action medical research for children

RESEARCH REVIEW 2025

Saving and changing children's lives

Contents

WELCOME

At Action Medical Research we work to save and change children's lives by funding groundbreaking research that can lead to medical breakthroughs, treatments and cures.

In 2025, we funded 15 new research projects across the UK to help babies, children and young people. This research brings hope to families affected by conditions including premature birth, cystic fibrosis, sight loss in children and T-cell acute lymphoblastic leukaemia.

We currently have over £11 million invested in the work of around 200 top researchers, working on around 50 different projects across the UK.

Working in partnership is important to us, helping to accelerate progress through collaboration. In 2025, we were delighted to fund new research alongside Borne, the British Paediatric Neurology Association and Cystic Fibrosis Trust.

We also awarded three new Research Training Fellowships. These support doctors and researchers at a critical early stage in their research careers. Our new Fellows are investigating pain assessment in babies after surgery, spine fractures in young people with Duchenne muscular dystrophy, and Rasmussen's encephalitis, a rare and serious brain inflammation in children. Since our scheme began, we have supported 194 Research Training Fellows, many of whom have gone on to become leaders in children's health research.

Every year, we review the impact of previously funded research. You'll find progress updates throughout this Research Review. These include how Action funding enabled the first randomised controlled trial comparing treatments for children with amblyopia, or lazy eye. This has led to new guidance to improve treatment. We also report on how early-stage research has helped pave the way towards clinical trials to treat Rett syndrome.

Our work to save and change children's lives is only possible thanks to the support of many. We are grateful for the generous backing of trusts, foundations, companies, groups, and individuals. We also appreciate the knowledge and expertise of our scientific advisory panel, and thank our local committees, volunteers and those who remember Action in their wills.

Please join us in the fight to help the hundreds of thousands of sick and disabled babies and children across the UK who need our help.

Chief Executive
Action Medical Research for children

HELPING SICK BABIES

All babies should have the best possible start in life. Research we fund aims to find ways to prevent premature birth, to protect babies from complications that can cause lifelong disabilities and to improve care for those who need it most.

Understanding the power of phages to help premature babies

Action-funded research has significantly increased understanding of why feeding with breast milk could reduce the risk of some life-threatening complications for preterm babies.

Necrotising enterocolitis (NEC), a serious gut disease, and sepsis are two of the most common serious illnesses that can affect very premature babies. Sadly, some lose their lives due to these dangerous complications – and those who survive are often left with lifelong disabilities.

It was already known that breast milk could reduce the risk of NEC and sepsis in premature babies, with evidence suggesting that this was due to immune-boosting factors.

With Action funding, Professor Darren Smith and his team analysed breast milk samples. They focused on the role played by phages, naturally occurring viruses that can infect, kill or change how bacteria grow. These tiny viruses are believed to play a key role in shaping the gut microbial community of premature babies, supporting the immune system in early life.

Studying milk samples donated by mothers of very premature babies, the team discovered that phages are abundant from the first week after birth and remain throughout the first 100 days of lactation.

The research also found that these phages can bind to naturally occurring fats in the breast milk and are delivered from mother to child.

In laboratory tests, the researchers found that phages taken from breast milk could change the composition of bacteria, suggesting they may help shape early gut health.

Thanks to this Action-funded study the team, based at the University of Northumberland, has since been able to launch a further larger study, with £1.2 million of funding from the UKRI Biotechnology and Biological Sciences Research Council.

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

> **“We’re extremely excited to have received significant further funding, which follows on from our Action-funded project.**
>
> Professor Darren Smith

PROGRESS MADE

Towards a new test to detect CMV in babies

Action funding has helped researchers to develop a low-cost biosensor, which could enable newborn screening for a common but often hidden virus that can cause serious harm.

Cytomegalovirus, or CMV, is a common and usually harmless virus. But it can cause serious health problems – including sight or hearing loss – for some babies whose mothers unknowingly pass it on during pregnancy.

Early detection and treatment helps to improve outcomes. But most babies show no symptoms at birth – and there is currently no routine screening in the UK, partly because of the lack of a suitable diagnostic device.

At Swansea University, Professor Vincent Teng and his team have developed a low-cost printed biosensor that can quickly detect CMV in urine or saliva.

With Action funding awarded in 2023, the team has refined both the manufacturing and performance of the biosensor, which they've now shown can outperform current, more complex, tests.

