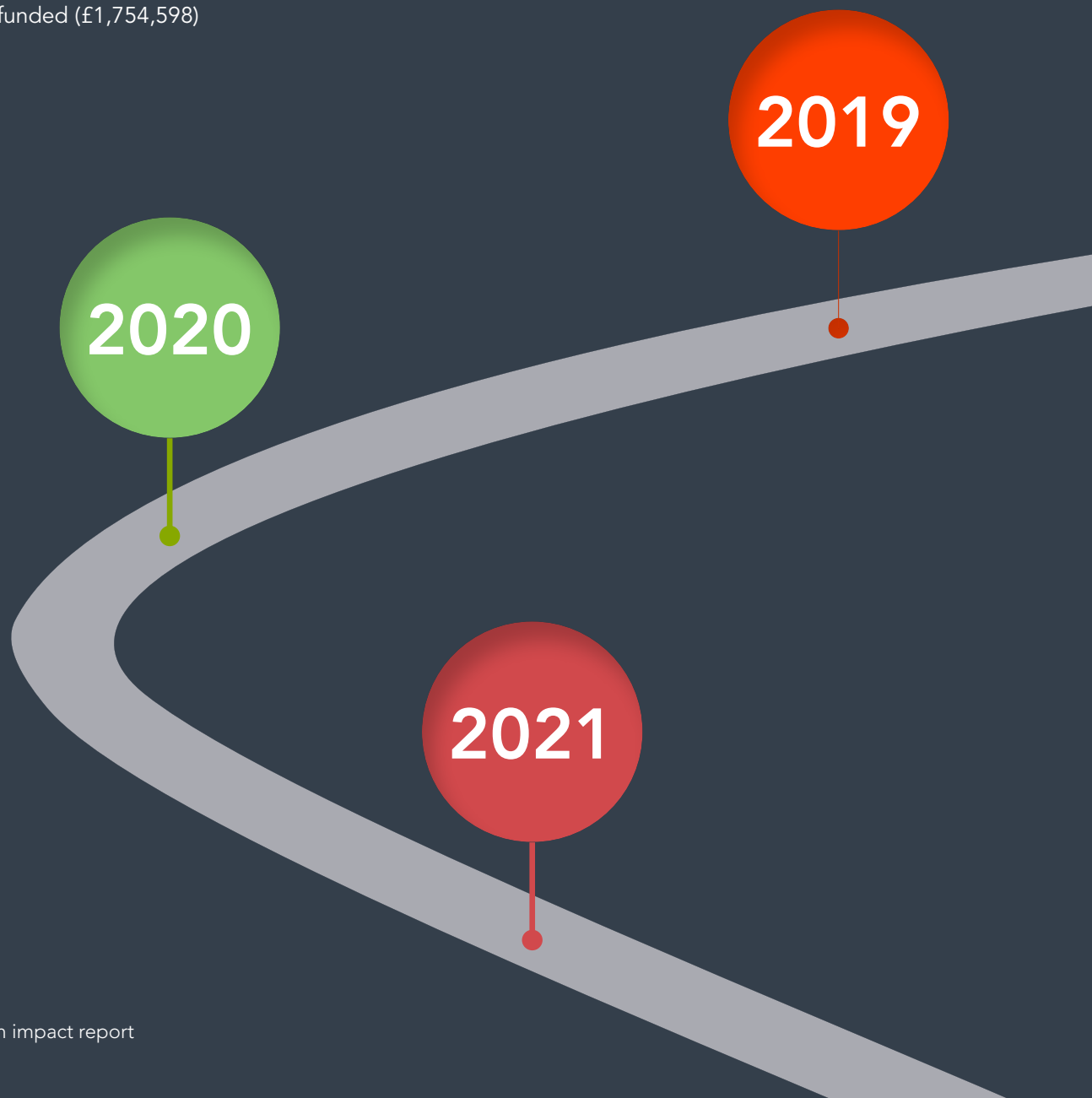
- ▶ First research strategy designed to urgently fill research gaps and to find better and kinder treatments for children with cancer
- ▶ Special Named Funds portfolio launched to increase dedicated funding for cancer in children. This was a new way of supporting families and their communities to raise funds for specific research that was important to them

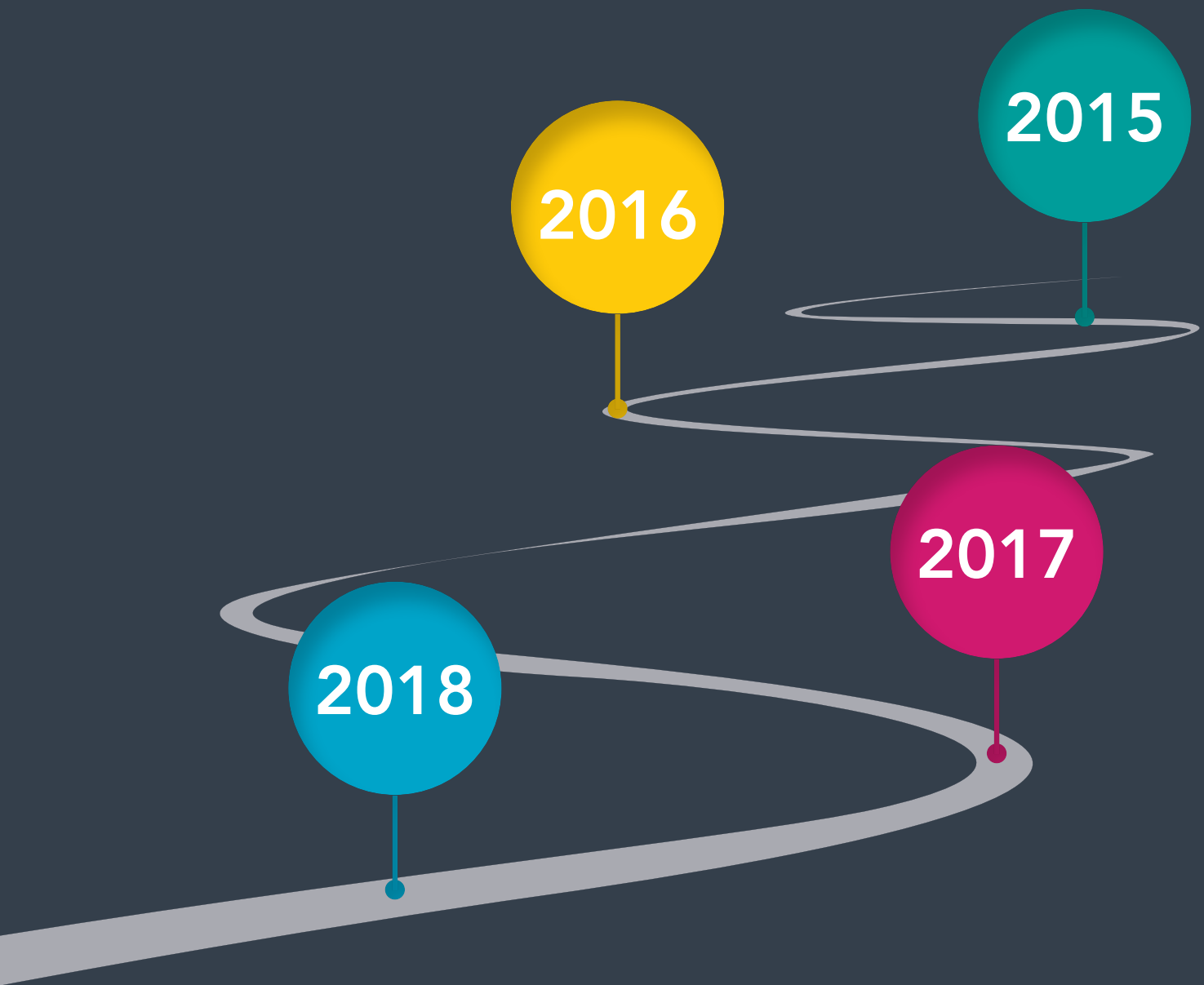
2016

- ▶ Expert-led Research Advisory Group formed of senior researchers and scientists from across the cancer research sector
- ▶ The Little Princess Trust partner with CCLG to release their first funding grants
- ▶ CCLG 40th anniversary funding grants released to support new projects
- ▶ 9 projects funded (£603,387)

2017

- ▶ Accredited member of the Association of Medical Research Charities (AMRC), demonstrating we robustly assess all research applications to make sure that we only fund high-quality research that will make a real difference to children with cancer
- ▶ Grace Kelly Childhood Cancer Trust partnership to fund more research into rare and aggressive cancers
- ▶ 26 projects funded (£1,754,598)





2018

- ▶ CCLG's Childhood Cancer Research Funds launched to open up fundraising opportunities for research
- ▶ 9 projects funded (£555,000)

2019

- ▶ Dr Sarah Evans joins CCLG as Research Manager to create our first dedicated research team
- ▶ 31 projects funded (£5,152,166)

2020

- ▶ CCLG's online grants management system implemented for efficient processing and review of applications
- ▶ CCLG's first parent research advisory group created to represent parent views
- ▶ Launch of Children's Cancer Priority Setting Partnership with the James Lind Alliance to identify top research priorities for children
- ▶ New research grants released for research into long-term side effects of childhood cancer
- ▶ 24 projects funded (£2,901,860)

2021

- ▶ AMRC awards CCLG with 'Best Practice in Medical and Health Research'
- ▶ Research Awards launch for the Winter Meeting 2022, open to all researchers involved with CCLG's research
- ▶ 21 projects funded (£3,732,607)

Our national impact

Our highly-skilled research teams work across all parts of the UK. 32 different institutions in 22 different cities from across the UK and Ireland have received research funding through CCLG.

