action medical research for children

Research Review

Saving and changing children's lives

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Action Medical Research is the leading UK-wide charity dedicated to saving and changing children's lives through medical research.

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"Research leads to improvements in treatments, which means fewer families who are forever incomplete. This is why the work of Action Medical Research is so important"

Louise, mum of Jessica who had a rare heart condition and sadly passed away aged just six

WELCOME

Over the last year, breakthroughs in the fight against COVID-19 have once again shown the enormous difference medical research can make.

Despite ongoing challenges from the pandemic, thanks to our amazing supporters we were able to invest in more new and important research to help save and change children's lives. Research that brings hope to families who are dealing with the trauma of a baby born too early, striving to support a child affected by disability, or facing the challenge of caring for a child with a devastating disease for which there is no cure.

In 2021 we were proud to fund thirteen new projects. These included research into premature birth, rare anaemias, epilepsy and inflammatory bowel disease in children, as well as COVID-19 and long COVID.

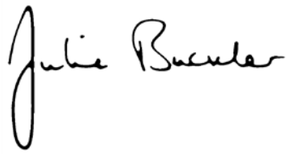
A new report also demonstrated the life-changing impact of previous research funding – helping to develop a new treatment that can reduce seizures in children with drug-resistant focal epilepsy. One of these children, who had been using a wheelchair, was able to walk and run again after receiving the new treatment, and a young boy, who was having up to 200 seizures a day, became completely seizure-free for seven years. A further new report showed how an exciting new technology could be used to treat twin-twin transfusion syndrome during pregnancy.

Other exciting progress made in research funded by our charity included a promising new drug combination for treating T-cell acute lymphoblastic leukaemia. Now included in a new international trial, this could influence the care of patients in the next two to three years.

At Action Medical Research for children we fund groundbreaking research to help find answers that can lead to medical breakthroughs, treatments and cures. The difference for children and their families that we make possible can only happen with the support of so many individuals, trusts, foundations, companies and groups.

Now in 2022, our 70th year of saving and changing lives through medical research, we remain ever grateful to every single one of our supporters, including our network of hardworking volunteers and local committees, as well as those who generously remember Action with gifts in their wills.

Supporting Action makes new research breakthroughs possible. Please help us fight for children's lives.



Julie Buckler
Chief Executive
Action Medical Research for children

RESPONDING TO COVID-19

As we all adapt to living alongside COVID-19, Action continues to fund research to help better understand the impact of the virus on babies and children, and to help children being affected by long COVID.

Over the last two years, life has changed in ways we could never have predicted. Medical research has been heavily relied upon to deliver the life-saving breakthroughs needed to gain control of the virus – and thanks to the rapid development of vaccines and new treatments, most restrictions on public life have now been lifted. But ongoing research remains crucial as we continue to navigate the unknown path of this pandemic.

Here at Action our broad remit, funding research across a wide range of diseases and conditions, allowed us to respond quickly to the new and serious health need as it happened. By late summer 2020 we had launched our COVID-19 children's research appeal and were ready to support vital work in this new area.

We have since then funded seven new research projects focusing on how the virus affects babies, children and young people. Most recently we have begun to support work to help the growing numbers of children affected by long COVID.

There are still many unanswered questions and uncertainties. But the research we are funding now is already giving new insights into the virus and how to best help those most affected by it. This important work could lead to new developments and approaches for the future. It could help to prevent or reduce serious illness and also help inform public health measures, both now and in any future pandemics.

As we celebrate our 70th year as a charity, this work is also a reminder of our heritage. We began when polio was a threat in the 1950s. Decades later we're still here, helping children during the COVID-19 crisis and beyond.

> "The charity has been an absolute lifeline by being agile, responsive and rapid in its approach to funding research during the pandemic"
>
> Dr Nazima Pathan

NEW FINDINGS

Research supported by Action funding, and published in the leading journal *Nature*, has found that children's innate immune response is better at fighting COVID-19. This helps to explain why most are less likely than adults to become seriously ill.

The study, led by Dr Marko Nikolić of University College London and Dr Kerstin Meyer at the Wellcome Sanger Institute, is comparing infection in adults and children across multiple organs.

Initial findings show a stronger, rapid immune response in the airways of children helps to restrict viral replication early on, stopping the virus from invading other parts of the body where the infection is harder to control.

IDENTIFYING RISK FACTORS FOR SEVERE ILLNESS

While most children are not severely affected by COVID-19, some have developed a rare and serious condition in the weeks following infection. Action-funded research aims to understand why and who is at greatest risk.

In April 2020, NHS doctors were told to look out for a rare but dangerous reaction that had struck some children who had previously tested positive for COVID-19. Known as Paediatric Multisystem Inflammatory Syndrome (PIMS), this can occur several weeks after the initial infection.

Action is funding SEVEN projects on COVID-19 in children

Children affected by PIMS can experience a much wider range of symptoms than adults who are seriously ill with COVID-19, suggesting more widespread inflammation.

Some children become critically ill, needing intensive care. Most will recover– but they could be left with lasting problems that affect their long-term health and wellbeing.

Supported by Action, Dr Nazima Pathan's research team aims to identify biological factors that influence a child's risk of severe COVID-19 and long-term complications from the illness.