The technology is now a step closer to providing an affordable and practical large-scale screening method for newborn babies.

> "The support from Action Medical Research has been absolutely vital for our progress."
>
> Professor Vincent Teng

Making a difference for children like Mylo in the future

Mylo was diagnosed with congenital CMV when he was three months old. Sadly, he has been severely affected and has hearing loss, visual impairment and other complex needs.

When Mylo was born, his parents already knew there was potentially something wrong. A routine scan at 36 weeks had shown excess fluid on his brain, but the cause was unknown.

After a few days in the neonatal unit, he seemed well enough to be discharged and went home. However, he had soon failed his newborn hearing test and began to miss early milestones in his development.

When he was three months old, Mylo was finally diagnosed with congenital CMV, after a doctor suggested going back to his Guthrie card to do further tests on his newborn bloods.

Mylo was offered antiviral drugs which can help to limit the damage caused by the virus, especially if given soon enough. But though the family initially felt optimistic, sadly their hopes soon faded.

"We were hoping for a miracle treatment," says his mum, Ruth. "But it didn't appear to make any significant difference. As time went on his complex needs emerged, seemingly more by the day."

Mylo, who is now 10, is profoundly deaf in one ear, with milder hearing loss in the other. He also has cerebral visual impairment and is completely non-verbal, communicating only by crying and laughing. His mobility is limited to being able to take just a few steps unaided.

"Mylo is the light of our life, but caring for a child with his needs is not easy by any stretch of the imagination," says Ruth. "It was difficult to comprehend – and still is now – how Mylo would be one of the children that is affected so severely."

"We were told that ideally treatment for CMV should be administered within six weeks to maximise the effect. That Mylo has been affected so severely is still a hard pill to swallow."

> "**Had we known at birth that Mylo had CMV, he could have had immediate treatment, which may have made it more successful.**
>
> Mylo's mum, Ruth

Mylo, who was born with congenital CMV

NEW RESEARCH

A cot-side scanner to identify babies at risk of brain injury

Action-funded researchers are testing an innovative scanning device that provides an unprecedented window into how a baby's brain is working.

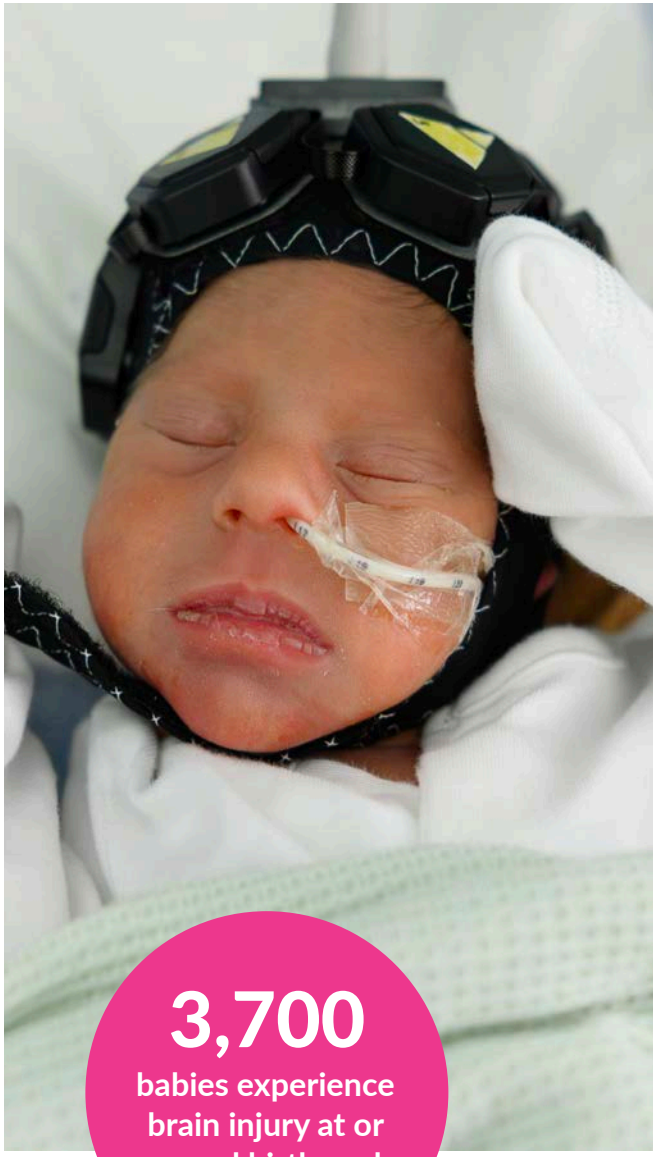
Brain injury can occur during or soon after birth, often as a result of complications that interrupt the supply of oxygen and blood to a baby's brain. Advances in neonatal care mean more babies than ever are now surviving these difficult starts. However, many of these infants remain at increased risk of long-term disabilities.

