action medical research for children

Research Review 2019

Saving and changing children's lives

Welcome

At Action Medical Research for children we fund groundbreaking research to help find answers. Answers that can lead to cures, treatments and medical breakthroughs. Answers that save lives.

In 2019 we funded 14 new research projects, including into traumatic brain injury, childhood leukaemia and cerebral palsy. Today we are proud to have £9.5m of research underway across the UK, involving more than 230 researchers across almost 60 projects.

With our BORN TOO SOON campaign shining a spotlight on premature birth, we funded new research to help preterm babies get the best treatments to combat infection.

We also worked in partnership with other charities, launching a relationship with LifeArc to develop treatments for children with rare diseases, funding our first two research projects together.

We reported the impact made by our research through new advice to help reduce stillbirth. A second report showed the difference our funding is making to support children born very prematurely in their education.

Exciting progress included a new technique to treat epilepsy in children. Then a huge step was made towards more personalised treatment for children with brain tumours. And a clinical trial is planned for next year with the hope of developing a cure for boys with the rare condition XLP.

Such fantastic results for children and their families are only possible with the support of many individuals, groups, trusts and companies. We are grateful to everyone in our community, including those who generously remember Action with gifts in their wills, as well as our network of hardworking committees and volunteers.

With the emerging global COVID-19 health crisis, medical research is now more important than ever. By joining with us you make possible more research that could make such a difference. Please help us fight for children's lives.

Julie Buckler
Chief Executive Action Medical Research

14
new projects funded in 2019

Supporting more than **230** researchers

£280
can fund a pioneering research project for one day

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

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England and Wales no. 208701; Scotland no. SC039284

Powering vital research to save little lives

By the end of 2019, our BORN TOO SOON campaign, which shines a spotlight on the devastation caused by premature birth, had raised more than £400,000 towards vital research that could in the future help families like Wilfred's.

Born extremely prematurely, Wilfred was so very delicate that his parents were unable to hold or even touch him for his first three weeks of life. In his earliest days, they feared they would lose him.

Imogen's pregnancy had been progressing smoothly until labour pains started suddenly one night, more than three months too soon. Imogen and husband Euan went straight to their local hospital, where it soon became clear that baby Wilfred was on his way – despite medics' best attempts to try and delay her labour.

Tiny Wilfred was born weighing just 2lb 6oz and within hours transferred to another hospital where staff were more able to care for babies born so early. Sadly, many weeks of anxiety were to follow for Imogen and Euan.

Wilfred was tube fed and needed a ventilator to help him breathe. Despite this, he was initially considered to be doing well. However, tests soon found that he had suffered a bleed into his brain – and there was no way of knowing how badly he might be affected in the future.

Doctors then feared that a potentially deadly bowel disease, necrotising enterocolitis (NEC), had taken hold. So, at just six days old, Wilfred endured surgery to have 25cm of his bowel removed and a stoma bag fitted to manage his body's waste.

More than
1,000
babies die each year in the UK as a result of being born too soon

It was a deeply distressing time for the new family. "I cried a lot," says Imogen quietly. "We felt so helpless."

Wilfred stayed in hospital, more than an hour away from home, for four months before finally, following a successful operation to reverse his stoma, he was ready to go home.

> "IT WAS AWFUL – FIRST WE THOUGHT HE WOULD DIE AT BIRTH, THEN HE WAS WELL, AND THEN HE GOT NEC. WE FELT SO HELPLESS." **Wilfred's mum, Imogen**

Now a busy toddler, Wilfred recently turned three. He has been diagnosed with cerebral palsy but thankfully is mildly affected, leaving his left side a little weak, meaning he needs to wear a splint on his left leg. He is also a little behind his peers in terms of speech and vocabulary, but very chatty: "He's a happy, determined little character and takes everything in his stride," says his proud mum.

For Imogen, the experience of premature birth left her with many questions. During her second pregnancy, with Wilfred's sister Edith, Imogen finally got some answers. But despite treatment to try and prevent another early delivery, her waters still broke at 33 weeks, meaning Edith was also born prematurely.

Imogen is in no doubt that research to prevent premature birth is vitally important, so that other families might be spared the experiences hers has been through.

BORN TOO SOON

New research to prevent premature labour

Action is funding research that aims to develop more effective treatments that could safely stop premature contractions, reducing the risk of early birth.

Although the causes of premature birth are complex, it often occurs when the mother goes into spontaneous early labour. Unfortunately, there are no treatments currently available that can reliably prevent premature contractions while being risk-free for mother and baby. Many drugs that work to reduce contractions may also relax blood vessels and affect blood flow to the womb or placenta, endangering the baby.

With Action funding, a team of researchers at Newcastle University is studying new ways to specifically target just the muscles of the womb, without affecting its blood vessels or those of the placenta.