"Our findings could lead to early, personalised treatment for children who are severely affected, giving them the best chance of a full and rapid recovery," says Dr Pathan.

This work is also expected to uncover new knowledge about why children are generally better protected from COVID-19 compared to adults – and why they have different symptoms.

HELPING CHILDREN LIKE ROSIE AND MAY

Some children, like sisters Rosie and May, suffer ongoing, debilitating symptoms long after an initial COVID-19 infection.

Thankfully, most children and young people infected with SARS-CoV-2, the virus that causes COVID-19, will have a short illness with few or no symptoms. But some are enduring long-lasting problems that affect their physical, mental or social wellbeing, interfering with their daily lives.

Rosie, now 16, and May, 13, first caught the virus in April 2020, just as the world went into lockdown. Two years on, both girls are still living with the effects of long COVID.

Their mum, Mary, believes they have made more than 40 visits to their GP as the family has struggled to find answers and support.

May has been especially affected, missing many months of school due to ongoing illness and extreme fatigue. She suffers terrible headaches that have left her writhing in pain.

Both girls have experienced 'brain fog' and difficulty concentrating. Their mental health and confidence has also been impacted, says Mary, as a result of the stress of being unwell and exhausted for such an extended period of time.

NEW RESEARCH FOR CHILDREN WITH LONG COVID

Action is funding two studies to improve understanding of long COVID in children and develop ways to help.

As long COVID is such a new illness, there are many unanswered questions. It is unclear how and why some children are affected and what the long-term consequences might be, making new research especially crucial.

One possibility is that some symptoms may be caused by ongoing mild inflammation in the brain. So researchers are carrying out brain scans to see if affected children have, or may have had, such inflammation. If these children do have detectable brain changes, it could provide vital new insight into the underlying disease processes, the likely long-term outcomes and how best to aid recovery.

In a second study, another team of researchers are working alongside children with long COVID to develop a new online treatment programme. This aims to reduce the impact of their symptoms, particularly ongoing breathlessness and feelings of anxiety or low mood.

> "As my girls continue to suffer, there is still so much work to be done so that we can understand their illness and give them the treatment they need to recover"
>
> Mary, mum of Rosie and May

FIGHTING FOR TINY LIVES

In 2021, we continued to fund research to help the most vulnerable babies, including new projects to prevent premature birth and to help further improve the care and future development of children who are born too soon.

While advances in treatment have led to improved survival of babies who are born prematurely, these children still face an increased risk of long-term complications.

One new Action-funded study is looking at the importance of natural sleep cycles on the developing brains of babies born too soon. These babies are cared for in an environment quite unlike that of the womb, which may interrupt their sleep cycles and in turn affect brain development at a critical time.

Researchers are testing this theory, with the aim of improving future care in neonatal units. Using a non-invasive imaging system, which is a lightweight flexible cap fitted with sensors, they are studying changes in blood flow in different parts of the brain.

New research is studying sleep cycles in premature babies

STEPS FORWARD

PROGRESS MADE FOR CHILDREN BORN TOO EARLY

Babies born extremely prematurely may be more susceptible to anxiety in later life. With Action funding, researchers have used brain scans to better understand which babies are most at risk.

Babies born before 32 weeks of pregnancy are thought to be nearly twice as likely to have problems with anxiety during their teenage years.

With funding from Action and Dangoor Education, Professor Chiara Nosarti and her team at King's College London have been working to develop a way to identify which children are most likely to be affected.

Their findings have showed that early changes in babies' brains can be used to predict how they may regulate their emotions later in life.

This could allow the most vulnerable children to be identified early and to receive targeted therapies or treatments to protect their mental health as they grow up.