Some babies go on to develop conditions such as cerebral palsy or epilepsy, or experience learning or behavioural difficulties. These challenges can significantly affect children's health and quality of life, as well as putting emotional and practical strain on families.

Early diagnosis allows doctors to provide more targeted support and interventions. But while current brain imaging is good at detecting injury, it is less effective at predicting long-term outcomes.

Professor Topun Austin and his team at the University of Cambridge aim to improve this. They are testing a non-invasive brain scanning device designed specifically for newborn babies. The portable system combines two advanced imaging technologies to provide real-time information about how a baby's brain is functioning.

This new tool could help provide doctors with a better understanding of which babies are most at risk of developing long-term problems. It could ultimately transform the diagnosis and treatment of brain injuries, improving long-term outcomes for sick and preterm babies worldwide.



3,700
babies experience brain injury at or around birth each year in the UK

> **"This system could help transform neonatal care by enabling early diagnosis and more targeted treatment for brain injuries.**
>
> Professor Topun Austin

NEW RESEARCH

Using AI to protect babies from pain

Many babies undergo surgery each year, but it can be challenging for doctors to recognise and treat their pain afterwards.

Experiencing pain is distressing for babies and their families and may also negatively affect brain development. But it can be hard to judge when babies are in pain – and how severe it is.

Doctors and nurses use pain scores to monitor babies after surgery. These include observing behaviours, such as facial expressions, crying and activity levels, and vital signs like heart rate. However, checks are done intermittently, and different staff may score the same baby differently. This means there is a risk that pain is missed and left untreated.

With Action funding, Dr Roshni Mansfield, at the University of Oxford, aims to improve pain monitoring. She and her team are using AI and machine learning to create a new tool that automatically generates continuous pain scores using video, audio and vital signs. They are collecting data from babies as they recover after surgery to repair a hernia. They will also assess how scores change after pain relief is given.

Dr Mansfield is a recipient of an Action Research Training Fellowship. These awards help doctors and scientists to develop their careers in research.

> **We hope to guide more personalised pain management, helping to support babies' recovery and care.**
>
> Dr Roshni Mansfield

NEW RESEARCH

Developing a treatment to help prevent premature birth

Professor Rachel Tribe and her team are developing a new treatment that aims to prevent spontaneous preterm birth by modifying the mother's immune and inflammatory responses during pregnancy.

Preterm birth is sadly the leading cause of newborn death – and children who survive after being born too soon can often face lifelong disabilities.

Most preterm births happen spontaneously, that is naturally, and are often linked with inflammation or infection in the mother.

During early pregnancy, the mother's body must maintain the baby in the womb, which involves suppressing her natural immune responses. Specialised cells in the womb lining – called human decidual stromal cells (hDSCs) – are known to play an important role in this process.

Previous research, which Action helped to support, found that delivering human decidual stromal cells during pregnancy can prolong pregnancy in a laboratory model of preterm birth.

Now, Professor Tribe and her team, based at King's College London, are testing whether this cell treatment can also delay delivery to help save lives. They are also studying the cells' effects on inflammation in mother and baby and finding the best dosage and timing to maximise its success.

"This research will provide important evidence to support future clinical trials to assess its potential benefits in women at high risk of giving birth too soon," says Professor Tribe.

If successful, this innovative treatment could ultimately lead to better outcomes for many babies and their families.

This research is jointly funded with Borne.

> **"Current treatment options to prevent babies from being born too soon are very limited – and there's an urgent need for effective ways to delay labour and improve outcomes.**
>
> Professor Rachel Tribe

Helping babies like Fletcher

Fletcher was born extremely prematurely, weighing just 705 grams. He spent 15 weeks in neonatal intensive care, fighting life-threatening complications.

Fletcher's mum Rosie's pregnancy was exhausting and for much of it she was on bed rest. She'd experienced unexplained early bleeding, as well as suffering from excessive nausea and vomiting, and severe migraines. Then, just over 24 weeks into the pregnancy, she suddenly began to bleed very heavily and was taken to hospital, where she was rushed to the delivery suite.