This research has been jointly funded as part of our partnership with Borne.

> "THIS WORK IS REALLY IMPORTANT – TO KNOW THAT RESEARCH IS TRYING TO ENSURE THAT PEOPLE DON'T HAVE TO GO THROUGH PREMATURE BIRTH."
>
> **Wilfred's mum, Imogen**

Helping babies fight infection

Bacterial infections are especially dangerous for premature babies. It's hoped new research will lead to life-saving new tests and better treatment.

Sadly, many premature babies lose their lives due to a severe bacterial infection, and those who do survive repeated infections can be left with life-changing disabilities.

Around **60,000** babies are born prematurely each year in the UK

With Action funding, Dr Deena Gibbons and her team at King's College London aim to improve understanding of how the immune system of premature babies reacts to these infections, and develop new ways to improve outcomes.

Normal tests are more difficult to interpret in premature babies, making confirming infection harder than usual. At the same time, doctors need to be very cautious, meaning some babies may receive antibiotics when they might not need them, or be treated for longer than necessary. This is not ideal – antibiotics, though life-saving when needed, can cause other serious complications in newborn babies. Plus, unnecessary use could fuel the development of resistant strains of bacteria in the future.

This research team will study blood samples taken from premature babies, hoping to identify specific features that could be used to aid diagnosis and treatment.

"Ultimately, we hope this will lead to new tests that can help identify babies who may be at higher risk of developing a severe infection so that steps can be taken to help protect them in the critical, first few weeks of life," says Dr Gibbons.

MAKING AN IMPACT

Supporting premature children at school

We're delighted to have funded the development of new online resources for teachers, designed to help them better understand and support children who were born too soon.

Children born early are known to be more likely to experience learning difficulties or special educational needs as they grow up. But research carried out by Professor Samantha Johnson and her team at the University of Leicester found that many teachers were not aware of the issues these pupils may face and had limited training on how they could help.

While children born extremely prematurely, before 28 weeks of pregnancy, are most likely to need extra support, those born just a few weeks early can still face difficulties. "Many might have subtle cognitive, social or emotional difficulties that can be quite difficult to detect in the classroom," explains Professor Johnson.

With Action funding awarded in 2015, Professor Johnson has developed an interactive e-learning resource, working with colleagues from five universities.

It explains the impact preterm birth can have on a child's development and learning, and includes practical strategies that can be used to give support. When tested by teachers, 97% said they would recommend it to others.

This has the potential to make a difference to thousands of young lives and is freely available to download at **pretermbirth.info**

Jago's story

The resource is one that Jago's mum, Georgie, feels is 'invaluable'. Jago, now seven, was born 15 weeks early. He has mild hearing loss and can find school challenging.

"Jago's first year at school was much harder than we'd expected," says Georgie. "Making friends has always been slow as he was quite isolated in his earliest years, and he finds school very tiring. He has to work harder to overcome problems associated with background noise. He has struggled with maths but finds writing and reading the hardest."

"This resource is one that I hope every teacher around the world can learn from."

On average, **2 or 3** children in a typical UK primary class will have been born prematurely

Making a difference for children with disabilities

Aiden suffered a brain injury at birth. He is severely affected by cerebral palsy as a result, but a range of treatments and therapies are helping him make positive progress. Action is funding research to help children facing the challenges of a range of disabling conditions.

Aiden had a traumatic start to life. Complications at his birth meant he was deprived of oxygen for almost half an hour and suffered a serious brain injury called hypoxic-ischaemic encephalopathy.

To give him the best possible chance, Aiden was whisked away to intensive care where he received specialist cooling treatment. His tiny body was wrapped in an ice blanket for three days to reduce his core temperature and try to help limit further brain damage. This life-saving treatment is now routine in the UK for babies who've suffered a shortage of oxygen at or around birth, and was the product of a 20-year programme of research to which Action contributed more than £1 million.

Aiden's prognosis on discharge from hospital was initially very encouraging, but as time went on the damage to his brain, and impact this had on his movement and coordination, became more evident. He was diagnosed with cerebral palsy when he was two years old.

"Aiden has been a fighter since the moment he was born and continues to fight for every milestone he achieves," says his dad, Keith. "Currently he cannot sit or walk unaided and can only say a few words but with continued therapy he is making progress."

Cerebral palsy affects around **2,000** babies born in the UK each year

> "WITHOUT PREVIOUS RESEARCH, AIDEN'S OUTCOME COULD HAVE BEEN EVEN MORE SEVERE. RESEARCH THAT COULD FURTHER ADVANCE TREATMENTS IS SO IMPORTANT." **Aiden's dad, Keith**

Now five, Aiden is a happy little boy who loves to play and be around people. He's rarely seen without his cheeky smile and zips around in his walker, despite his parents previously being warned he might never be able to do so.