BELFAST

Queen's University Belfast

MANCHESTER

University of Manchester

LEEDS

University of Leeds

DUBLIN

Trinity College, Dublin
University College, Dublin

BIRMINGHAM

University of Birmingham
Birmingham Children's Hospital

NOTTINGHAM

University of Nottingham
Nottingham University Hospitals

CAMBRIDGE

Cambridge University Hospital NHS Foundation Trust
University of Cambridge
Wellcome Sanger Institute

CARDIFF

Children's Hospital for Wales

BATH

University of Bath

LONDON

Great Ormond Street Hospital
Institute of Cancer Research, London
King's College London
UCL Institute of Child Health
University College London

OXFORD

University of Oxford

GLASGOW

University of Glasgow

EDINBURGH

University of Edinburgh

NEWCASTLE

Newcastle University

YORK

University of York

ULSTER

Ulster University

SHEFFIELD

University of Sheffield

SWANSEA

Swansea University

SOUTHAMPTON

University Hospital
Southampton NHS
Foundation Trust
University of
Southampton

SURREY

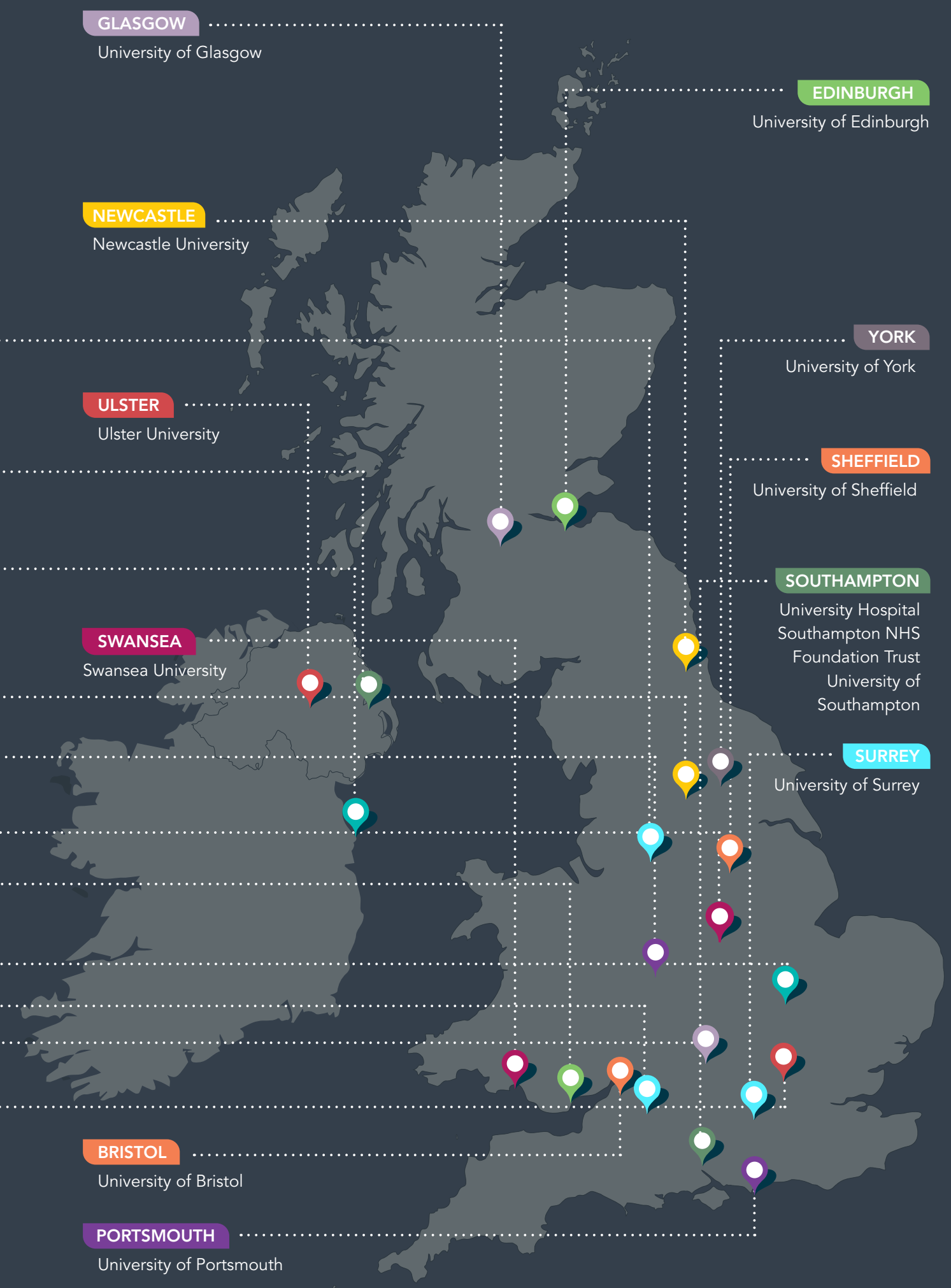
University of Surrey

BRISTOL

University of Bristol

PORTSMOUTH

University of Portsmouth



How we make a difference

Research increases our understanding, builds knowledge and gives important information on which to make decisions for the benefit of patients. Our aim is for all children with cancer to directly benefit from research as quickly as possible through communication and collaboration within the paediatric oncology community.

1

Sharing new knowledge

- 46 published journal articles
- Presentation of research at national and international conferences, including the International Society of Paediatric Oncology (SIOP)
- Poster prizes awarded to researchers who presented findings from CCLG projects

2

Developing innovative tools and techniques as a resource

- Innovative models of cancerous tumour cells to support more realistic laboratory study
- Freely available genomic, transcriptomic and proteomic data about the cells causing cancer

3

Influencing decision-making about healthcare practice

- Engagement with families and the public
- Discussions about MDT teams, proton therapy selection and many others

4

Stimulating further research

- Follow-on grants
- External funding

5

Protecting a future generation of researchers

- Supporting doctoral students to train in children's cancer research: 60 post-doctoral researchers have gained vital experience in specialisms through our research
- Dr James Grist – won the Sir Peter Mansfield Prize for Innovation in MRI with a project funded by The Little Princess Trust in partnership with CCLG
- Dr John Apps – won the 2021 British Neurological Oncology Research Young Investigator Prize with a project funded by The Little Princess Trust in partnership with CCLG



The Little Princess Trust partnership: Building a stronger funding base for children's cancer research

“The Little Princess Trust works in partnership with CCLG to fund childhood cancer research, searching for kinder and more effective treatments to improve the lives of young people.

We believe that CCLG holds a unique position in the landscape of children's cancer charities, and our grant-making activities have been superbly enhanced by its experience, infrastructure, knowledge, and expertise.

CCLG is perfectly placed to act as a bridge between research and patients, and to promote research to both the scientific community and the public.

The world of medical research is wide, complex, and daunting to those unfamiliar with funding applications and rigorous peer reviews. The dedicated team at CCLG has helped us overcome many challenges in such a professional manner and has played a pivotal role in The Little Princess Trust becoming one of the largest charity funders of childhood cancer research in the UK.”



*Phil Brace
Chief Executive Officer,
The Little Princess Trust*

“My training is in stem cell biology and early embryonic development. I come at paediatric cancer research from a different direction than most scientists in the field, and I bring different ideas on how to approach childhood cancer research. One of the first funding grants I received was from the The Little Princess Trust/CCLG grant scheme. This helped to kick-start my biology research into children's cancer. Once we understand how tumours start, then we can research how to make the cancers stop.”



*Dr Anestis Tsarkiridis
University of Sheffield*

Understanding how and why childhood cancer begins

We are unravelling why cancer cells begin to grow and how they develop. Our research builds a foundation for developing new and pioneering treatments that will specifically target cancer cells in children. Here are some research examples:

Investigating why one cancer gene can make leukaemia cells resistant to treatment

Dr Stefan Meyer, University of Manchester (funded by CCLG and CCLG Special Named Funds)

Having a high expression of a cancer gene called EVI1 can mean that a child's leukaemia could be more high-risk and the child has less chance of survival. Dr Stephen Meyer and his team looked into why EVI1 affects leukaemia cells so that they become resistant to treatment. They discovered that molecular changes can happen to EVI1

when it's exposed to damaging agents, such as chemotherapy, or when the cells divide and move through the cell cycle. This is important as further research can use this knowledge to develop more effective treatment for children with EVI1-driven leukaemia.

Finding genetic clues for treating neuroblastoma patients

Prof Deborah Tweddle, Newcastle University (funded by The Little Princess Trust in partnership with CCLG)

Neuroblastoma is a childhood cancer that has one of the lowest survival rates of all childhood cancers. We urgently need new knowledge to learn more about how neuroblastoma develops and how genes are involved in the different sub-groups of neuroblastoma. This will help us to find out which patients are most likely to benefit from which treatments.

Through a series of research grants funded by The Little Princess Trust, Prof Tweddle's research group has been able to look into how neuroblastoma progresses, what genetic abnormalities make this happen, and what types of treatments could be developed for children with particular sub-groups of neuroblastoma.

“We have created a research database on relapsed neuroblastoma that contains important clinical and genetic information which is essential for epidemiological and genetic studies in the future.”

Prof Deb Tweddle

Using knowledge about one type of children's cancer to help identify potential treatments for another

Dr Karim Malik, Bristol University (funded by CCLG and CCLG Special Named Funds)

Many cancers occur because they contain too many growth-promoting proteins, or 'oncoproteins'. One of these proteins, called MYCN, is known to cause many cancers, and lots of research has already been done to understand its role in neuroblastoma. Dr Karim Malik's study builds on this research to collect evidence about its involvement in a children's kidney cancer called Wilms tumour. This is very important

for patients, as there are many novel drugs being developed to inhibit the actions of MYCN. This research has developed a laboratory model to test the role of MYCN in causing Wilms tumour.

“As there are many novel drugs being developed to inhibit the actions of MYCN, this study is providing the foundations to pave the way for new and specific treatments for Wilms tumour.”



Dr Karim Malik

“Research is important and can make a difference because cancer cells with errors in their genetic code provide researchers with the opportunity to target these errors directly. Cancer cells are very clever and manage to develop ways to make sure they stay alive and grow quickly. We want to find new ways to target these genetic errors to try to outsmart the cancer cells.”



Dr Lisa Russell

Unlocking the genetic codes of childhood cancer

We now live in a new era of genetic research where we are understanding more about the different genes that help cancers grow and spread. Our research sits at the forefront of this specialised field to find targeted approaches that are personalised to each child's own cancer type. Here are some examples of our research projects:

Dissecting genetic changes to learn more about how to treat a rare type of childhood cancer

Prof Suzanne Turner, University of Cambridge (funded by CCLG and CCLG Special Named Funds)

Anaplastic large cell lymphoma (ALCL) is a cancer of the immune system that affects a type of white blood cell called a T-cell. It is important to know what genetic changes are present in ALCL as these can tell doctors whether the patient has a good or a poor prognosis, meaning appropriate treatment can be tailored for each child.