Rosie's labour was long and incredibly challenging. Consultants regularly visited to explain potential procedures and possible outcomes – and 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. He would remain dependant on a ventilator for 57 days and still needed oxygen support when he finally went home.

In neonatal intensive care, Fletcher endured many complications that often affect very premature babies. He fought sepsis, *E.coli* infection, pneumonia, respiratory problems and the life-threatening bowel disease necrotising enterocolitis (NEC). His parents saw other babies, born at a similar gestation, sadly pass away – and there were times when they too felt very fearful for their son's life.

Today, Rosie and Bobby are incredibly proud of the progress Fletcher continues to make, though he does have some developmental and learning issues linked to being born so early.

"He continues to amaze us every day – going from a baby you could hold in one hand to a bubbly little boy," says Rosie. "But we know that not every family has the same outcome. We wouldn't be here as a happy family if it weren't for all the research that goes into prematurity and the amazing medical staff."

Baby Fletcher

Fletcher spent 105 days in neonatal intensive care

> **"When we started our neonatal journey, we were quite naive. It was hard to truly understand what our little boy would go through because he was born too early.**
>
> Fletcher's mum, Rosie

DISABLING AND CHRONIC CONDITIONS

We fight to help children facing a lifetime of challenges caused by conditions such as cerebral palsy, to help those with a learning disability, and for those living with long-term conditions like cystic fibrosis or inflammatory bowel disease.

Developing digital exercise programmes for children with disabilities

Action-funded work has helped to spark global research to develop digital and physical exercise programmes for young people with cerebral palsy and other disabilities.

A pilot study co-funded by Action has become the launchpad for a much larger programme of work that is set to help improve the lives of children with disabilities in the UK and beyond.

In 2017, Professor Helen Dawes and her team began investigating the potential benefits of different classroom-based exercise programmes for children with cerebral palsy. Children with cerebral palsy can spend long periods sitting, due to difficulties with movement and coordination.

Following this initial study and wider collaborations, the team has gone on to secure more than £10 million funding from the European Commission Horizon programme. Exercise interventions have been adapted for children and young people with autism spectrum disorder, attention deficit hyperactivity disorder (ADHD) and developmental coordination disorder (DCD).

A key focus now is the use of 'exergaming' – a gamified exercise platform delivered through virtual reality. This is designed to make physical activity more enjoyable, accessible and motivating for neurodiverse children, while helping to break long periods of inactivity and encourage exercise. Further developments aim to help children with Duchenne muscular dystrophy and ataxia.

Over the next five years, the team aims to develop AI-driven, personalised exercise programmes for use in the NHS. In the longer term, the plan is to expand activity to include wheelchair users worldwide.

Professor Dawes says, "This pilot study was an important first step towards proving that scheduling regular breaks in the school day for physical activity sessions could have wide-ranging benefits, improving children's academic performance, strength, mobility and their overall health and wellbeing."

This research was co-funded with the Chartered Society of Physiotherapy Charitable Trust.

30,000
children in the UK have cerebral palsy

> **"This study has now led to changes in practice and the development of novel inventions to improve the lives of thousands of children across the world.**
>
> Professor Helen Dawes

PROGRESS MADE

A rapid test for inflammatory bowel disease

Action funding has helped to develop the basis for a new blood test to improve diagnosis and guide doctors in selecting the best treatment for each child.

Around 500,000 people in the UK are living with inflammatory bowel disease (IBD), including Crohn's disease and ulcerative colitis – and up to one in five are diagnosed in childhood.

Diagnosis currently relies on invasive hospital procedures under general anaesthetic, which can be distressing for children and families.

With Action support, researchers at the University of Oxford have confirmed specific markers in the blood that can diagnose IBD in children very accurately. These markers can also help doctors identify children who need more intensive treatment.

As a result of this research, a rapid blood test is being developed with input from Oxford Nanopore Technology, a spin-out company of the University of Oxford, which delivers new technologies to the market. This could speed up diagnosis, reduce invasive procedures and support more personalised care for children with IBD.

> **"This test will reduce the time from when symptoms appear to diagnosis and help to identify children who do not require invasive investigations.**
>
> Professor Jack Satsangi

Around **500,000** people in the UK are living with IBD

NEW RESEARCH

Predicting spine fractures in Duchenne

Children with Duchenne muscular dystrophy, which causes muscle wasting, are sadly also at increased risk of osteoporosis. This means bones can become weak and prone to fractures.