"Cognitively he understands everything, it's just that his brain and his muscles don't talk to each other enough," says his mum, Fleur. "That's why there's so much physiotherapy needed. But it really does make a difference and every little bit of progress is amazing to see."

Family life revolves around Aiden's therapies. These treatments can help to strengthen muscles and allow children like Aiden to learn new movement patterns and skills.

With the help of specialist equipment, Aiden is able to enjoy being part of the world around him and he uses an eye gaze device to help him communicate. This is essentially a tablet that can be controlled by eye movements.

"There is no cure for cerebral palsy or the brain damage he has but we can help to reduce the impact it has on him and give him the best possible quality of life," says Fleur. "We hope in time he will reach a level of independence. We want to make life as amazing for Aiden as he is."

Keith adds: "Action has a very special place in our hearts. It's thanks to previous research that Aiden's getting a chance at life and we want to ensure that others get that same chance."

Research for children with cerebral palsy

In 2019 Action funded three new projects to help children with cerebral palsy – the most common serious physical disability in children.

Cerebral palsy causes difficulties with movement and coordination, and around half of those affected also have varying difficulties with speech. Researchers at the University of Newcastle are creating a UK version of a new computerised assessment tool to test how well children with severe movement problems understand spoken language. This would provide vital information to help each child get the best possible treatment and support.

Action is also supporting a study, using state-of-the-art technology, to understand why children with cerebral palsy who can walk often develop painful foot deformities. This team will examine, in unprecedented detail, how children walk and the forces affecting their muscles, bones and-

joints. This could identify those at most risk and pave the way for tailored treatments and strategies to keep them mobile.

And at Oxford Brookes University, a pilot study is testing whether a safe, non-invasive and painless type of brain stimulation can help improve movement when combined with physical therapy to the arms and legs. This is funded together with the Chartered Society of Physiotherapy Charitable Trust.

Reducing the impact of brain injury

Serious head injuries are sadly a leading cause of disability in children. Research is building a better understanding of the long-term effects on brain development.

Around **35,000** children are admitted to hospital each year with traumatic brain injury

Head injuries can have life-changing consequences, leaving some children with ongoing problems such as learning disabilities, or emotional or behavioural issues such as aggression. But it is currently difficult for doctors to predict who is likely to suffer these effects.

In previous Action-funded work, researchers used advanced MRI scans and neuropsychological assessments to study a group of children with traumatic brain injuries. They identified specific types of injury that were linked with marked impairments in memory, concentration and behaviour.

The team, based at Imperial College London, will now assess the same children again, comparing them with a group of uninjured children, to see if their brains follow expected growth patterns over time, and assess how any changes affect behavioural development.

Lead researcher Professor David Sharp says: "Knowing which children are more likely to struggle would mean they could get earlier access to treatment and support when it may be most beneficial."

Steps Forward

TREATING CHILDREN WITH EPILEPSY AND UNCONTROLLED SEIZURES

Over 60,000 children and teenagers aged 18 and under have epilepsy in the UK. Sadly, medication doesn't work for up to a third of these young people and other treatments are not always effective.

Dr Antonio Valentin, of King's College London, has developed 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.

In 2019 Dr Valentin reported that 12 children had already benefited from the technique, and it's estimated up to 30 a year could be treated in the future.

Sophie (left) suffered multiple seizures a day. They happened without warning, leaving her constantly vulnerable to serious injury. She became seizure-free straight away following treatment and has gone on to achieve A grades in her A Levels, visit 30 countries on a gap year and start university.

This research was jointly funded with Great Ormond Street Hospital Children's Charity.

Giving hope to families facing rare diseases

Danny's family are painfully aware that time with their youngest son will be cruelly cut short by Hunter syndrome, a condition with no cure and limited treatment options. Action is funding research that could change things for families like theirs in the future.

"Danny lives in the moment and enjoys whatever he has," says his mum Sally. "He loves kicking and throwing balls, and running in the opposite direction to where he's supposed to. His smile is totally infectious, and he wins hearts wherever he goes."

This 'in the moment' outlook is one that Sally herself tries hard to adopt, as time with Danny, now eight, is incredibly precious.

Hunter syndrome almost exclusively affects boys. It's caused by a faulty gene that leads to the lack of an enzyme that's vital for breaking down sugars in the body. Without this enzyme, waste sugar molecules build up in all the major organs, tissues and joints, causing a range of problems.

Children with the most severe form of the disease, like Danny, have progressive learning difficulties due to a build-up of sugars in their brain. In his younger years, this meant that Danny's behaviour could be extremely unpredictable and challenging.

The majority of rare diseases currently have no effective treatment

Life has got a lot calmer lately but for the saddest of reasons, as Danny is now losing skills he previously had. He no longer speaks, having previously said over 50 words and short phrases, and his parents are now having to think about adapting the house for when he becomes more reliant on his wheelchair. "I'd love to have my little trouble back in full force, because I know what this calm after the storm is leading to," says Sally.