Prof Turner's research group has collected the largest number of ALK-negative ALCL tumour samples from children worldwide – this is an exceptionally rare disease where only 11 cases have been seen across Europe in the last 10 years.

Prof Turner is working collaboratively with international groups to assess the genome

of tumour samples using a technique called 'fluorescence in situ hybridisation' (FISH) as well as next generation gene sequencing. The data collected will identify which genetic changes are present and relate this to impact on prognosis for each child, so treatment is better tailored for each child.

This is the first study to detail the genetics of children with this rare sub-type of cancer, and its findings will be able to help guide doctors on how to best treat children with genetic combinations involved in their cancer.



Prof Suzanne Turner

“ALK-negative ALCL is exceptionally rare in children so we have very little knowledge of how to treat this cancer in children. We have put together the largest global collection of these cases and are analysing them for changes to the DNA code that might be driving tumour growth.

In particular, we are looking to see if children with this cancer show the same genetic changes as adults where it is less rare. Once we know more about this, it will be easier to design personalised treatment approaches specific to an individual child's cancer.”

Transforming knowledge of childhood kidney tumours to help personalise treatments for each child (funded by The Little Princess Trust in partnership with CCLG)

Wilms tumour is the most common kidney tumour in children. Whilst most patients are treated successfully, in some children the tumour grows back. These three related projects looked at genetic changes which would predict which patients are most at risk of the cancer coming back so that they can be given more intensive treatment.

PROJECT 1

Using tumour DNA changes to predict relapse for children with Wilms tumours

(Prof Kathy Pritchard-Jones, University College London/Institute of Child Health)

Combines genetic analysis and clinical data in order to personalise treatment for children with Wilms.

PROJECT 2

Identifying when gene changes in Wilms tumours begin

(Dr Sam Behjati, Wellcome Trust Sanger Institute)

The aim of the research was to define the order in which mutations happen in Wilms so that better drugs can be found to target these mutations.



PROJECT 3

The Little Princess Trust Wilms Tumour Knowledge Bank

Cutting edge sequencing methods to build a 'knowledge bank' that combines genetic information with clinical outcome data.

“A valuable resource for researchers and clinicians around the world”

Predicting how cancer behaves in children

We are understanding more about how a child's cancer is likely to behave and respond to treatments. This is key to making sure children get the right amount of treatment at the right time, to improve outcomes and reduce side effects. Here are some of our research projects:

Using functional imaging for better diagnosis, management and monitoring

Prof Andrew Peet, University of Birmingham (funded by The Little Princess Trust in partnership with CCLG)

Imaging is central to diagnosing and monitoring solid cancers and scans are a key part of the patient and family journey. Imaging is often thought of as producing pictures from the inside of the body. However, there has been a shift towards using imaging to give information on the properties of tumours, such as the blood flow in them or their chemical make-up. MRI scans can be readily adapted to acquire a whole range of information on tumour properties, giving us a better idea of what type of tumour the child has, and how aggressive it is going to be. These methods together are sometimes called 'functional imaging', since they aim to investigate how the tumour is functioning.

The CCLG Functional Imaging Database now has more than 1,500 cases with scans collected from 10 children's cancer centres and is at the forefront of the international effort to develop imaging and artificial intelligence (AI).

Two projects have been funded by The Little Princess Trust. The first project focused on predicting survival for an individual child. Some tumours don't behave as doctors would expect, and usual treatments don't work. Unfortunately, it can take a long time to know whether a treatment is working, sometimes too long when tumours are not responding to treatment. Prof Peet and his team are using AI to help analyse the images coming off the MRI scanner before a child has started treatment to predict whether standard treatments will work, or if they would need to be considered for new therapies to treat their tumour.

The second project focuses on helping doctors interpret new scans, which can be challenging for them. The team have designed an app that allows hospitals to use these advanced scans to help diagnose their patients.

“In 2004, we set up the Functional Imaging of Tumours study with CCLG and our network of hospitals. We used computers and early AI to analyse the images. Success came quickly and we achieved impressive accuracy in diagnosing the main childhood brain tumour types.”



Prof Andrew Peet

Developing predictive tests to give personalised treatments for children with leukaemia

Dr Chris Halsey, University of Glasgow (Projects funded by CCLG and CCLG Special Named Funds, and The Little Princess Trust in partnership with CCLG)

Acute lymphoblastic leukaemia (ALL) is the most common form of childhood cancer and children with ALL are treated with chemotherapy. However, knowing how best to treat leukaemia that has spread to the brain (CNS) is a particularly challenging question. Curing ALL requires killing the leukaemia cells that hide in fluid around the brain, called cerebrospinal fluid (CSF).

Current tests for CNS leukaemia are basic, and do not predict CNS recurrence, which is often incurable, so all children receive large amounts of CNS-targeted chemotherapy to try and prevent this. This treatment is unpleasant and has serious side effects in some children, including seizures and lower intelligence. Better predictive tests are urgently needed to identify children who can safely receive less intensive treatment and identify those at high risk of leukaemia returning to the brain.

Funded by a CCLG project grant and by The Little Princess Trust through CCLG, Dr Chris Halsey and her team have been working on two projects to develop personalised treatments for directing

chemotherapy to the child's brain in ALL. Their first project researched the development of highly accurate tests for leukaemia that has spread to the brain, with an ultimate aim to deliver a personalised amount of chemotherapy, tailored to each child's specific risk. The team explored several approaches for tests using different markers of leukaemia cells which are now being explored in the next phase of research to adapt this into a safe test for use in patients.

Dr Halsey's new study, called CSF-FLOW, will be offered to children taking part in the current international leukaemia trial called ALLTogether-1. The team will collect CSF during chemotherapy treatments and use flow cytometry to accurately measure the amount of leukaemia in the CSF and how quickly it responds to treatment. They plan to use this information to identify children with both very low amounts of leukaemia in the CSF, who might be able to receive less brain-directed chemotherapy, and very high amounts of leukaemia in the CSF, or other high-risk features, who might be better cured with different treatment approaches.

“This development revolutionises the approach to treating leukaemia in the brain by adapting the amount of treatment to the risk of leukaemia coming back.”



Dr Chris Halsey

“ We are designing a new ‘super drug library’ of drug compounds. We will use this large library of approved drugs and test these in all cell lines to find the most promising drug candidates for AML.”



Dr Kyle Matchett

“This novel laboratory platform can be used to design therapies that are not only more efficient but also mitigate toxic side effects and minimise treatment-resistance.”



Dr Deepali Pal

“It is critical to understand why the cancer cells are not eliminated by the first round of chemotherapy. If the resistant mechanisms can be identified, drugs can be evaluated that may halt the ability of these cells to resist front-line therapies, and ensure that tumours do not relapse.”



Dr Karim Malik

“This research provides support for the introduction of new drugs into a recently opened clinical trial for children with rhabdomyosarcoma, and ultimately may lead to improved outcomes for high-risk rhabdomyosarcoma patients.”



Dr Janet Shipley

“Further funding has been awarded to use in silico and experimental models to validate the most effective small molecules that decrease Ewing sarcoma cell number and synergise with conventional chemotherapy.”



Prof Sue Burchill

“Many people believe that only aggressive tactics can cure cancer. This research is helping to demonstrate that there is a kinder way to tackle cancer in children. If we want children with cancer to live long and happy lives, we must design drugs with optimism and thoughtfulness, planning for the life of the child after cancer.”



Dr Jessica Taylor

Upgrading treatments to give better outcomes

We are looking at understanding more about treatments for childhood cancer to see if we can refine them to make them more effective. Here are some of our research projects:

Identifying the benefits and challenges of proton beam therapy in treating abdominal tumours

Dr Mark Gaze, University College Hospitals London (funded by The Little Princess Trust in partnership with CCLG)

This project has helped scientists and clinicians understand the benefits and challenges of proton therapy to the upper abdominal region. Proton therapy is known to be a more targeted form of radiotherapy which reduces harm to the surrounding critical organs. However, this research has shown that this may not always be the case for a small number of patients with abdominal neuroblastoma, due to various scenarios that can't be predicted, such as weight and bowel density changes.

Dr Mark Gaze and his team have identified certain tumour characteristics (size and location) on imaging that may potentially allow clinicians to identify patients who could be adversely affected from proton therapy due to its challenges. It has improved understanding of the technical challenges associated with the delivery of pencil beam scanning proton therapy to the abdomen and its potential consequences.

“Whilst this work has focused on abdominal neuroblastoma patients, the concepts and strategies to mitigate the effects are applicable to other abdominal tumours such as Wilms tumours and pelvic sarcomas, as similar challenges in regard to density changes within the abdomen are present.



Dr Mark Gaze

This work has formed the basis of a clinical study which is currently in development with NHS England to validate this selection process nationally in the UK. We feel that the results of this will lead to definitive national guidance in due course. Parents can be therefore reassured that the modality of radiotherapy chosen for their child has been done with their child's best interest in mind.”

Understanding and enhancing antibody therapies for patients with neuroblastoma

Dr Juliet Gray, University of Southampton (funded by The Little Princess Trust in partnership with CCLG)

Dr Juliet Gray's project is supporting immune biomarker analysis on samples from children participating in anti-GD2 antibody combinational studies, in order to provide insight into the therapy's

effectiveness. This will help to find the best combinations of treatment and also to identify which patients will benefit from these, so more children can be cured without further toxic treatment.

Assessing the use of antibody therapy in treating life-threatening immune responses in children

Dr Frank Mussai, University of Birmingham (funded by The Little Princess Trust in partnership with CCLG)

Haemophagocytic lymphohistiocytosis (HLH) and macrophage activation syndrome (MAS) are life-threatening activations of the immune system occurring alongside cancer and infections in children. Current treatment is with untargeted, immunosuppressive chemotherapy, and the outlook is extremely poor with 25-50% of patients dying of

disease. This research is a small clinical study, led by Dr Frank Mussai, to test the use of antibody therapy called 'gemtuzumab ozogamicin' for children and adults with HLH/MAS. It has the potential to provide a new therapy for children and adults who have no other treatment options.