HELPING BABIES LIKE FLETCHER

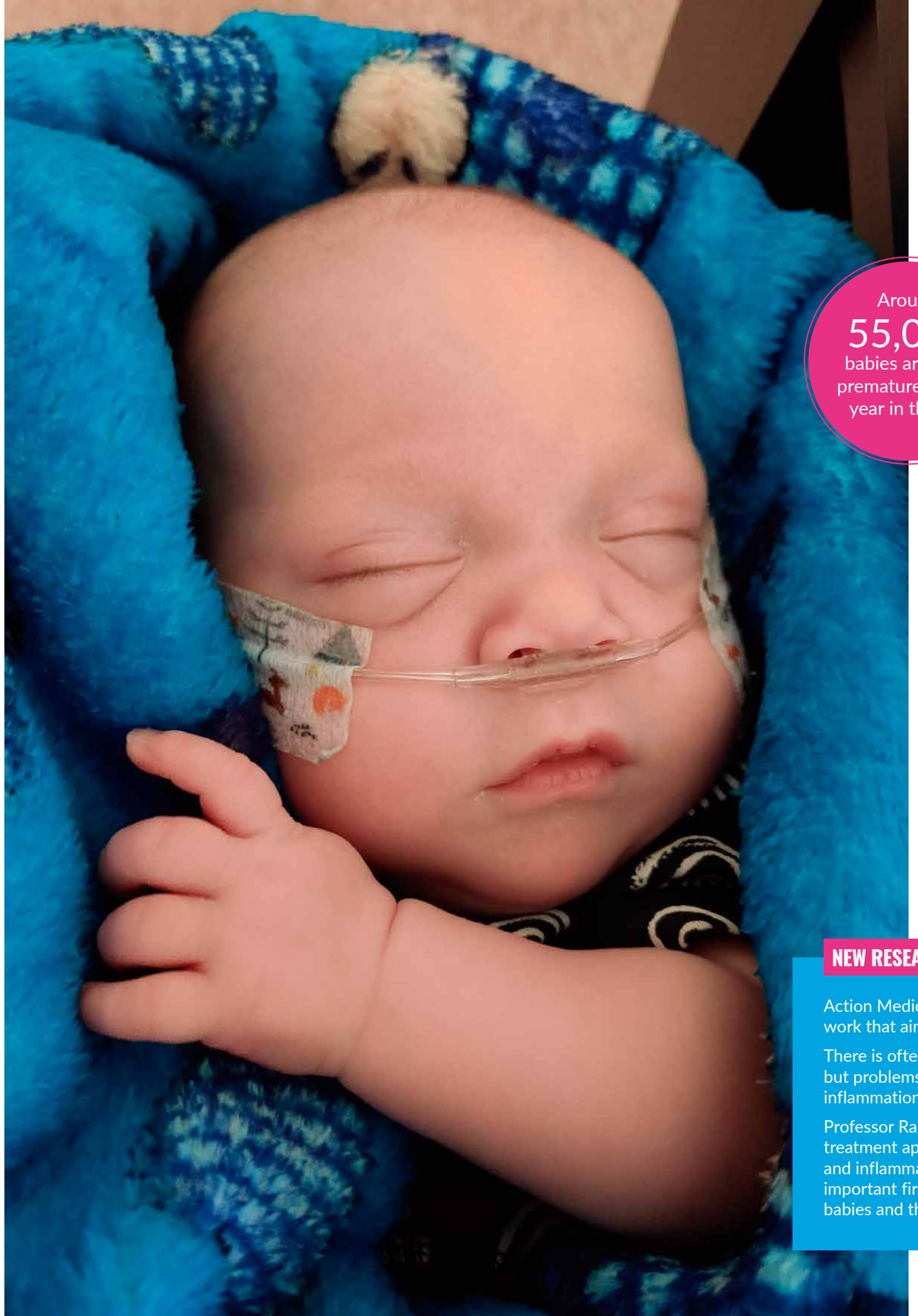
Fletcher was born 16 weeks prematurely. He weighed just 705 grams, less than a bag of sugar, and endured more than 15 weeks of treatment before he was finally allowed home – all in the midst of the first wave of the COVID-19 pandemic.

Fletcher's mum Rosie's pregnancy was exhausting and for much of it she was on bed rest. On a few occasions she experienced early bleeding, but a cause was never determined. She'd also suffered from excessive nausea and vomiting, and severe migraines. Then, just over 24 weeks into the pregnancy, Rosie suddenly haemorrhaged and was taken to hospital, where she was rushed to the delivery suite.

Rosie says that although her labour was incredibly challenging, the paediatric team were brilliant. Consultants regularly visited to explain potential procedures and possible outcomes. But this is also when she and partner Bobby learnt the terrifying reality that their baby might not make it.

Tiny Fletcher was resuscitated at birth and put on ventilation support immediately. He had to be taken straight to the neonatal intensive care unit without a hold or a cuddle with his parents.

> "We wouldn't be here as a happy family if it wasn't for all the research that goes into prematurity and the amazing medical staff"
>
> Fletcher's mum, Rosie



Around **55,000** babies are born prematurely each year in the UK

"When we started our neonatal journey, we were quite naïve to what we'd have to go through," says Rosie. "We were told to expect a rollercoaster, and it was hard to truly understand what our little Fletcher would be subjected to because he was born too early."

Fletcher endured multiple complications, including sepsis, *E.coli* infection, pneumonia, respiratory problems and the life-threatening bowel disease necrotising enterocolitis (NEC). His parents would see babies born at a similar gestation sadly pass away, and there were times when they too felt incredibly fearful for their son's life.

Fletcher was dependant on a ventilator for 57 days and when he was finally discharged from hospital, he still needed oxygen support at home.

"It's been incredibly hard, and we couldn't thank the members of staff at Royal Stoke Hospital enough, they saved our boy's life multiple times," Rosie says.

Today, Rosie is incredibly proud of the progress her son has made. "We are in absolute awe and he continues to amaze us every day – going from a baby you could hold in one hand to this wonderful, chatty, bubbly little boy. But we know that not every family has the same outcome as ours, and the infections Fletcher was lucky to overcome are still taking precious little lives."

NEW RESEARCH

Action Medical Research and Borne are together funding work that aims to prevent premature birth.

There is often no obvious reason for a preterm birth, but problems with the mother's immune system and inflammation or infection may be involved.

Professor Rachel Tribe and her team aim to develop a new treatment approach that involves modifying the mother's immune and inflammatory responses in pregnancy. "This research is an important first step and could lead to happier outcomes for many babies and their families," says Professor Tribe.