Danny was diagnosed with Hunter syndrome when he was three years old, having had a history of developmental delay. When he was finally referred to a paediatrician, the doctor immediately homed in on his looks, which his parents discovered were typical of his condition.

"As soon as I read the symptoms and saw pictures online, I knew that he had it," says Sally. And what she read was heartbreaking for any parent.

Sally and husband Craig waited several agonising weeks before tests confirmed their fears and life changed forever. Tragically, later tests would find that Danny had the worst possible outlook – a complete gene deletion in his DNA.

Danny began enzyme replacement therapy (ERT) – a treatment that can help stop the further build-up of waste within the body, relieving some of the physical symptoms. However, this cannot currently reduce neurological damage.

> "JUST LIKE THAT, EVERYTHING IS RIPPED AWAY. ALL HOPES AND DREAMS FOR YOUR CHILD GONE. BIT BY BIT, WE'RE GOING TO LOSE HIM." **Danny's mum, Sally**

For several years Danny was also part of a clinical trial, but sadly this treatment didn't work for him. "We knew it was the end of any hope," says Sally, "We will lose our beautiful boy to Hunter syndrome."

Sally knows that research now will come too late for them but says: "Each new development brings real hope. And any new hope is worth fighting for. So that families in the future don't have to feel that the bottom is dropping out of their world."

Improving treatment for Hunter syndrome

Being able to reduce the neurological damage caused by this disease could transform the outlook for boys like Danny. New research is testing a way of overcoming the problem of getting much-needed treatment into the brain.

Hunter syndrome, which is also known as mucopolysaccharidosis type II, is caused by a faulty gene that leads to the lack of a vital enzyme in the body's cells. Without enough of this enzyme, sugar molecules build up causing a wide range of distressing symptoms.

Professor Brian Bigger and his team at the University of Manchester have previously developed a stem cell treatment, which inserts a correct copy of the faulty gene into the body.

These cells then produce the missing enzyme, helping to reduce some of the effects of the disease. However, the impact made in alleviating the debilitating and progressive brain symptoms has been sadly limited as the blood-brain barrier prevents treatment from crossing into the brain efficiently. The team is now investigating a new way to try and overcome this.

Currently, there are over **6,000** known rare diseases

This research has been jointly funded as part of our partnership with LifeArc to develop treatments for children with rare diseases.

Steps Forward

CLINICAL TRIAL TO TEST NEW XLP CURE

Action funding has helped drive research to develop gene therapy for a rare immune system disorder, with a clinical trial due to start in 2021.

X-linked lymphoproliferative disease, or XLP, is a life-threatening disorder that usually only affects boys. Sadly, without treatment around seventy percent of those with the disease would die by the age of 10.

Currently, the only hope for a cure is a bone marrow transplant, but finding a well-matched donor, at the right time, isn't always possible.

Building on earlier research, also funded by Action, Professor Bobby Gaspar and Dr Claire Booth, of University College London's Institute of Child Health, have been developing a new gene therapy treatment. This involves replacing the faulty gene that causes XLP with a healthy copy.

Further joint funding from Action and Great Ormond Street Hospital Children's Charity has now enabled the team to modify the faulty gene in T-cells, from children's own immune systems, using a special gene transfer system. These modified cells have now been shown to improve symptoms of XLP in laboratory tests.

The next step will be to transplant corrected cells into child patients in a clinical trial, due to start next year. "This would not have happened without Action funding," says Dr Booth.

Steps Forward

HELPING TINY HEART PATIENTS

Researchers have used advanced computer modelling techniques to study two treatments for babies born with a rare heart condition, revealing important new information.

Babies born with hypoplastic left heart syndrome face a series of complex, life-saving operations – the first when they are less than two weeks old. This initial operation can be carried out using one of two techniques.

With funding from Action Medical Research and Great Ormond Street Hospital Children's Charity, Dr Pablo Lamata and his team at King's College London used advanced computer analysis to retrospectively compare MRI scans of babies treated using each approach.

Their new techniques allowed them to create 3D models of each heart and revealed subtle changes that couldn't previously be seen. They found that one of the treatments could impair the growth and function of babies' hearts, and the other did not.

The team also studied the aorta, the main blood vessel in the heart, and discovered a new 'weak link', which places additional burden on these vulnerable babies – this was detected thanks to new non-invasive methods for estimating central blood pressure within the heart.

This research has yielded valuable knowledge to help guide surgical decision-making in the future and improve monitoring and assessment for children living with congenital heart conditions. This could reduce risks to children and allow closer monitoring, as well reducing the costs of treating these children.