With Action funding awarded in 2025, Dr Sarah McCarrison, at the Royal Hospital for Children, Glasgow, is developing a tool to predict which young people are most likely to experience spinal fractures, so their care can be more personalised.

Spine fractures can be painful, difficult to detect, and cause long-term back problems. But medicines to strengthen bones can have significant side effects, so are usually only used after a fracture has occurred. Earlier treatment could help prevent fractures, but doctors cannot yet identify who would benefit most.

This research will analyse clinical records and spinal scans from boys with Duchenne to develop a risk scoring tool. This will then be tested in a group of patients. The aim is to offer early treatment to those at high risk while avoiding unnecessary medication for others.

Supported by a generous gift left in Moira Carle's will.

Protecting boys like Digby

Digby was diagnosed with Duchenne muscular dystrophy at just two years old. Now aged 10, the risk of spinal fractures is a huge concern, as his mum, Lisa, explains.

Reduced physical activity and the use of long-term steroids – the standard treatment for preserving muscle function – means that boys like Digby face a higher risk of developing weak bones.

"Digby has annual bone density scans, and these often feel a long time coming," says Lisa. "We rely heavily on them for news – and we live in trepidation between these checks."

"The need to remain on long-term steroids puts so much strain on boys with Duchenne, and the inevitability of bone damage is a hard consequence for us to swallow. We need all the support we can get and in the main this usually comes in the form of reactive, not proactive, measures – so a risk assessment tool would be amazing."

> **"A proactive risk assessment tool would be something the Duchenne community would welcome.**
>
> Digby's mum, Lisa.

Digby

100
boys are born with Duchenne muscular dystrophy each year in the UK

NEW RESEARCH

Closing the treatment gap for cystic fibrosis

New medicines have transformed life for many children, but they still don't work for all. New research hopes to ensure every child has the best chance possible.

Though recent years have seen many improvements in treatments for cystic fibrosis (CF), it remains an incurable and life-limiting condition. Children with CF build up thick, sticky mucus in their lungs and digestive system. This can make it hard for them to breathe and digest food and leaves them very vulnerable to serious chest infections.

Medicines called CFTR modulators have transformed life for many children by targeting the underlying cause of CF. But sadly they do not work for all, leaving around one in 10 children in urgent need of new options.

Dr Guy Moss and his team at University College London are testing existing drugs that could help boost the effectiveness of current treatments for CF. Importantly, the team will also look for new drugs that work in different ways to help children who do not benefit from CFTR modulators.

Both projects on this page are funded in partnership with Cystic Fibrosis Trust. Since 2014 our two charities have invested over £1.2m into seven joint studies to help children with cystic fibrosis.

"

Our goal is to help more children with cystic fibrosis to live longer, healthier lives.

Dr Guy Moss

Helping families with an unclear diagnosis

Newborn screening helps to detect cystic fibrosis early, but some babies receive inconclusive results, creating uncertainty about their future health.

For these babies, the heel-prick test suggests they have cystic fibrosis (CF), but follow-up tests do not confirm a diagnosis. While most children will remain healthy, a small number may develop CF or a related condition as they grow older.

Doctors will closely monitor these children to spot early symptoms, but this must be balanced against the risk of unnecessary medical care for those who are well.

Dr Rebecca Dobra at Imperial College London is leading research to identify ways to predict long-term outcomes. This study will follow children with inconclusive results and compare them with healthy children. Using non-invasive tests and advanced imaging, the team will look for the earliest signs of lung damage.

Their findings could help to shape future guidelines – and reduce anxiety for families, ensuring children most at risk receive timely, targeted support

New treatments have made such a difference for Sophia

The latest cystic fibrosis drugs have been 'a blessing' to Sophia and her family – but they are acutely aware that not all children benefit, making research to change this crucial.

Diagnosed with cystic fibrosis (CF) at just two weeks old, daily life for Sophia has always revolved around keeping her well. This means physiotherapy and nebulisers to help clear mucus from her lungs, enzyme tablets with each meal to help her absorb nutrients, and often antibiotics to fight infection.

In recent years, medicines called CFTR modulators have been a game-changing development for many with CF. Rather than just managing the symptoms, these help treat the underlying cause. But they don't work for everyone.