RESEARCH FOR BABIES WITH HEART CONDITIONS

Congenital heart disease means a heart condition or defect that develops in the womb, before a baby is born. New research aims to help babies with one such condition, coarctation of the aorta.

Each year, more than 200 UK babies are born with coarctation of the aorta. This means that their aorta, the main artery, is narrower than usual. In severe cases, this can restrict blood flow, cause heart failure and damage other vital organs.

The condition cannot be confirmed until after a baby is born and diagnosis can currently take up to a week.

Advanced MRI scans could change this, allowing earlier treatment and reducing the anxious wait faced by parents.

Action funding is supporting detailed MRI assessments of babies' blood flow to identify measurements that could help diagnose the condition sooner.

Each day in the UK **13 babies** are diagnosed with congenital heart disease

STEPS FORWARD

PREVENTING BRAIN DAMAGE IN BABIES WITH CONGENITAL HEART DISEASE

Up to half of all children born with a heart condition also experience problems with learning and development. Action-funded research used specialist brain scans to look at the causes and further work is now testing potential new treatments.

Thanks to advances in early diagnosis and treatments, the majority of children born with congenital heart disease now survive into adulthood. But many experience neurodevelopmental problems as they grow up.

Professor Serena Counsell and her team at King's College London found that lower levels of oxygen delivered to the brains of babies and children with congenital heart disease are associated with less grey matter and poorer cognitive performance.

Further investigations into treatments to protect babies from brain damage are now underway and will use findings from this research to assess how well the potential treatments work.

> "Our ultimate aim is to reduce the long-term consequences of brain injury in babies with congenital heart disease"
>
> Professor Serena Counsell

FIGHTING RARE DISEASES

Thousands of UK children are living with a rare disease for which there is no cure or very few treatment options. Action is funding research that has the potential to unlock vital medical breakthroughs for these forgotten families.

New research funded in 2021 is searching for much-needed new treatments for children with a rare and very severe form of epilepsy.

Children with *KCNT1*-related epilepsy suffer severe seizures that usually begin in the first days or months of life. Many will also have delayed development, learning difficulties, behavioural or movement problems. Some will never learn to walk or talk and sadly many lose their lives before adulthood, like Josie who is pictured.

Researchers are striving to identify chemical compounds that could form the basis of new treatments. Ultimately this could lead to new drugs to control seizures and prevent brain damage, transforming the lives of children and families affected by these devastating conditions.

New research aims to find new drug treatments for a very rare and disabling form of childhood epilepsy

STEPS FORWARD

IMPROVING TREATMENT FOR CHILDHOOD LEUKAEMIA

Researchers have moved a step closer to developing a new treatment option for T-cell acute lymphoblastic leukaemia.

Around 100 children and young people are diagnosed with this fast-developing form of leukaemia each year in the UK. Thankfully, most can be cured. But for those where the disease returns or who don't respond to existing treatments, the outlook is sadly much less positive.

With Action funding, Dr Frederik van Delft, of Newcastle University, has been investigating whether combining two existing drugs could offer a new treatment.

Excitingly, based on this research, and similar results from other research groups, this new drug combination has now been included in an international trial due to start in the UK this year.

We are very grateful to the Team Lewis Trust and other charitable trusts for supporting this project.

HELPING CHILDREN LIKE FINLEY

Finley has Diamond-Blackfan anaemia, a very rare condition for which there is currently no cure. In his first four years of life, Finley has endured more than 100 blood transfusions, countless tests and two major operations.

"Finley is completely unique and he has taught us and his siblings so much about life," says his mum Ellie. "We didn't realise when we named him, but Finley means warrior and that he truly is. Every single task is a battle for him, but he never ever shows it. He absolutely loves life and laughs every single day."