"THIS VALUABLE KNOWLEDGE COULD HELP SURGEONS CHOOSE THE BEST SURGICAL TECHNIQUE IN THE FUTURE."

Dr Pablo Lamata

Testing a new leukaemia treatment

With Action funding, researchers are investigating a new drug combination for a hard-to-treat type of leukaemia that typically strikes babies under a year old.

Leukaemia is the most common childhood cancer and affects white blood cells, causing them to grow too fast and take over the bone marrow.

Leukaemia affects around **500** babies, children and young people in the UK each year

While the majority of children can now be cured, sadly some types of leukaemia remain difficult to treat. One is a subtype of acute leukaemia that is caused by a particular DNA change in the cancer cells – and it usually affects babies. In these patients, part of the MLL1 gene has fused with another gene, causing blood cells to make a cancer-causing protein that drives the disease.

Based at the UCL Great Ormond Street Institute of Child Health, Dr Owen Williams and his team have already shown that a combination of two existing drugs can destroy the cancer-causing protein. They will now carry out further tests to explore the safety and effectiveness of the treatment, and the best dose.

This research is funded jointly with LifeArc.

"THIS COULD SAVE THE LIVES OF BABIES AND CHILDREN FOR WHOM NO EFFECTIVE TREATMENTS CURRENTLY EXIST."

Dr Owen Williams

Steps Forward

A NEW TARGET TO KILL CANCER CELLS

With previous Action funding, Dr Owen Williams and his team have identified a new target to direct treatment at and fight acute lymphoblastic leukaemia. Significantly, they believe their findings could be relevant to many subtypes of the disease – an exciting development that was unexpected.

This work, funded jointly with Great Ormond Street Hospital Children's Charity, focused on a mutated, cancer-causing gene called TEL-AML1. This rogue gene is present in up to a quarter of children suffering from acute lymphoblastic leukaemia.

The research led to a better understanding of the disease biology – and the discovery of a signalling pathway that allows leukaemia cells to survive and multiply. Laboratory experiments then showed that blocking this pathway causes leukaemia cells to die. This helped to identify a target for developing a drug treatment.

The team is now working to identify existing drugs that could be repurposed to hit this target. If successful, they envisage clinical trials in patients within the next five years. Dr Williams says: "This may also be applicable to a much wider range of leukaemias, including those more difficult to treat by standard chemotherapy."

"THIS WORK IS AMAZINGLY IMPORTANT. TREATMENT CAN BECOME MORE TAILORED, MORE INDIVIDUAL AND SO POTENTIALLY CAUSE THE CHILD LESS HARM."

Joey's dad, Darren

Steps Forward

KINDER TREATMENTS FOR CHILDREN WITH BRAIN TUMOURS

Children with brain tumours face prolonged and gruelling treatment, with surgery, chemotherapy or radiotherapy. While these can prove life-saving, they can also cause serious, long-term side effects.

Jointly funded by Action Medical Research and The Brain Tumour Charity, Professor Andrew Peet and his team at the University of Birmingham have developed a new, non-invasive technique to assess brain tumours.

The researchers found that levels of lipids and glutamine, biological substances in the brain, are direct indicators of how aggressive a tumour will be. Using an MRI scanner, these substances can be measured with greater accuracy than is currently done in routine practice.

This approach particulary benefits children with tumours in high-risk areas who may not ordinarily be able to undergo a biopsy.

It also enables doctors to have a more accurate understanding of each tumour and to tailor treatments accordingly, so that they are only as toxic as they need to be.

Professor Peet believes this is a huge step towards introducing more personalised treatments.

Joey, pictured, was diagnosed with a brain tumour when he was just four. Thankfully, his treatment worked and he now hopes to become a doctor. His dad Darren says: "We are so delighted that this progress has been made that will help make treatment even better in the future."

Protecting children, now and always

For almost 70 years, we have risen to the medical research challenges of the day. We've helped develop life-saving vaccines, treatments and cures, and developed future leaders of research. As 2019 drew to a close, a new health crisis was emerging. The unfolding COVID-19 pandemic highlights that medical research has never been more important.

Developing vaccines and treatments

Inspired by the need to protect children from the terrible threat of polio, Duncan Guthrie laid the foundations for Action Medical Research back in 1952.

Seventy years ago polio was one of the most feared diseases in the developed world. In the early 1950s, 8,000 people were paralysed by polio each year in the UK, and five to 10 per cent died after their breathing muscles became immobilised.

Duncan Guthrie's own daughter, Janet, was diagnosed with polio when she was young. Thankfully, she recovered but many weren't so lucky.

Frustrated by the lack of research and treatment centres in the UK, Duncan set up the charity that would become Action Medical Research. Within 10 years, the UK polio vaccines were introduced and have kept millions of children safe from this deadly virus ever since.