Evaluating the effectiveness of immunotherapy in treating relapsed and refractory anaplastic lymphoma

Dr Amos Burke, Cambridge University Hospitals (funded by The Little Princess Trust in partnership with CCLG)

This clinical trial, led by Dr Amos Burke, is assessing the effectiveness of nivolumab (an immunotherapy drug) in treating relapsed and refractory anaplastic

lymphoma kinase-positive anaplastic large cell lymphoma (ALK+ALCL).

“The ultimate aim is to see the relapsed/refractory ALK+ ALCL disease permanently controlled and for patients to have the best possible quality of life after being treated. The importance of this in paediatric patients cannot be overstated.”



Dr Amos Burke

Designing better patient care through clinical practice

We are refining the way in which clinical practice is organised and delivered so that children receive the best possible care throughout their cancer experience. To do this, we need to look at evidence to see what does and doesn't work so we can truly influence change. Here are some examples of our research projects:

Reviewing the effectiveness and organisation of national advisory panels

Dr Jessica Bate, University Hospital Southampton (CCLG 40th Anniversary Grant)

Multi-disciplinary teams (MDTs) are a central part of children's cancer care in the UK, with all children having their care reviewed by an MDT at a principal treatment centre (PTC). This team typically includes oncologists, surgeons, radiologists, pathologists, radiation oncologists, nurses and an administrative coordinator. Each patient's information, including that on their imaging and pathology, is reviewed by the MDT, which gives recommendations on a diagnosis or course of treatment.

Recent years have seen an increase in the number of national advisory panels (NAPs) for children's cancer. These differ from MDTs and are usually made up of national experts for a particular cancer type. Consultants may seek advice from these panels for an individual patient. It is important that this newer type of panel is properly evaluated, so that their role and remit in supporting children's cancer care is developed based on research evidence.

This project, led by Dr Jessica Bate, used interviews, focus groups and a review of documentation to evaluate selected NAPs in terms of the service they provide, and their value and impact for both clinicians and patients. The views of patients, parents and clinicians about national and local MDTs formed a central part of this research.

The project's first phase demonstrated the huge amount of work the national advisory panels undertake. The second phase focused on establishing the impact and value of the panels for clinicians and patients. Results showed that panels regularly enhance a local MDT plan, and their recommendations are implemented by the local team in more than 80% of cases.

The results of this patient-centred research are enhancing MDT working so that childhood cancer patients receive higher standards of care, ultimately improving patient experiences and outcomes.

“Focus group members felt national forums were well placed to support decision making for rarer situations such as rare tumours, unexpected complications of treatment and refractory/relapsed disease. A primary role was recognised as offering expertise and a second consensus in circumstances in which there may not be a defined treatment pathway.”



Dr Jessica Bate

Assessing quality of life in children with sarcoma

Dr Madeleine Adams, Children's Hospital for Wales (funded by CCLG and CCLG Special Named Funds)

There is currently very little known about the quality of life (QoL) for children with sarcoma during their treatment, but it is important that these issues are understood when designing treatment strategies. The international clinical trial for rhabdomyosarcoma (FaR-RMS) will measure QoL between the different radiotherapy randomisations and we need sensitive tools to show any differences. Dr Madeleine Adams' project is developing a questionnaire which can be used as a measure of QoL for children undergoing treatment for sarcoma - the Sarcoma Assessment

Measure: Paediatric Version (SAM-Paeds). Early versions of this tool have already been developed, and once finalised, SAM-Paeds will be used in the FaR-RMS study to better understand quality of life for children during treatment, and whether there is any difference between radiotherapy before and after surgery.

“By better understanding the impact of sarcoma and the burden of therapy for patients and families, we can provide better support for patients who need it.”



Dr Madeleine Adams

Assessing whether routine surveillance improves outcomes after relapse in solid tumours

Dr Jessica Morgan, University of York (CCLG 40th Anniversary Grant)

The follow-up of children who have been treated for cancer involves reviews by their medical team, as well as scans. But having lots of scans has some problems, including increasing the risk of having another cancer from radiation, having many anaesthetics, and increasing anxiety for families. This project explored in greater detail the early indications that most instances of children's cancer are found because the child develops symptoms, and that finding cancer from scans doesn't prevent children dying from the recurrence of their cancer. With the exception of Wilms tumour, Dr Jessica

Morgan and her research team found no evidence that regular scans after treatment help children live longer after their relapse. The findings of this research highlights the uncertainty surrounding the benefits of routine surveillance imaging for children with solid tumours outside of the brain. This research is sparking further discussion about routine surveillance in paediatric oncology, and a meeting of patients, parents and professionals has already been held to determine the next research steps in this area.

“The research involved patients and families in the design, interpretation and dissemination of the study, which is relatively uncommon in systematic reviews, but proved very successful in this work.”



Dr Jessica Morgan

Helping parents make difficult decisions about treatment and care

Dr Bob Phillips, University of York (funded by CCLG and CCLG Special Named Funds)

In children whose rhabdomyosarcoma (RMS) has not responded to treatment (refractory), or has come back after treatment (relapsed), there are difficult decisions to make about what treatment to give next. Only around one in five children with relapsed or refractory rhabdomyosarcoma can be cured, and therefore there are complicated choices to be made about how to prioritise their care.

The REFoRMS project, led by Dr Bob Phillips, is collecting information to better understand how decisions are made about treatment. The results will be collated into a best practice statement which will provide advice and support to clinicians and families about the important things to consider when discussing treatment options.

Research evidence is being collected in two ways. A systematic review will look for all previous early phase studies in relapsed and refractory RMS and see how effective the different treatments are for

different children. This will help to provide families and professionals with more accurate information about what to expect from the options available. There will also be an interview study, where researchers will speak to patients and families about how they have made, or are making, decisions about treatment in relapsed or refractory RMS. This will help to understand the decision-making process and how best to support families making these choices.

Parents of children with relapsed and refractory RMS have been involved from the beginning, meaning they are helping to ensure that the project is asking and answering the right research questions. This research is making a difference by providing families and clinicians with the most accurate information about new treatments in relapsed or refractory RMS, and helping to understand decisions made in this setting, so families can be better supported in the future.

“Fundamental to all clinical studies, we argue repeatedly, is including families and young people in helping create, steer and share the research. This project is being undertaken with this desire to include families affected along the way, to make sure the studies are sensible and meaningful.”



Dr Bob Phillips

Optimising drug treatment monitoring for childhood cancer patients

Dr Gareth Veal, Newcastle University (funded by The Little Princess Trust in partnership with CCLG)

It is difficult to treat some groups of childhood cancer patients, especially infants in their first weeks of life. It can be challenging to know how much chemotherapy to give to these children. If some patients do not receive enough chemotherapy, this reduces their chances of treatment response and survival. If some patients get too much, this can cause serious long-term health problems.

Dr Veal's study is finding out the best way to treat patients with the widely used anticancer drug

carboplatin. This involves measuring drug levels/exposure in individual patients and modifying drug doses based on these measurements. The team is establishing a national research programme to expand this work to include a wide range of drugs for treating infants with cancer. Dosing regimens are being improved and the findings are being fed into national treatment guidelines in patient groups where drug exposure is most variable.

Improving treatment decisions and outcomes in low grade glioma

Dr Darren Hargrave, Great Ormond Street Hospital (funded by CCLG and CCLG Special Named Funds)

Childhood low grade glioma is a group of diverse tumour types which can affect any part of the brain or spine and at any age from infants to teenagers. Although many patients survive, some children still die. In many cases, children need to be treated for multiple tumour regrowth meaning that many survivors quality of life can be significantly impacted. This study is analysing over 1,000 childhood low grade glioma (PLGG) patients treated at Great Ormond Street Hospital over the past 40 years to look at the factors that influence patient survival and quality of life.

The team is collecting information from survivors and their families about all aspects of daily living

and quality of life, providing a snapshot of the impact of having this type of cancer on patients.

The study team is working directly with parents and survivors to consider the results and to co-produce a medical model and guidelines to assist both doctors, and patients and their families, in making informed and shared treatment decisions. This is allowing a more individualised approach in the choice and timing of specific treatments for patients during the course of their illness.



Dr Darren Hargrave

“The strength of this project is that the data is based on ‘real world’ existing data from a multi-disciplinary team of doctors and scientists working at the largest paediatric brain tumour centre in the UK and one of the biggest centres globally.”

Improving bone marrow transplant for patients with high-risk leukaemia

Dr Robert Wynn, University of Manchester (funded by The Little Princess Trust in partnership with CCLG)

Bone marrow transplant (BMT) often cures children with leukaemia where chemotherapy has failed. The donor bone marrow makes both normal blood and a new immune system in the patient. However, sometimes this new immune system may attack the body of the patient because it is different from the donor.

There is scientific evidence that cord blood donation (taken from the placenta of a newborn baby) is better than bone marrow taken from a donor at eradicating leukaemia and also less likely to cause it to be rejected.

Dr Wynn’s team has shown that if white cells are given during a cord blood transplant, then cord

blood immune cells (known as T-cells), are increased very early after transplant. These T-cells are important as they are primed to attack the patient’s leukaemia, but also appear not to increase the risk of transplant rejection.

In this study, the team are undertaking a small clinical trial of children with leukaemia who are undergoing a BMT using cord blood and using white cell transfusions to stimulate the cord T-cells. The team will study the cord blood T-cells to understand how they are killing the cancer and to better understand whether this process is influencing successful BMT in leukaemia patients.

Transforming care for childhood cancer survivors beyond their treatment

We are supporting research into how we can understand the long-term side effects and challenges of having had cancer as a child. Many survivors have physical, social and emotional struggles after treatment has finished, and we need more knowledge so we can support them better throughout their life. Here are some of our research projects:

Encouraging childhood cancer survivors to take part in more physical activity

Prof Linda Sharp, Newcastle University (CCLG 40th Anniversary Grant)

Childhood cancer survivors have an increased risk of heart problems, which can sadly lead to some dying earlier than they should as a young adult. Despite this risk, and the known benefits of physical activity in reducing it, studies show that children who have a history of cancer take part in less exercise, but the reasons why haven't yet been well understood.