Currently, there are over **7,000** known rare diseases

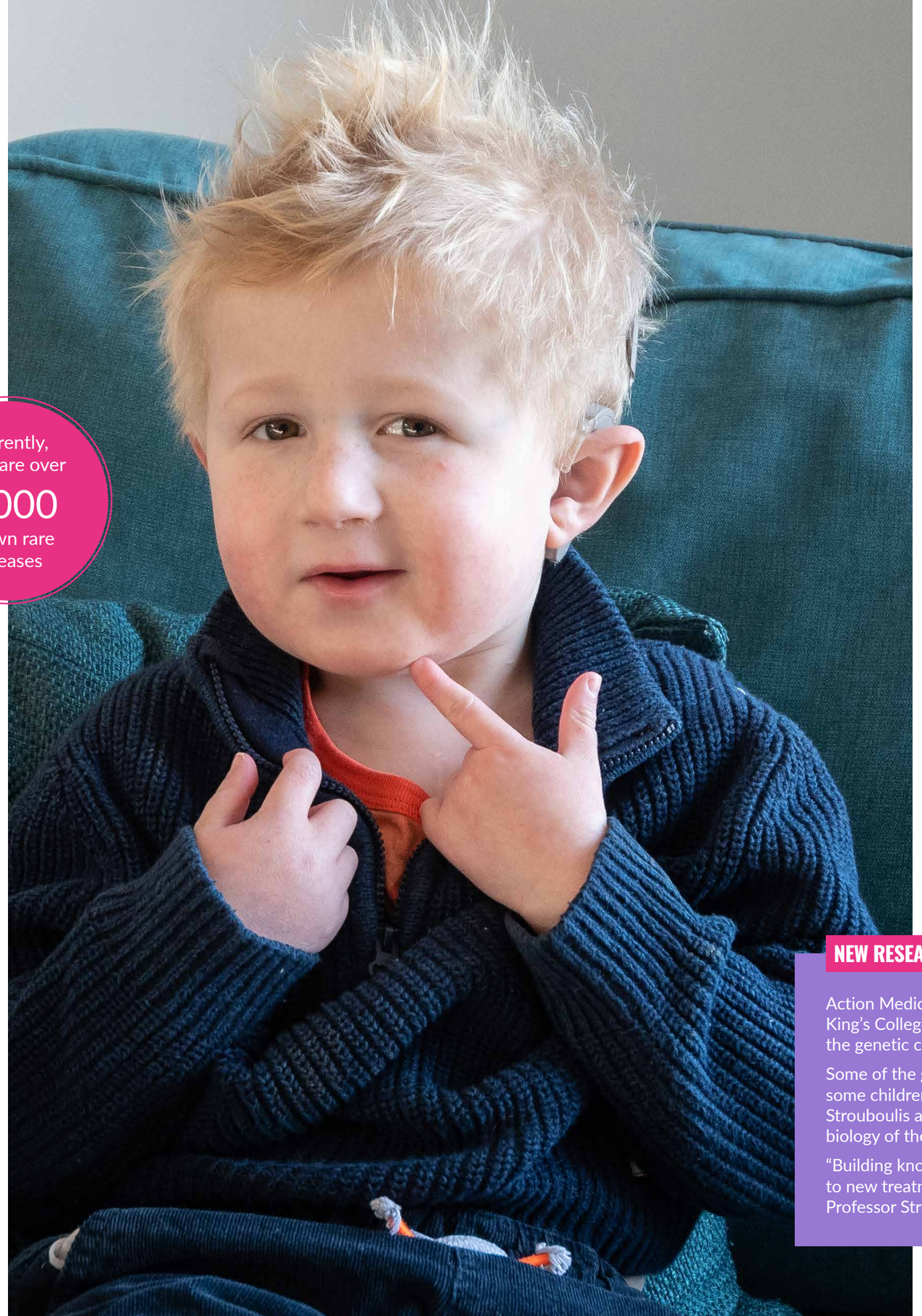
Finley has been fighting since the day he was born. At birth, his oxygen levels were dangerously low, and tests found that he was severely anaemic. He needed three blood transfusions in his first 24 hours of life.

While in special care, baby Finley was also found to have a serious heart condition, and a scan showed enlarged ventricles in his brain. His family were warned that this could cause developmental delay. Then, at six weeks old, he was diagnosed with profound hearing loss.

"We found ourselves on a conveyor belt of poor prognosis, and lived on a constant knife-edge," recalls Ellie.

At three months old, Finley was finally diagnosed with Diamond-Blackfan anaemia (DBA), an extremely rare condition where the bone marrow fails to produce enough red blood cells. It is caused by changes in certain genes and often causes other physical problems – for Finley, his heart condition and hearing loss are linked.

Finley had cochlear implants fitted at 13 months old, to allow him to perceive sound. This was followed by open heart surgery when he was three. He has also fought meningitis twice.



> "Because DBA is so rare, research funding and opportunities are limited. A cure is our dream"
>
> Finley's mum, Ellie

Finley's current treatment regime sees him have monthly blood tests, followed by a four-hour blood transfusion the next day.

The most difficult aspect of his transfusions is managing the iron overload they cause. Left untreated, this causes organ failure. So he takes daily tablets to remove excess iron – but these too cause side effects. His care is a constant delicate balance.

Finley is also at greater risk of developing certain cancers, especially leukaemias, so this too is monitored.

Looking to the future, Finley could need a bone marrow transplant if his current treatment stops working. His family also hope that gene therapy might one day be another option to help him. "Without hope, we wouldn't be where we are today," says Ellie.

NEW RESEARCH

Action Medical Research is currently funding a team based at King's College London, who are working to better understand the genetic causes of Diamond-Blackfan anaemia.

Some of the genes responsible are already known but for some children there is currently no explanation. Professor John Strouboulis and his team aim to improve understanding of the biology of the disease and identify further genes that cause it.

"Building knowledge about the underlying causes could lead to new treatments and improved diagnosis for children," says Professor Strouboulis.