Today we no longer focus on a single condition, giving us the flexibility to respond to serious health needs as they emerge. And our work has continued to fight infections.

In the 1960s and 1970s, we supported the testing of the early rubella vaccine. Rubella can cause severe abnormalities in an unborn baby if contracted in the early stages of pregnancy. Before immunisation was introduced, epidemics were a regular occurrence. Today, rubella is very rare.

Over the last 45 years, Action has also invested over £1.7 million in a range of meningitis research projects. This included supporting the team who helped establish the Hib vaccine to prevent what used to be the most common cause of bacterial meningitis, Haemophilus influenzae type b. Hib infection previously caused around 800 cases of meningitis each year in England and Wales. The introduction of the vaccine, in 1992, saw incidence in children under five fall by 98 per cent.

Supporting future research leaders

Action is proud to have played a role in supporting some of the doctors and scientists who are now at the forefront of the fight against COVID-19.

Professor Andrew Pollard was awarded an Action Medical Research Training Fellowship in 1995. Our Fellowship scheme funds promising doctors and researchers early in their careers and supported Professor Pollard's early work studying meningitis in young children.

> "RESEARCH IN THE COVID-19 PANDEMIC HAS RIGHTLY FOCUSED ON SEVERE DISEASE IN ADULTS, BUT CHILDREN ARE ALSO SIGNIFICANTLY AFFECTED DIRECTLY AND INDIRECTLY BY THE DISEASE AND ITS CONSEQUENCES."
>
> **Professor Andrew Pollard**

Today, he is Professor of Paediatric Infection and Immunity at the University of Oxford and Director of the Oxford Vaccine Group, which has identified a potential vaccine for COVID-19. He is leading the UK's first clinical trial. If the vaccine is safe and effective, it could provide an exit strategy for the pandemic and save lives.

Professor Pollard is also a member of an expert advisory group that Action has brought together with the aim of finding out more about how the virus affects children.

Professor Wendy Barclay, Action Medical Research Chair of Virology at Imperial College London, is also using her expertise to help fight coronavirus. Her background is in studies to help understand how viruses, like influenza, replicate and how they are transmitted. Her work has previously contributed to the production of new vaccines and she is now focusing her efforts on tackling COVID-19.

We must not forget children in the fight against COVID-19

Medical research is underway to help beat the new virus but there's a lack of research focusing on children and how it affects them. We want to fund research to find answers to help protect our children and their futures.

Parents are living in fear of the unknown with many unanswered questions that need tackling.

While adults seem to be more susceptible, COVID-19 can and does affect children too. There is also much still unknown about the risks of the virus in pregnancy and to babies. Plus there are many questions to be answered about the mental health effects on children and young people, whose daily lives have been turned upside down by the pandemic, and about the impact on those already living with life-threatening and disabling conditions.

Finding out why most children have been less affected by COVID-19 could also help us to better understand and fight the disease in all age groups.

Building on our impressive track record of funding high-quality research that saves and changes children's lives, we are now bringing together an expert advisory group of leading children's health researchers to help us fund research to better understand how COVID-19 affects children.

For more information visit action.org.uk/covid19

Research grants awarded in 2019

Action Medical Research is funding around 60 projects across the UK, 14 of them awarded in 2019. **The next medical breakthrough could be on your doorstep.**

Brain injury at birth – could a new treatment help protect brain cells from damage?
Lead researcher: **Dr C Thornton**
King's College London

Cerebral palsy and severe movement problems – assessing language comprehension [1]
Lead researcher: **Dr L Pennington**
Newcastle University, University College London, Manchester Metropolitan University, Northumberland Tyne and Wear NHS Foundation Trust and Great Ormond Street Hospital for Children NHS Foundation Trust

Cerebral palsy – understanding the causes of foot deformity
Lead researcher: **Dr C J Stewart**
Robert Jones and Agnes Hunt Orthopaedic Hospital, Imperial College London, Oxford University Hospitals NHS Foundation Trust, Keele University

Cerebral palsy – improving movement through brain stimulation [2]
Lead researcher: **Professor N H Dawes**
Oxford Brookes University, University of Oxford, Oxford University Hospitals NHS Foundation Trust, Royal Free London NHS Foundation Trust, Brunel University London

Childhood leukaemia – investigating a new drug treatment [3]
Lead researcher: **Dr O Williams**
UCL Great Ormond Street Institute of Child Health, Great Ormond Street Hospital for Children, UCL Cancer Institute, University College London

Ear infections – understanding how the body responds
Research Training Fellowship: **Mr M W Mather**
Newcastle University, Freeman Hospital, Newcastle, University of Sheffield

Ear reconstruction – developing implants using tissue engineering [4]
Research Training Fellowship: **Mr T H Jovic**
Swansea University

Hunter syndrome – improving gene therapy [3]
Lead researcher: **Professor B W Bigger**
University of Manchester