Professor Linda Sharp's research team talked to survivors of cancer and their families to better understand what helps and hinders physical activity for them. The team worked with survivors, parents, healthcare professionals and other experts in a

series of workshops where they explored together how attitudes towards taking part in physical activities could be improved. They then used this information to design and develop ideas for what should be included in a support package to help improve participation in physical activity in children who have survived cancer.

The results of this patient-centred research are enhancing multi-disciplinary team (MDT) working so that childhood cancer patients receive higher standards of care, ultimately improving patient experiences and outcomes.

Understanding the challenges of living with treatment-related hearing loss for brain tumour survivors

Dr Carmen Soto, Great Ormond Street Hospital (CCLG Late Effects Grant)

Children who are treated for brain tumours are at risk of developing hearing loss because of the disease and the treatments they receive. We understand a little about the effect that hearing loss has on children who have been treated for a brain tumour. They seem to have the same difficulties as other children with hearing loss (for example, problems with language, school performance, and relationship and emotional difficulties), but we don't really know what it's like to live with that hearing loss when you're also dealing with growing up after treatment for a brain tumour. It is really important to know this information because having a brain

tumour in childhood can also lead to lots of other complications, like problems at school, or poor eyesight. Children and families also have to deal with a return to 'normal life' after cancer and coping with other late effects of treatment. This means that we must understand what their experiences are like, rather than simply relying on what we know from other children with hearing loss. This project, led by Dr Carmen Soto, will interview children and their families about their experience of living with hearing loss after treatment for brain cancer. The findings can then be used to develop better support for the issues that have been identified.

Helping children with cancer make the most of educational opportunities

Prof Faith Gibson, University of Surrey (CCLG Late Effects Grant)

Education for children with cancer may be significantly disrupted both during and after treatment, with long absences possible due to periods in hospital or attending clinics. Going to school helps children feel normal, be more like their friends, and keeps them connected to their peer group. It provides structure and stability when everything else is feeling very different. Returning to school can therefore be a relief for many, a much-needed break from the sick role. But for some, it can also create further worries, whether around fitting back in, looking different and being bullied, or not being able to catch up on lost schooling.

Professor Faith Gibson and her team are undertaking a comprehensive scoping review, looking at what has been published for healthcare and education professionals to help children with cancer 'continue with learning'. The results of this review will be shared with established childhood cancer networks and professionals involved with children's ongoing care together with children, adolescents, and their family members. This will help to raise awareness of the issues and what suggestions are helpful for those in practice, as well as to identify areas for further in-depth research.

Finding out what works well in remotely-delivered acceptance and commitment therapy for childhood brain tumour survivors

Dr Sam Malins and Dr Sophie Thomas, Nottingham University Hospitals (CCLG Late Effects Grant)

Childhood brain tumour survivors have the poorest quality of life of all cancer survivors. There are many late effects of childhood brain tumour treatment on physical and mental health. Patients and carers have identified psychological and social needs as the most important to research and most likely to get missed.

Acceptance and commitment therapy (ACT) is an evidence-based psychological therapy that could improve young brain tumour survivors' physical and mental health. However, we do not know what

young people think about ACT when it is offered remotely, via video-link. This is important, because most health services have increased remote delivery of care and it is likely to remain a way to receive care in future.

Dr Sam Malins' research is collecting information from patients undergoing ACT over video about their experiences to help clinicians learn the best ways of conducting video-therapy for young brain tumour survivors.

“For young brain tumour survivors, making sure that video-therapy is fit-for-purpose from patients' perspectives is a key priority. These findings will be important for developing video-therapy techniques for young people who have had other cancers, too.”



Dr Sam Malins

Solving challenges through innovation

We are exploring new solutions to difficult issues on how best to treat children with cancer. Our researchers are using ground-breaking techniques to think 'outside the box' and to discover new perspectives. Here are some of our research projects:

Assessing whether coloured dye makes surgery more effective

Dr Max Pachtl, Birmingham Children's Hospital (funded by CCLG and CCLG Special Named Funds)

This pioneering study is assessing whether indocyanine green (ICGG) dye can increase the amount of lymph nodes that can be taken out during surgery for kidney cancer, and to see if the whole tumour

can be removed. This research is important because this technique promises to be the most significant change in surgical practice since the introduction of keyhole surgery in the 1970s.

Preventing leukaemia in children

Prof Sir Mel Greaves, The Institute of Cancer Research (funded by CCLG and CCLG Special Named Funds)

Although treatment is now very successful for children with acute leukaemia, the treatment is traumatic and toxic. Preventing leukaemia from occurring would be better.

Prof Sir Mel Greaves' research over three decades has provided robust evidence that common infections may trigger acute lymphoblastic leukaemia (ALL) in children who are susceptible for different reasons, including that they have a deficit of natural germ exposure in their early life.

This study has taken a crucial, early stepping stone in an ambitious, long-term programme to develop a preventative strategy for childhood acute leukaemia. It has developed a mouse model system to test the

possibility that leukaemia may be preventable with exposure to benign or harmless bacteria early in life.

Successful completion of this pilot study has supported the research team to obtain funding for a larger research study to test whether ALL can be prevented through the transplant of gut bacteria. This offers the exciting prospect of a preventative intervention for children that could reduce the risk of the most common form of paediatric cancer.



Prof Mel Greaves

“All parents know that prevention, if possible, would be preferable to treatment.”

Neurosurgically-applied chemotherapy for childhood brain tumours

Dr Ruman Rahman, University of Nottingham (funded by The Little Princess Trust in partnership with CCLG)

There are many challenging issues that need solving to help give children better treatment options, and these require research which approaches developing solutions in a different way. There can be devastating consequences to radiotherapy, which can lead to speech loss and learning delays. In addition, chemotherapy delivered through the blood causes damage to healthy parts of the body. There is therefore an urgent need to develop safer treatments.

Ruman Rahman's pioneering research is about how to get around the challenge of the blood-brain barrier issue to more effectively deliver chemotherapy drugs to the brains of children with tumours. He is developing a biodegradable paste to deliver drugs to the brain immediately after surgery, which bypasses this barrier. The paste is applied to the space created after the tumour has been removed and releases drugs targeting the remaining

cancer cells which surgery cannot remove safely. Applying chemotherapy directly to the tumour site during surgery in a safe manner may potentially deliver high drug concentrations with associated survival benefits, whilst avoiding toxic effects to the rest of the body. Moreover, if this form of treatment is more effective than radiotherapy (or at least comparable), long-term debilitating side effects may be reduced as reduction of radiotherapy may be justified in children diagnosed with medulloblastoma or atypical rhabdoid/teratoid tumours (AT/RT).

The team can identify cancer genes which are switched on when this happens. Using the 3D model, they are testing 80-100 drugs to select those which can stop the identified cancer genes from working properly.



Dr Ruman Rahman

“Growing childhood brain cancer cells in 3D shows how cancer cells ‘talk’ to healthy brain cells. This mimics what occurs after surgery in patients, when the remaining cancer cells use the healthy brain cells to survive.”

Creating an essential framework to categorise brain cancer in teenagers and young adults

Dr Madhumita Dandapani, University of Nottingham (funded by The Little Princess Trust in partnership with CCLG)

This project is forming an essential stepping-stone to define future research that will characterise CNS cancers in teenagers and young adults. Clinical and genetic data of medulloblastoma patients were collected using a reproducible analysis workflow. This research found that teenagers and young adults with medulloblastoma have distinct molecular subgroups that are different from those of adults or

children with the same disease. The framework is designed to collect all publicly available brain cancer data in a convenient format, which allows the team to access and analyse the data to identify patterns to help accelerate the research with teenagers and young adults.

Understanding the evolution of neuroblastoma to improve treatment

Dr Alejandra Bruna, The Institute of Cancer Research (funded by The Little Princess Trust in partnership with CCLG)

One of the key issues with neuroblastoma tumours is that they are clinically very variable. This is one of the reasons that there aren't many successful treatments for neuroblastoma. Existing treatments are generally very toxic and cause long-lasting health issues for survivors.

Drs Alejandra Bruna and Cecilia Roux have combined their expertise to study the evolution of neuroblastoma. By reading the DNA of real-life tumour cells with single-cell sequencing, they planned to see how genetic changes associated with aggressive neuroblastoma led to different responses to treatment.

Using a cutting-edge technique called 'cellular barcoding', they tracked the changes to individual

cells within models of neuroblastoma tumours as resistance to treatment was induced. This was done by slowly increasing the concentrations of treatment to the models until the cells are resistant. These cells were then assessed to see what changes were apparent at each stage of resistance.

The changes in neuroblastoma cells and DNA from both stages will then be used to optimise current neuroblastoma treatments or suggest new targets for drugs. This new knowledge will be shared across the paediatric cancer research community to make treatment more effective and personal.



Dr Alejandra Bruna

“This research is important and can make a difference because current anti-cancer therapies are far from effective in curing the disease. This is mainly because of the intrinsic ability of cancer cells to evolve and adapt to stressors such as treatment exposure.”

Identifying personalised therapy for children with spinal ependymomas

Prof Richard Grundy, University of Nottingham (funded by CCLG and CCLG Special Named Funds)

This research is important and can make a difference because it represents the first effort to characterise spinal ependymoma in children. It underscores the importance of molecular profiling by stratifying paediatric spinal ependymoma patients into two subgroups. The findings and datasets from

this project will set the stage for further research investigating potential therapeutic targets for spinal ependymoma in children.



Prof Richard Grundy

“This data will help build up a profile, or a molecular fingerprint, for the detection of ependymoma at various stages of presentation, treatment and follow-up.”

Developing innovative new treatments for difficult to treat types of medulloblastoma

Prof Steve Clifford, Newcastle University (funded by The Little Princess Trust in partnership with CCLG)

Medulloblastoma is the most common malignant brain tumour in childhood. Over the last 50 years, advances in standard treatments have led to long-term survival rates of approximately 70%. However, medulloblastoma comes back in 30% of patients. This is often driven by the gene 'MYC' and is usually fatal. MYC-driven medulloblastomas account for one in 10 of all childhood cancer deaths, and are therefore an important challenge facing researchers.

To address this challenge, and minimise the chance of resistance to treatment, Prof Steve Clifford plans to combine two different treatment approaches; drugs which indirectly target the MYC gene, and

a type of immune therapy using chimeric antigen receptor T-cells (CARTs), which are also designed to target MYC-driven medulloblastoma.