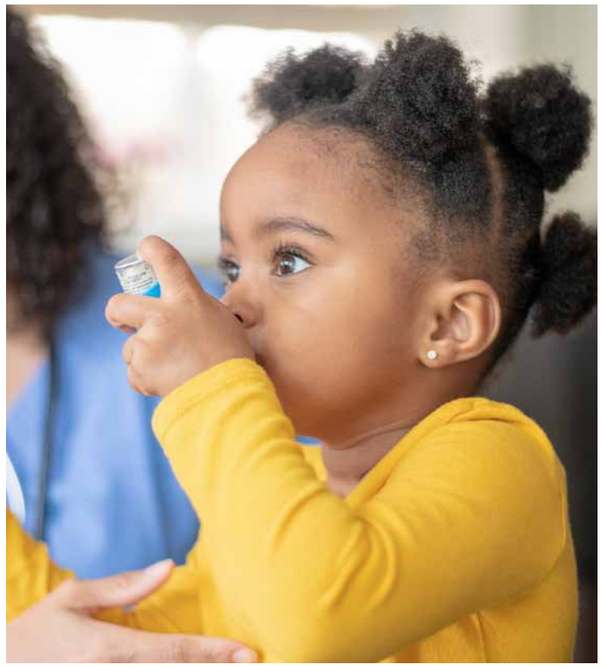
FIGHTING CHRONIC CONDITIONS

Many chronic conditions develop during childhood. Most cannot be cured, only controlled, and affect quality of life. Action is funding research to help children coping with long-term conditions such as asthma and inflammatory bowel diseases.

Asthma is the most common long-term condition among children and young people in the UK. Around 1.1 million are affected and it continues to be one of the top causes of emergency hospital admission.

Supported by Action, new research aims to design a computer-based tool to predict which pre-school children who experience severe wheezing are most likely to go on to develop asthma. While many outgrow these symptoms, approximately one in three will have asthma by school age.

"We hope this work will ultimately lead to targeted interventions that can help prevent the condition from developing in children most at risk, reducing the lifelong impact on their lung health," says Professor Sejal Saglani, who is leading the research at Imperial College London.



New research aims to develop a tool to predict which young children are at greatest risk of developing asthma

STEPS FORWARD

UNDERSTANDING CAUSES OF CROHN'S DISEASE

Action Research Training Fellow Dr James Ashton has developed genetic testing which could help diagnose and treat more children in the future.

Crohn's is the most common form of inflammatory bowel disease in children and sadly rates are increasing. Inflammation in the gut causes the symptoms, including diarrhoea, abdominal pain and tiredness, but it is unclear what triggers this.

Dr Ashton has analysed how patients' genes, the immune system and gut bacteria work and interact together. He identified specific and new genetic changes in some affected children. Some of these changes were linked to specific symptoms and disease complications.

"We believe that some of our findings could help in the diagnosis and treatment of more children with Crohn's disease in the near future – enabling doctors to use genetic information to personalise care for each patient," he says.

HELPING CHILDREN LIKE GEORGIA

Georgia was diagnosed with Crohn's disease when she was just two years old. Thankfully medication has worked well but as the condition can be unpredictable, she could face further flare-ups in the future.

Georgia was a healthy baby but at around 12 months old, she began to suffer ongoing diarrhoea and developed a severe and recurrent rash.

After becoming very unwell with a high temperature, Georgia was admitted to hospital. Tests to establish the cause of the rash were arranged, with inflammatory bowel disease mentioned as a possible cause.

Little Georgia endured two invasive procedures under general anaesthetic – an endoscopy and a gastroscopy, where a thin, flexible tube with a tiny camera on the end is used. These, and a biopsy, confirmed she had Crohn's disease.

Doctors immediately prescribed a course of steroid tablets to help control her symptoms. After that, she moved onto a daily medicine designed to soothe the digestive system.

Thankfully now, four years on, the condition has little impact on her daily life. But she will continue to need to be monitored as she grows up. "I'm optimistic but the future is uncertain," says her mum, Laura. "I have tried to press doctors for an answer as to what to expect but nobody can really say. That's why research is so important."

> "It's great to know that work like this is happening"
>
> Georgia's mum, Laura

More than **500,000** people in the UK have Crohn's or colitis

NEW RESEARCH

Action Medical Research is funding work to develop a simple new blood test to improve the diagnosis of inflammatory bowel disease and help select the best treatment for each child.

"We aim to develop a new test that can improve diagnosis and personalise treatment for children – improving outcomes, reducing invasive investigations and unnecessary treatment," says lead researcher Professor Jack Satsangi.

The team, at the University of Oxford, has previously discovered specific changes in a child's DNA that can tell very accurately if they have inflammatory bowel disease. They now plan to use cutting-edge technology to create a test that can rapidly examine these and other genetic changes linked with the condition.

TRANSFORMING LIVES FOR CHILDREN WITH EPILEPSY

With Action funding, researchers have developed a life-changing new treatment to help children with uncontrolled epilepsy.

Epilepsy causes frequent seizures, which occur when there is a sudden burst of intense electrical activity in the brain. These can be very unpredictable and frightening for affected children and their families.

For most, the condition can be controlled with medication, but for around one third of children the usual treatments do not work.

Living with uncontrolled epilepsy can be a real struggle, affecting children's mental health, wellbeing and performance at school, as well as their social life and activities. It can seriously disrupt and even endanger lives, so new treatment options are urgently needed.

A NEW TECHNIQUE

Action funding, awarded in 2015, has helped Dr Antonio Valentin and his team at King's College London to develop a new way to treat focal epilepsy, which originates in localised areas of the brain. It involves suppressing the area triggering the seizures by stimulating very specific parts of the brain, using electrodes placed under the skull.

The technique, called sub-acute cortical stimulation, has been shown to reduce children's seizures and can result in longer, completely seizure-free periods for some.