Neurodevelopmental delay – predicting those at risk
Lead researcher: **Dr C E Aiken**
University of Cambridge

[1] Funded together with Newcastle Upon Tyne Hospitals NHS Charity and the Great North Children's Hospital Foundation
[2] Jointly funded with the Chartered Society of Physiotherapy Charitable Trust
[3] Jointly funded with LifeArc
[4] The VTCT Foundation Action Medical Research Training Fellowship is supported by the VTCT Foundation
[5] Jointly funded with Borne
[6] Funded by a generous donation from the Garfield Weston Foundation

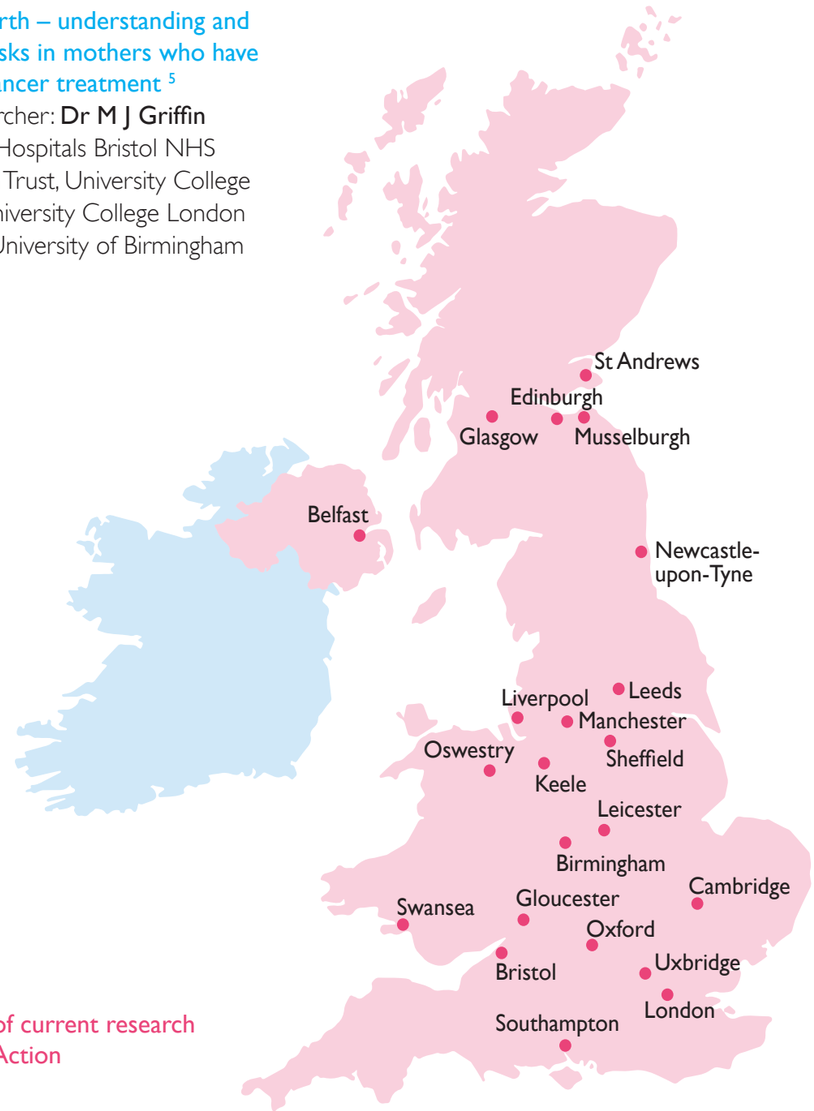
Preterm birth – identifying babies with infection or those at high risk of infection
Lead researcher: **Dr D L Gibbons**
King's College London, Homerton University NHS Trust, London

Preterm birth – preventing premature contractions [5]
Lead researcher: **Professor M J Taggart**
Newcastle University, Royal Victoria Infirmary, Newcastle upon Tyne Hospitals NHS Trust, Queen's University Belfast

Preterm birth – understanding and reducing risks in mothers who have survived cancer treatment [5]
Lead researcher: **Dr M J Griffin**
University Hospitals Bristol NHS Foundation Trust, University College London, University College London Hospitals, University of Birmingham

Traumatic brain injury – long-term impact [6]
Lead researcher: **Professor D J Sharp**
Imperial College London, UCL Great Ormond Street Institute of Child Health, University College London

X-linked lymphoproliferative disease – working towards a new cure
Lead reseacher: **Dr C Booth**
UCL Great Ormond Street Institute of Child Health



Locations of current research funded by Action

Summarised financial statements

for year ended 31 December 2019

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 22 April 2020. The full financial statements, on which the auditor, Buzzacott LLP, gave an unqualified audit report on 15 May 2020, was submitted to the relevant statutory bodies, including the registrar of companies, on 19 May 2020. An emphasis of matter paragraph was included in the audit report to draw attention to the trustee disclosures in the financial statements regarding the COVID-19 global pandemic. The audit opinion was not modified in respect of this matter.