Already established as treatments individually, the researchers will test them together to track how tumours respond to treatment, identifying potential mechanisms of treatment resistance. This will help progress this novel treatment into clinical trials, where they could help patients with resistant and relapsed medulloblastoma.



Prof Steve Clifford

“MYC-driven’ medulloblastomas account for a high proportion (10%) of all childhood cancer deaths, and therefore present some of the most significant unmet clinical challenges in paediatric oncology.”



Dr Alejandra Bruna and Prof Steven Clifford, recipients of The Little Princess Trust's Innovation Grants for 2021. These grants bring together world-leading teams to use cutting-edge techniques to investigate children's cancer and find new treatments.

“Such ambitious and world-leading research is only possible with the highest calibre of researchers backed by large institutions. With the help of our research partners at CCLG, The Little Princess Trust are proud to have been able to bring all of this together to fund such exciting work.”

Simon Tarplee, LPT's Lead Trustee for Research

How we engage with patients and families

Finding out what is important to young patients, their families and the healthcare professionals who care for them, is vital in making sure research is targeted to their needs.

Children's Cancer Priority Setting Partnership (PSP)

The PSP seeks gaps in research for children's cancer as identified by children, parents, families and professionals. Using a PSP structure is a tried and tested method developed by the James Lind Alliance, a non-profit making initiative. It brings together patients, carers and clinicians on an equal footing to develop a 'Top 10' list of unanswered research questions or topics that need addressing.

The project will report its results by the end of 2022. Uniquely, for a PSP, we are making sure that children's voices are heard by developing an age-appropriate questionnaire and a series of workshops, so that the priorities identified reflect those of children too, not just the adults around them. Find out more at www.childrenscancerpsp.org.uk

CCLG Special Named Funds programme

We support families to raise money for research that matters most to them, which is usually research into the specific type of cancer that affected their child. Families decide to set up a fund for many reasons and it is a valuable way of making a difference for future children affected by cancer. Over the last five years, our Special Named Funds have supported

21 research projects across a range of cancer types, including leukaemia, rhabdomyosarcoma, neuroblastoma, lymphoma and more. With more than 70 family-led funds currently raising money to support research, we can invest in more projects each year, focusing on better treatments, fewer side effects and improving outcomes for all children.

“After losing Toti, we immediately set about raising money in his memory. To start with, we didn't have a focus for our fundraising. CCLG helped us enormously by finding a research project that we cared about and allowed us to feel connected to. Most importantly, we wanted peace of mind that the money raised by The Toti Worboys Fund was being well invested, researching children's leukaemia. It was a huge privilege, comfort and joy to meet Dr Stefan Meyer and his research team at Manchester University.”



Nick Worboys, dad of Toti who died of leukaemia in 2014 and founder of The Toti Worboys Fund, pictured with Toti's family and Dr Meyer's research team

Future horizons

Our vision is to make sure every child diagnosed with cancer will be cured. Since 2016, we have been pushing boundaries and investing in pioneering research and scientific innovations to make this happen. We are proud of what we have achieved and are aiming high for 2022 and beyond by expanding into many different research areas. Here are some of our plans:





Final thoughts



*Prof Andrew Peet
Chair of Research Advisory Group
2015-2020*

I hope you enjoyed reading our first research impact report. Research has always been at the core of CCLG's activities, and we recognised that improvements in the diagnosis, understanding and treatment of children's cancer only occur with a flourishing research programme. In 2015, CCLG created a clear structure and strategy for research, and an advisory group was formed to further develop and drive forward the research agenda.

It was a great privilege for me to be the first Chair of the CCLG Research Advisory Group and see this group flourish. Along with our research funding partners, there is now a large portfolio of research studies funded and managed through CCLG encompassing a whole range of translational studies from laboratory to clinical. At the same time, we are supporting a cohort of new and early career researchers to achieve their ambitions in the field of children's cancer research.

It is therefore with great pleasure that this report brings together some of the fantastic achievements attained by the world-leading researchers associated with this venture. Professor Karim Malik is now Chair and is already bringing new inspiration to the group. I am looking forward to seeing the amazing progress made in finding a cure for children's cancer over the next few years.

“Research is the driving force behind not only improving survival for children with cancer but also minimising the harsh side effects of treatment through kinder treatments. CCLG is fully committed to developing and enhancing research in the UK and was one of our key strategic aims for our future vision. Through a fantastic partnership with the Little Princess Trust, we have exceeded our hopes and are very proud of the phenomenal research impact of our symbiotic relationship with them.”



*Prof Richard Grundy
CCLG Chair*

“CCLG has increasingly been at the forefront of advancing world-class paediatric research. In partnership with other charities, CCLG supports cutting-edge, innovative projects into curing types of children's cancer as well as a wide array of 'bench-to-bedside' studies investigating cancer imaging, early cancer detection, drug discovery, relapse, supportive care and late effects research. The growth of CCLG investment continues to expand. This will enable further pioneering approaches such as immunotherapy and personalised medicine, which hold immense potential for eradicating childhood cancer in the future.”



*Dr Karim Malik
Current Chair of CCLG Research
Advisory Group*

Research Advisory Group

We thank the following experts for their dedication and commitment in ensuring the success of CCLG's research programme in the UK over the last few years:

Chair of CCLG's Research Advisory Group

Professor Andrew Peet (2015-2020)

Professor of Clinical Paediatric Oncology, Institute of Cancer and Genomic Sciences, University of Birmingham

Dr Karim Malik (2021-present)

Professor of Molecular Oncology, University of Bristol

Group members

Professor Adam Glaser

Professor of Paediatric Oncology and Late Effects, Leeds University

Dr Susan Picton

Consultant Paediatric Oncologist, Leeds Children's Hospital

Dr Allison Blair

Principal Clinical Scientists, University of Bristol

Professor Andrew Peet

Professor of Clinical Paediatric Oncology, Institute of Cancer and Genomic Sciences, University of Birmingham

Dr Bob Phillips

Senior Clinical Academic, University of York and Leeds Children's Hospital

Mr Charles Stiller

Epidemiologist, Public Health England

Professor Mehmet T Dorak

Head of School of Life Sciences, Pharmacy and Chemistry, Kingston University, London

Professor Faith Gibson

Professor of Child Health and Cancer Care, University of Surrey and Great Ormond Street Hospital

Dr Jessica Bate

Consultant Paediatric Oncologist, University of Southampton

Professor Ken Mills

Professor of Experimental Haematology, Queens University, Belfast

Dr Marianna Szemes

Research Associate, University of Bristol

Dr Kyle Matchett

Lecturer in Molecular Immunology, University of Ulster

Helen Pearson

Clinical Nurse Specialist, Royal Marsden Hospital

Professor Richard Feltbower

Professor of Epidemiology, University of Leeds

Dr Ramya Ramanujachar

Consultant Paediatric Oncologist, Southampton Children's Hospital

Dr Rebecca Hill

Wolfson Cancer Research Centre, Northern Institute for Cancer Research

Professor Richard Grundy

Professor of Neuro-Oncology and Cancer Biology, University of Nottingham

Dr Timothy Ritzmann

Clinical Lecturer in Paediatric Oncology, Health Education East Midlands

Dr Anestis Tsakiridis

Centre for Stem Cell Biology, University of Sheffield

Funded research 2016-2021

Funded in 2016

- **Anestis Tsakiridis**, University of Sheffield, Award £24,990.00. Defining the cellular origins of neonatal and paediatric brain tumours.
- **Lisa Storer**, University of Nottingham, Award £24,989.00. In vitro evaluation of the potential of glucose restriction as an adjuvant therapy for paediatric brain tumours.
- **Ruman Rahman**, University of Nottingham, Award £24,806.00. Identifying the metabolic 'Achilles heel' of childhood brain cancers.
- **Deborah Tweddle**, Newcastle University, Award £93,074.38. Pre-clinical efficacy and biomarker studies of ALK, MAPK and MDM2-p53 inhibitor combinations in neuroblastoma.
- **Deepali Pal**, Newcastle University, Award £99,991.60. Screening for novel drug combinations in B-cell acute lymphoblastic leukaemia.
- **Vikki Rand**, Newcastle University, Award £99,998.70. Identification of new drug targets to improve treatment options and reduce treatment-related toxicity for children diagnosed with aggressive B-cell non-Hodgkin lymphoma (B-NHL).
- **Ruman Rahman**, University of Nottingham, Award £99,901.00. Brain distribution models to select polymer-delivered drugs for the treatment of childhood brain cancers.
- **Deborah Tweddle**, Newcastle University, Award £37,875.14. Understanding neuroblastoma heterogeneity: genetic studies of circulating neuroblastoma tumour cells.
- **Stefan Meyer**, University of Manchester, Award £97,762.00. Investigations into EVI1 mediated epigenetic modulation in childhood leukaemia.