> "Some children have had a phenomenal transformation"
>
> Dr Antonio Valentin

1 in 3
children with epilepsy find their condition cannot be controlled with drugs

It is also less invasive than surgical treatments, meaning it is safer and may be used in situations where surgery is not possible.

Already 16 children have benefited from the new treatment and a larger study, using a new device developed as a result of this work, is planned for this year.

"Some children have had a phenomenal transformation," says Dr Valentin. "One, who had been using a wheelchair, was able to walk and run again."

It is estimated that in the near future up to 30 children a year could be treated using the new stimulation techniques, reducing their seizures and improving their quality of life.

"Without Action funding, we couldn't have got this far," says Dr Valentin. "We have patients waiting who could benefit from these new treatments and the Action-funded research has led to not only ideas, but the ability to take this further."

This research was jointly funded with Great Ormond Street Hospital Children's Charity. We are also grateful to the Alison Hillman and the Anne & John Walters charitable trusts for their support of this work and the epilepsy project on page 11.

SEIZURE FREE FOR SEVEN YEARS

Danny (left) was one of the first children to be treated with sub-acute cortical stimulation, with life-changing results.

Danny was diagnosed with severe epilepsy as a baby and over time various medications were no longer helping. By the age of seven, his seizures were almost constant and he couldn't live a normal life.

"Nothing was working. It just seemed to be getting worse. It was really scary and upsetting," say his parents, Jon and Jane.

As a last resort, the family agreed to brain surgery. However, when the procedure began, it became clear that Danny's epilepsy involved too large an area to safely operate.

He was then one of the first to be offered electrical stimulation and it worked better than anyone had dared hope. For seven years his seizures stopped completely.

"We are forever grateful and pray that the treatment continues to develop, so it can go on to help as many others as possible," says Jon.

> "If Danny had not received the stimulation treatment, he would not be the boy he is today"
>
> Danny's dad, Jon

TAKING ACTION FOR 70 YEARS

We've been funding medical breakthroughs since we began in 1952 and have funded research that has helped save thousands of children's lives and changed many more. Please help us fund more research taking small steps, making big breakthroughs.

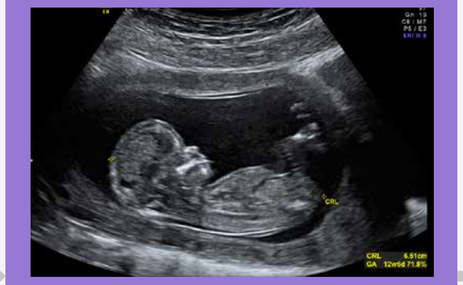
SAVING LIVES FOR
70 YEARS

1952

Action is set up by Duncan Guthrie to help fund research into polio and develop the first UK vaccines

1960s

Discovering the importance of folic acid in pregnancy



1970s

Helping introduce ultrasound scanning in pregnancy

1980s

Creating the award-winning Matrix seating system for disabled children

1990s

Establishing a vaccine to prevent meningitis

2000s

Helping to develop cooling therapy to prevent brain damage in babies

2010s

Developing new advice to reduce the risk of stillbirth leading to a Sleep on Side public health campaign

2020s

Helping to bring dedicated eyecare services to children in special schools across the UK

RESEARCH GRANTS AWARDED IN 2021

Action Medical Research is currently funding around 55 projects across the UK, 13 of them awarded in 2021. **The next medical breakthrough could be on your doorstep.**

Asthma – predicting which pre-school children with wheezing will go on to develop the condition later in childhood
Professor Sejal Saglani
Imperial College London and Royal Brompton Hospital, London

Anaemia – understanding the causes of a rare inherited anaemia to improve its diagnosis and treatment
Dr Chris Babbs
University of Oxford, John Radcliffe Hospital

COVID-19 – exploring the impact of the pandemic on the diagnosis, treatment and outcomes of children with brain tumours
Dr Ibrahim Jalloh
Cambridge University Hospitals NHS Foundation Trust, Lancaster University, University of Nottingham, University of Cambridge

COVID-19 – identifying risk factors for severe disease or long-term complications in children
Dr Nazima Pathan
University of Cambridge, Addenbrookes Hospital, Wellcome Sanger Institute, UCL Great Ormond Street Hospital Institute of Child Health, University Hospital Birmingham, University of Birmingham and Birmingham Children's Hospital, University College London

Diagnosing a heart condition earlier so newborn babies can be treated faster
Dr Malenka M Bissell
University of Leeds, Leeds General Infirmary, King's College London, Evelina Children's Hospital

Diamond-Blackfan anaemia – understanding the causes to improve diagnosis and treatment of children with the condition
Professor John Strouboulis
King's College London

Epilepsy – searching for a new treatment for a group of rare childhood epilepsy syndromes
Dr Jonathan D Lippiat
University of Leeds

Inflammatory bowel disease – developing a new blood test to improve diagnosis and personalise treatment for children
Professor Jack Satsangi
University of Oxford, John Radcliffe Hospital, University of Southampton, Southampton General Hospital, University of Edinburgh

Long COVID – developing a new online treatment programme to address breathing difficulties and mental health problems in children and young people
Dr Samatha Sonnappa
Royal Brompton Hospital, London, University College London Hospitals NHS Foundation Trust, Imperial College Healthcare NHS Trust, Evelina London Children's Hospital, Guy's and St Thomas' NHS Trust