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 2019.

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

Phil Hodkinson, Chair

Income and expenditure	2019	2018
	£000s	£000s
Net incoming resources		
Donations and legacies	2,728	2,686
Investments	(2)	44
Total net incoming resources	2,726	2,730
Outgoing resources		
Medical research projects	2,598	2,642
Medical dissemination	748	798
Net outgoing resources	(620)	(710)
Net gains (losses) on investments	901	(785)
Net movement in funds	**281**	**(1,495)**

Balance sheet	31 Dec 19	31 Dec 18
	£000s	£000s
Fixed assets		
Tangible	311	333
Investments	11,656	11,609
Total fixed assets	11,967	11,942
Current assets	1,382	1,328
Current liabilities within one year	(5,293)	(5,309)
Liabilities falling due after one year	(2,009)	(2,195)
Total net assets	6,047	5,766
Representing:		
Unrestricted funds	6,047	5,766
Restricted funds	-	-
Total funds	**6,047**	**5,766**

Thank you

We are hugely grateful to the many individuals, companies, trusts and foundations who have so generously donated to the vital work supported by Action Medical Research.

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 2019.

Supporters

Airport Parking & Hotels
AON
Arun Estates
Benenden Hospital
BGC
BioIndustry Association
Boongate Kia
Brett Group
Buckles Solicitors
COOP Community Fund
Dangoor Education
de Brye Charitable Trust
DMH Stallard
DM Thomas Foundation
Edith Florence Spencer Memorial Trust
Garmin
Gingerman Group
Grafton Group
Hospital Saturday Fund
Liberty Specialty Markets
Milton Damerel Trust
Next Retail
Oso Foundation
Paddington at Paddington Station
Price Bailey
Quai Administration Services
Rouleur
Rusk & Rusk
Scrap Car Comparison
Sir William Coxen Trust Fund
Skerritts Consultants
Willmott Dixon

Legacy gifts

Phyllis Collier
Barbara Ellis
Brian J Hawkins
Rosemary Horton
Malcolm Hunter
Carol (Louise) Oliver
Carol M Pratt
Sydney Scott
Daphne Stoddart

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 2019

Patron

HRH The Prince Philip Duke of Edinburgh KG KT OM GCVO GBE ONZ QSO AK GCL CC CMM

President

Field Marshal The Lord Guthrie GCB LVO OBE DL

Vice Presidents

Stephen May
Richard Price
The Duchess of Northumberland

Trustees

Phil Hodkinson, Chair
Luke Bordewich, Honorary Treasurer
Esther Alderson
Professor Sarah Bray BA MPhil PhD FMedSci
Professor David Edwards MA MBBS DSc MRCR FRCP FRCPCH FMedSci
Kathy Harvey
Karen Last – appointed November 2019
Val Remington-Hobbs – retired July 2019
Professor David Rowitch MD PhD ScD
Richard Stoneham-Buck – appointed April 2019
Richard Wild

Scientific Advisory Panel

Professor David Rowitch MD PhD ScD, Chair
Professor Graeme Black OBE DPhil, FRCOphth – retired June 2019
Dr Claire Booth MBBS MSc PhD MRCPCH
Professor Clare Bryant BSc PhD BVetMed
Professor Yanick Crow MBBS MRCP CCST PhD FMedSci – appointed December 2019
Professor Inderjeet Dokal MBChB MD FRCP FRCPCH FRCPath FMedSci
Professor Alicia El Haj FREng FRSB FEAMBES
Professor Jonathan Grigg BSc MBBS MD FRCPCH
Professor Catherine Hawrylowicz PhD
Professor Mark Johnson PhD MRCP MRCOG
Professor Samantha Johnson PhD CPsychol AFBPsS
Professor Fenella Kirkham MB BChir MD FRCPCH
Professor Scott Nelson PhD MRCOG – retired June 2019
Professor Andrea Nemeth BSc MBBS DPhil (Oxon) FRCP – appointed December 2019
Dr Adam Shortland BSc PhD MIPEM CSci
Professor Shiranee Sriskandan FRCP PhD
Professor Michael Taggart BSc PhD

Thank you

With your support we can fund medical breakthroughs for some of the toughest fights our children face.

A member of the:

amrc
ASSOCIATION OF MEDICAL RESEARCH CHARITIES

Registered with FUNDRAISING REGULATOR

FSC
www.fsc.org
MIX
Paper from responsible sources

Action Medical Research is a registered charity: England and Wales no. 208701; Scotland no. SC039284

Photos: David Brunetti and Ben Rector