Funded in 2017

- **Karim Malik**, University of Bristol, Award £94,940.00. In vitro modelling of MYCN-driven poor prognosis Wilms' tumour for assessment of novel therapies.
- **Kathy Pritchard-Jones**, UCL Institute of Child Health, Award £84,998.00. Circulating molecular biomarkers for earlier identification of high-risk Wilms tumour.
- **Ken Mills**, Queen's University Belfast, Award £86,458.00. Repurposing mebendazole and albendazole as novel therapies in paediatric acute myeloid leukaemia.
- **John Paterson Thomson**, University of Edinburgh, Award £9,600.00. Analysis of genome-wide 5-hydroxymethylcytosine patterns in human hepatoblastoma tumours during chemotherapy treatment.
- **Juan Pedro Martinez-Barbera**, UCL Institute of Child Health, Award £10,000.00. Molecular profiling of relapsing craniopharyngioma.
- **Alistair Easton**, University of Southampton, Award £9,855.42. Investigation of ganglioside-specific receptor expression by tumour-infiltrating immune cells.
- **Chris Halsey**, University of Glasgow, Award £9,670.00. Defining the tumour microenvironment in extramedullary acute leukaemia.
- **Maureen O'Sullivan**, Trinity College, Dublin, Award £49,600.00. Unravelling the impact of SMARCB1 loss on the chromatin landscape in malignant rhabdoid tumour to identify novel therapeutic opportunities.
- **Daniel Williamson**, Newcastle University, Award £49,506.00. Improved therapeutic targeting in malignant rhabdoid tumours using discovery proteomics analysis.
- **Sam Behjati**, Wellcome Trust Sanger Institute, Award £63,844.00. Ordering of driver mutations in bilateral Wilms' tumour.
- **Keith Brown**, University of Bristol, Award £99,465.00. Epigenetic deregulation of splicing in childhood renal malignancies.
- **Deborah Tweddle**, Newcastle University, Award £99,999.53. Investigation of the effects of DNA repair inhibitors in pre-clinical models of neuroblastomas with ATM, MYCN and TP53 abnormalities.
- **Chris Halsey**, University of Glasgow, Award £99,993.00. Developing Leukaemic Biomarkers to Enable Personalised CNS-Directed Therapy.
- **Anbarasu Lourdasamy**, University of Nottingham, Award £99,961.00. Comprehensive molecular characterisation of paediatric spinal ependymomas.
- **Andrew Peet**, University of Birmingham, Award £99,027.15. Improving the diagnosis of children's brain tumours by Functional Radiomics.
- **Chris Bacon**, Newcastle University, Award £14,145.44. Histopathology of lymphomas in children with primary immune deficiency.
- **Madhumita Dandapani**, University of Nottingham, Award £24,891.00. Investigating the arginine auxotrophy of paediatric brain tumours.
- **Paul Murray**, University of Birmingham, Award £111,331.97. Targeting GPNMB in Hodgkin Lymphoma.
- **Richard Grundy**, University of Nottingham, Award £111,118.87. Minimal residual disease detection of ependymoma by microRNA biomarker profile evaluation as part of SIOP Ependymoma II trial.
- **Janet Shipley**, The Institute of Cancer Research, Award £98,329.00. High-risk rhabdomyosarcoma patient derived 3D cultures to screen for novel drugs that enhance sensitivity to standard treatment.
- **Deborah Tweddle**, Newcastle University, Award £99,956.80. Characterisation and validation of recurrently mutated genes in relapsed neuroblastoma as targets for novel therapies.

- **Farhana Haque**, University of Nottingham, Award £99,887.30. Molecular pathophysiology of histone G43R mutated childhood brain tumours: towards the development of novel targeted therapies.
- **Faith Gibson**, University of Surrey, Award £63,425.00. Routine and systematic monitoring of symptoms: introducing a system into clinical practice. [Original title: Giving children a voice in healthcare encounters: implementing a multi-platform interactive technology with children who have cancer].
- **Jessica Bate**, University Hospital Southampton NHS Foundation Trust, Award £74,722.00. Evaluation of national advisory panels for childhood cancer.
- **Jessica Morgan**, University of York, Award £25,950.64. Does routine surveillance imaging improve survival after relapsed extra-cranial solid tumours? A systematic review and meta-analysis.
- **Linda Sharp**, Newcastle University, Award £73,923.83. Promoting physical activity in childhood cancer survivors: using qualitative and co-design methods to inform the development of an evidence-based intervention.

Funded in 2018

- **Martina Finetti**, Newcastle University, Award £10,079.00. RNA sequencing to characterise malignant rhabdoid tumours heterogeneity: a pilot study in archival frozen material.
- **Alem Gabriel**, Newcastle University, Award £6,473.35. A pilot study of expression profiling using RNAseq from formalin fixed neuroblastoma and paired diagnostic and relapsed frozen neuroblastomas.
- **Deborah Tweddle**, Newcastle University, Award £123,947.50. A genome wide study of unresectable, MYCN non-amplified, unfavourable histology neuroblastomas in patients > 18 months of age.
- **Karim Malik**, University of Bristol, Award £99,986.00. Overcoming drug resistance for efficacious neuroblastoma therapeutics.
- **Anbarasu Lourdasamy**, University of Nottingham, Award £99,911.31. Teenagers and young adults with primary CNS cancers: a systematic biological characterisation.
- **Helen Bryant**, University of Sheffield, Award £98,111.00. Targeting the Fanconi Anaemia pathway in neuroblastoma.
- **Ken Mills**, Queen's University Belfast, Award £98,712.00. Facing the MuSIC - identification of synergistic repurposed drug combinations as novel therapies in paediatric acute myeloid leukaemia.
- **Matthew Allen**, University of Cambridge, Award £10,000.00. Identifying Molecular Drivers of Disease Progression in Ewing's sarcoma.
- **Suzanne Turner**, University of Cambridge, Award £8,047.00. Investigation of epigenetic mechanisms of tumour growth control in anaplastic large cell lymphoma.

Funded in 2019

- **Ken Mills**, Queen's University Belfast, Award £74,997.00. Identifying Combination Therapies Targeting Apoptosis Pathways in Paediatric AML (CAuSAL study).
- **Mel Greaves**, Institute of Cancer Research, Award £96,810.40. Modelling prophylactic (microbial) prevention of childhood acute lymphoblastic leukaemia.
- **Chris Halsey**, University of Glasgow, Award £98,793.00. Unravelling clinical heterogeneity in Philadelphia positive ALL.
- **Karim Malik**, University of Bristol, Award £99,956.00. Evaluating a novel protein methyltransferase inhibitor for poor-prognosis rhabdomyosarcoma therapy.
- **Kyle Matchett**, University of Ulster, Award £74,933.00. Targeting mutant NRAS in paediatric AML.
- **Yinyin Yuan**, Institute of Cancer Research, Award £98,292.50. Deep learning: An integrated approach to define clinical significance to components of the tumour microenvironment of rhabdomyosarcomas.
- **Francis Mussai**, University of Birmingham, Award £94,060.69. Targeting Myeloid-Derived Suppressor Cells (MDSCs) and Tumour-Associated Macrophages (TAMs) with the anti-CD33 immunotoxin Gemtuzumab ozogamicin to restore anti-cancer immunity.
- **Lisa Russell**, Newcastle University, Award £100,580.00. RNA helicase DDX3X regulates JAK-STAT signalling in acute lymphoblastic leukaemia.
- **Vikki Rand**, Newcastle University, Award £104,545.00. Investigation of potential therapeutic targets in paediatric aggressive B-cell non-Hodgkin lymphoma - towards kinder, more effective treatments.
- **Tanzina Chowdhury & Kathy Pritchard Jones**, Great Ormond Street Hospital, Award £170,366.00. Improving prediction of relapse, treatment delivery and outcomes for children with renal tumours in the UK.
- **Mark Gaze**, University College London Hospitals, Award £82,643.00. The use of proton beam therapy to improve outcomes in childhood abdominal tumours.
- **Madeleine Adams**, Children's Hospital for Wales, Award £71,489.80. Development of a paediatric version of the Sarcoma Assessment Measure (SAM-Paeds): a specific tool for assessing quality of life in children with sarcoma.
- **Paul Huang**, Institute of Cancer Research, Award £80,000.00. Next generation proteomic profiling of extracranial malignant rhabdoid tumours.
- **Deborah Tweddle**, Newcastle University, Award £50,000.00. To improve and modernise the CCLG Tissue Bank Database, to support CCLG biological research.
- **Joan Boyes**, University of Leeds, Award £94,469.00. Towards inhibiting Cut-and-Run: An aberrant V(D)J Recombination Reaction that leads to Lymphoid Cancers.
- **Stuart Smith**, University of Nottingham, Award £95,696.31. Electrotherapy for childhood brain tumours.

- **Susan Burchill**, University of Leeds, Award £111,246.07. Repurposing of drugs targeting drug resistant self-renewing Ewing's sarcoma cells to accelerate new treatments into clinical trials to improve outcomes.
- **Karim Malik**, University of Bristol, Award £98,601.00. Targeting gain of function p53 in poor prognosis Wilms' tumour via histone methyltransferase inhibition.
- **Lucy Donaldson**, University of Nottingham, Award £95,820.30. Can we reduce or eliminate the sensory nerve damage caused by vincristine chemotherapy? Potential novel adjunct neuroprotective/analgesic therapies.
- **Juliet Gray**, University of Southampton, Award £111,236.00. Understanding and improving the mechanism of action of anti-GD2 monoclonal antibody therapy in neuroblastoma.
- **Anbarasu Lourdasamy**, University of Nottingham, Award £99,942.64. Deciphering the New Molecular Landscape in Pediatric Recurrent Ependymoma: Implications for Molecular Targeted Therapy.
- **Amos Burke**, Cambridge University Hospitals NHS Foundation Trust, Award £89,882.94. NIVO-ALCL -Phase II trial of nivolumab for paediatric and adult relapsing/refractory ALK+ anaplastic large cell lymphoma, for evaluation of response in patients with progressive disease (Cohort 1) or as consolidative immunotherapy in patients in complete remission after relapse (Cohort 2)-.
- **Madhumita Dandapani**, University of Nottingham, Award £96,449.07. Exploring alterations in amino acid metabolism as novel therapeutic targets in paediatric glial tumours using advanced metabolomics methods.
- **Julie Irving**, Newcastle University, £96,715.00. Understanding and therapeutically exploiting clonal evolution in chemo-resistant acute lymphoblastic leukaemia.
- **Alexander Thompson**, University of Nottingham, Award £238,793.41. Targeting refractory and dormant stem cells in childhood leukaemia.
- **Andrew Peet**, University of Birmingham, Award £99,959.68. Moving Artificial Intelligence of Functional Imaging for children's tumours into a multi-centre environment through a Clinical Decision Support System.
- **Sam Behjati**, Wellcome Sanger Institute, Award £950,819.77. The Little Princess Trust Wilms Tumour Knowledge Bank.
- **Kathy Pritchard Jones**, UCL Institute of Child Health, Award £479,505.75. The Little Princess Trust Wilms Tumour Knowledge Bank.
- **Anestis Tsakiridis**, University of Sheffield, Award £72,656.00. Establishment of an in vitro model of neuroblastoma initiation using pluripotent stem cell differentiation.
- **Deborah Tweddle**, Newcastle University, Award £149,104.00. Clinical and biological factors associated with relapse and length of survival following relapse in UK neuroblastomas.
- **Francis Mussai**, University of Birmingham, Award £197,574.00. A phase II trial to assess the activity of Gemtuzumab Ozogamicin Therapy in haemophagocytic lymphohistiocytosis (HLH)/Macrophage activation syndrome (MAS) or relapsed/refractory cancers.
- **Ruman Rahman**, University of Nottingham, Award £676,229.33. Neurosurgically-applied chemotherapy for childhood brain tumours arising in the posterior fossa using a biodegradable paste.