Long COVID – understanding how COVID-19 affects the brains of children with persistent symptoms
Dr Nathalie E MacDermott
King's College London, Evelina London Children's Hospital, Guy's and St Thomas' NHS Trust, University College London and Great Ormond Street NHS Trust

Preterm birth – developing immunotherapy to prevent spontaneous preterm birth
Professor Rachel M Tribe
King's College London, Guy's Hospital

Preterm birth – investigating the importance of sleep cycles on brain development
Professor Topun Austin
Cambridge University Hospitals NHS Foundation Trust, University College London

Vasculitis – developing a new gene therapy for a rare inherited type of blood vessel inflammation
Professor Despina Eleftheriou
UCL Great Ormond Street Institute of Child Health, UCL Royal Free Hospital Campus, University College London

Locations of current research funded by Action

FIND OUT MORE

These are just some of the research projects we are currently funding. Ongoing work also includes:

- Improving care in pregnancy and for sick babies
- Preventing life-threatening infections like RSV
- Helping children with disabilities, such as cerebral palsy
- Developing potential new treatments for devastating rare diseases such as Hunter syndrome and Duchenne muscular dystrophy.

Scan the QR code to find out more.

SUMMARISED FINANCIAL STATEMENTS

for year ended 31 December 2021

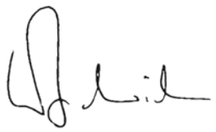
Report by the trustees on the summarised financial statements

The summarised financial statements below are extracted from the full trustees' annual report and financial statements, which were approved by the trustees and signed on their behalf on 28 April 2022. The full financial statements, on which the auditor, Buzzacott LLP, gave an unqualified audit report on 30 May 2022, was submitted to the relevant statutory bodies, including the registrar of companies, on 17 June 2022.

The auditor has confirmed to the trustees that, in their opinion, the summarised financial statements are consistent with the full financial statements for the year ended 31 December 2021.

These summarised financial statements may not contain sufficient information to gain a complete understanding of the financial affairs of the charity. The full trustees' report, financial statements and auditor's report may be obtained from Kabba Njie, Vincent House, Horsham, West Sussex RH12 2DP.

Signed on behalf of the trustees



Luke Bordewich Chair

Income and expenditure	2021	2020
	£000s	£000s
Net incoming resources		
Donations and legacies	1,899	1,417
Investments	(11)	(75)
Other income	4	152
Total net incoming resources	1,892	1,494
Outgoing resources		
Medical research projects	2,114	1,912
Medical dissemination	669	676
Net outgoing resources	(891)	(1,094)
Net (losses) gain on investments	1,051	1,691
Net movements in funds	**160**	**597**

Balance sheet	31 Dec 21	31 Dec 20
	£000s	£000s
Fixed assets		
Tangible	302	295
Investments	12,496	11,952
Total fixed assets	12,798	12,247
Current assets	1,186	1,520
Current liabilities within one year	(5,530)	(5,237)
Liabilities falling due after one year	(1,650)	(1,886)
Total net assets	6,804	6,644
Representing:		
Unrestricted funds	6,804	6,644
Restricted funds	0	0
Total funds	**6,804**	**6,644**

THANK YOU

We are always hugely grateful to the many individuals, companies, trusts and foundations who so generously donate to the vital work supported by Action Medical Research. In 2021, as we continued to negotiate the impact of the COVID-19 pandemic, this support was more important than ever.

We would also like to express our thanks to the organisations listed for their contributions and involvement with the charity.

Thank you too to those who have made provision for Action in their will and to those who left us a legacy gift in 2021.

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Milton Damerel Trust
Next Retail
Oso Foundation
Peter Sowerby Foundation
Reed Foundation
Rouleur
Satchel
TSML
Willmott Dixon
The Worshipful Company of Grocers

Legacy gifts

Dr Sebastian Lazareno
Edna Pigg
Janet Chisholm
Ronald Wright
Lilian Cooper
Marilyn Taylor

If you would like to find out more about leaving a gift in your will to Action, please contact Gill on **T 01403 327413** **E legacy@action.org.uk** or visit **action.org.uk/giw**

WHO'S WHO 2021

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President

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Dr Adam Shortland BSc PhD MIPEM CSci
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Professor Michael Taggart BSc PhD

Scientific Advisory Panel for joint Action Medical Research/LifeArc project grant applications on rare disease research

Professor David Rowitch MD PhD ScD, Chair
Professor Claire Booth MBBS MSc PhD MRCPCH
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Professor Yanick Crow MBBS MRCP CCST PhD FMedSci
Professor Alicia El Haj PhD FREng, FRSB, FEAMBES
Dr Thorsten Forster PhD – joined July 2021
Professor David Gray PhD
Professor Jonathan Grigg BSc MBBS MD MRCP FRCPCH
Professor Catherine Hawrylowicz PhD
Professor Andrea Nemeth BSc MBBS DPhil (Oxon) FRCP
Professor Stephanie Schorge PhD
Dr Adam Shortland BSc PhD MIPEM CSci
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Thank you

A member of the:

amrc
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