Funded in 2020

- **Rob Dineen**, University of Nottingham, Award £149,970.49. Non-invasive identification of clinically relevant ependymoma subgroups; a radiogenomic approach.
- **Suzanne Turner**, University of Cambridge, Award £65,231.00. Dissecting the genetics underlying paediatric Anaplastic Lymphoma Kinase negative (ALK-), Anaplastic Large Cell Lymphoma (ALCL): A European Intergroup for Childhood Non-Hodgkin Lymphoma (EICNHL) biological study.
- **David O'Connor**, University College London, Award £83,299.92. Deciphering the genomic landscape of childhood refractory T-cell Acute Lymphoblastic Leukaemia.
- **Chris Bacon**, Newcastle University, Award £74,582.00. Towards Chemotherapy-Free Treatment of Paediatric Post-Transplant Lymphoproliferative Disorders.
- **Janet Shipley**, Institute of Cancer Research, Award £99,864.00. MG-fnRMS and CINSARC Gene Expression Signatures to Predict Relapse in Fusion Gene Negative Rhabdomyosarcomas: Assessing an Approach to Improve Patient Outcomes.
- **Bob Phillips**, University of York, Award £96,129.79. Understanding treatment decision-making processes in families where a child or young person has relapsed/refractory rhabdomyosarcoma.
- **Max Pachi**, Birmingham Children's Hospital, Award £63,178.39. An open label, single centre, single arm, prospective feasibility study evaluating the effectiveness of near-infrared fluorescence (NIRF) using indo-cyanine green (ICG) in intra-abdominal or intra-thoracic minimally invasive surgery (MIS) in paediatric oncology.
- **Carmen Soto**, Great Ormond Street Hospital, Award £14,954.46. Living with Treatment-Related Hearing Loss: Experiences of Survivors of Childhood Brain Cancer.
- **Sam Malins**, Nottingham University Hospitals, Award £14,065.93. What Helps and What Hinders in Remotely Delivered Acceptance and Commitment Therapy for Survivors of Childhood Brain Tumours: A Diary and Interview Study.
- **Faith Gibson**, University of Surrey, Award £9,844.28. Access to and Experience of Education for Children and Adolescents with Cancer: A Scoping Review Consultation Exercise.
- **Chris Halsey**, University of Glasgow, Award £247,415.00. ALLTogether1 CSF-FLOW Study.
- **Joan Boyes**, University of Leeds, Award £110,775.20. The Role of Cut-and-Run, an Aberrant V(D)J Recombination Reaction, in the Development of Acute Lymphoblastic Leukaemias with Poor Prognosis.
- **Paul Huang**, Institute of Cancer Research, Award £199,372.00. Optimising tyrosine kinase inhibitor therapy in newly diagnosed metastatic Ewing Sarcoma.

- **Laura Donovan**, Institute of Child Health, London, Award £118,148.93. Enhancing CAR T-cell expansion and prolonged persistence for the effective treatment of paediatric medulloblastoma.
- **Dariusz Gorecki**, University of Portsmouth, Award £100,901.00. Investigating repurposed drugs to decrease the progression of Ewing's sarcoma.
- **Agamemnon Grigoriadis**, King's College London, Award £223,173.79. Drug repurposing targeting immunomodulatory Haem oxygenase-1 (HO-1) for prevention of osteosarcoma growth and metastasis.
- **Kyle Matchett**, University of Ulster, Award £216,108.00. Investigating the preclinical efficacy of albendazole in paediatric acute myeloid leukaemia.
- **Rob Ewing**, University of Southampton, Award £34,765.74. Towards a new therapy against childhood brain cancer: How does the Zika virus kill aggressive brain tumour cells?
- **Helen Bryant**, University of Sheffield, Award £196,341.00. Dissecting the role of MYCN in neuroblastoma initiation.
- **Susanne Gatz**, University of Birmingham, Award £103,773.99. Novel biomarkers for PARP inhibitor trials for children with cancer.
- **Jessica Taylor**, University of Cambridge, Award £71,334.49. Repurposing antihistamines to reduce treatment-related toxicity for children with WNTmedulloblastoma.
- **Zoe Walters**, University of Southampton, Award £204,883.92. Evaluating the efficacy of Enhancer of Zeste Homolog 2 (EZH2) inhibitors in combination with antiGD2/isotretinoin for the treatment of high-risk neuroblastoma: a pre-clinical study.
- **Anindita Roy**, University of Oxford, Award £219,194.66. Dissecting the role of CD133/PROM1 in MLL rearranged acute lymphoblastic leukaemia to develop novel targeted therapy.
- **Chris Halsey**, University of Glasgow, Award £184,553.00. Identifying drivers of central nervous system involvement in T-cell acute lymphoblastic leukaemia.
- **Anne-Sophie Darlington**, University of Southampton, £5,429.00. The SHARE Study (How to support children with cancer and their parents during the COVID-19 Outbreak)
- **Bob Phillips**, University of York, £9,831.59. The SHARE Study and Online Hope Programme (COVID-19 support for families)

Funded in 2021

- **Jonathan Bond**, University College Dublin, Award £78,303.03. Defining the molecular landscape of paediatric and adolescent acute leukaemia in Tanzania.
- **Darren Hargrave**, Great Ormond Street Hospital, Award £99,576.27. Development of a multi-factorial prognostic model to optimise treatment decision making and outcomes in paediatric low-grade glioma.
- **Steven Clifford**, Newcastle University Centre for Cancer, Award £1,053,351.30. Developing and delivering small molecule drug and immunotherapy combinations for MYC-driven medulloblastoma: Efficacy, evolution and exploitation.
- **Alejandra Bruna**, The Institute of Cancer Research, Award £499,912.00. Single-cell transcriptomics linked to lineage tracing to interrogate the role of intra-tumour heterogeneity in shaping therapeutic susceptibility and resistance in paediatric cancer.
- **Nick Jones**, University of Swansea, Award £208,532.67. Repurposing gliflozins for T-cell acute lymphoblastic leukaemia therapy.
- **Timothy Ritzmann**, University of Nottingham, Award £50,010.33. Understanding the immune environment in paediatric ependymoma in order to deliver effective immunotherapy and improve patient outcomes.
- **Francis Mussai**, University of Birmingham, Award £190,401.26. Targeting LAT-1 dependent amino acid uptake as a novel therapeutic approach for paediatric AML.
- **Gareth Veal**, Newcastle University, Award £174,580.00. Optimisation of the Treatment of Childhood Cancer Patient Populations through the use of Therapeutic Drug Monitoring.
- **Susan Burchill**, University of Leeds, Award £198,979.45. Integrating multiple data to validate and prioritise lead-hit therapeutic combinations for acceleration into clinical trials to improve outcomes for patients with Ewing sarcoma.
- **John Apps**, University of Birmingham, Award £39,510.00. Biological Response to Novel Treatments in Adamantinomatous Craniopharyngioma and Exploratory Profiling of Cystic Fluid.
- **Robert Wynn**, University of Manchester, Award £86,108.00. Immune priming of donor-derived cord blood T-cells during allogeneic transplant of high risk and refractory leukaemia.
- **Suzanne Turner**, University of Cambridge, Award £249,723.00. Translating the biology of Paediatric B cell Non-Hodgkin Lymphoma to improve the quality of life of children treated for BNHL.
- **Maria Victoria Niklison-Chirou**, University of Bath, Award £104,155.00. Repurposing Lipid Inhibitors for the Treatment of Aggressive Medulloblastoma.
- **Jonathan Fisher**, University College London, Award £244,413.68. Delivering gdT cells for osteosarcoma immunotherapy.
- **Madhumita Dandapani**, University of Nottingham, Award £87,678.73. Developing a biomarker for minimal residual disease in ependymoma.
- **Karim Malik**, University of Bristol, Award £198,991.57. Combined inhibition of autophagy and epigenetics as a novel therapeutic strategy for poor prognosis neuroblastoma.
- **Tariq Enver**, University College London/Cancer Institute, Award £168,381.38. Developing Less Toxic Therapies for Children with Acute Lymphoblastic Leukaemia Through Targeting RUNX1 Addiction.
- **Keith Wheatley**, University of Birmingham, Award £266,998.38. PHITT: Paediatric Hepatic International Tumour Trial.
- **Janet Shipley**, Institute of Cancer Research, Award £99,398.00. Novel Models and New Treatments for Relapsed Rhabdomyosarcoma Patients.

- **Lisa Russell**, Newcastle University, Award £99,988.00 Identifying critical interactions between super-enhancers and proto-oncogenes: driver events in T-cell acute lymphoblastic leukaemia.
- **Henry Mandeville**, The Royal Marsden Hospital, Award £15,000.00 Late severe infections in childhood cancer survivors following splenic irradiation.
- **Chris Halsey**, University of Glasgow, £31,793.00. ALLTogether4Research Scientific Committee Support
- **Katrin Ottersbach**, University of Edinburgh, £11,928.00. Understanding the development of leukaemia in infants – mechanism of lineage switching and the role of the cell-of-origina in MLL-rearranged infant leukaemia – contribution to project funded by Blood Cancer UK

Key

- Funded by CCLG
- Funded by Little Princess Trust in partnership with CCLG
- Funded by Grace Kelly Childhood Cancer Trust in partnership with CCLG
- Funded by CCLG and Neuroblastoma UK
- Funded by The Harley Staples Cancer Trust in partnership with CCLG



Children's
Cancer and
Leukaemia
Group

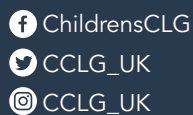
the EXPERTS
in CHILDHOOD
CANCER

amrc

ASSOCIATION OF MEDICAL RESEARCH CHARITIES

Children's Cancer and Leukaemia Group
3rd Floor, Century House
24 De Montfort Street
Leicester LE1 7GB

0333 050 7654
info@cclg.org.uk
www.cclg.org.uk



ChildrensCLG

CCLG_UK

CCLG_UK

© CCLG 2022

Registered charity in England and Wales (1182637)
and Scotland (SC049948).