"Different variants of CF are driven by different gene types," explains Sophia's mum, Sarah. "We are fortunate that Sophia has the most common type. Some children still don't have the same options, and that's horrible. There is guilt attached to it, because you feel blessed that your child can have these drugs, while knowing others can't."

For Sophia, now seven, these new treatments have improved both her health and overall quality of life.

"I can't remember the last time Sophia took an antibiotic, which is huge" says Sarah. "She'd previously ended up on IV drugs due to winter bugs that nothing else would clear. When Sophia does now get ill, it's not setting her back like it was before and not causing long-term damage."

It has also meant Sophia doesn't need to do her nebulisers as often, something which had become a big source of friction.

"Sophia doesn't stay still, so getting her to sit and do nebulisers was a battle. Now she usually only needs one daily treatment unless she has cold symptoms – and now she will actually recognise when more is needed, which is massive for her."

Sophia

> **"All those things that feel so scary about CF feel a little less daunting when you know they're on a medication that's stopping them from getting ill all the time.**
>
> Sophia's mum, Sarah

3,900+
UK children are living with cystic fibrosis

PROTECTING CHILDREN'S SIGHT

Childhood sight loss and vision problems can have many causes. These include genetic conditions, injury, being born prematurely or having a learning disability. We fund research to help preserve and protect children's vision.

Improving treatment for children with a lazy eye

Research funded by Action is set to update clinical guidelines around the world, ensuring more children get the best possible treatment to improve their sight.

Amblyopia, often called lazy eye, is the most common condition linked to vision problems in childhood. Affected children have better vision in one eye than the other, relying heavily on their 'good' eye to see.

In the UK alone, up to 5% of children have a lazy eye. It's estimated that around 90% of the work carried out by children's eye services is related to managing and treating this condition.

For more than 250 years, the main treatment has been to patch the stronger eye to improve vision in the weaker one. Currently, children are usually given glasses first, before patching starts. However, there was debate around how long children should wear their glasses before using a patch.

With support from Action, researchers at the University of Leicester carried out the first ever randomised controlled trial of eye patching for lazy eye.

They compared a longer period of wearing glasses, against a shorter period and earlier patching. This involved more than 300 children, from 30 hospitals, across five European countries.

The study found that patching sooner is better for most children. However, younger children with milder lazy eye may benefit from wearing glasses for longer before patching. These results are set to update clinical practice.

The researchers have published a suggested treatment approach that tailors the type of treatment to each child. They now aim to embed their new recommendations into clinical guidelines. This should see more children getting the treatment most likely to work best for them, leaving fewer with lifelong sight problems.

90%

of work by children's eye services in the UK is related to lazy eye

> **"We hope our findings will help children around the world to receive optimised treatment.**
>
> Dr Frank Proudlock

NEW RESEARCH

Helping children with learning disabilities to wear their glasses

With Action funding, researchers are developing a package of practical strategies to support children with learning disabilities to wear their glasses more successfully.

Children with learning disabilities are much more likely to have vision problems than other children and often need glasses to see clearly. Wearing their glasses consistently is important to support the development of healthy vision and prevent permanent sight loss. But for many children, this can be difficult.

Parents say the main problems for children are coping with the sensation of glasses on their face and not understanding why they need to wear them.

Dr Emma McConnell, of Queen's University Belfast and Belfast Health and Social Care Trust, is leading a team who will work with families.

They will develop and test practical strategies to help children with learning disabilities to wear their glasses more successfully.

These findings will inform the design of a larger clinical trial, laying the foundation for wider implementation in the future.

By making it easier for children to use their glasses, this project aims to support learning, confidence and long-term eye health.

349,000
UK children have a learning disability

> "By helping children wear their glasses more consistently, we hope they will see more clearly, engage better at school and enjoy an improved quality of life.
>
> Dr Emma McConnell

Finding ways to prevent genetic sight loss in children

Dominant optic atrophy can cause progressive sight loss from early childhood. Researchers aim to identify key genes involved, to reveal potential treatment targets.

Dominant optic atrophy (DOA) is a leading cause of inherited blindness in children, affecting at least 1 in 25,000 people in the UK. It occurs when the optic nerve, which carries visual information from the eye to the brain, gradually deteriorates.

Children with DOA typically begin to lose central vision – crucial for recognising shapes, colours and fine details – before their 10th birthday. There are currently no treatments to stop or reverse this vision loss, and many affected children are eventually registered as legally blind.

Around two-thirds of cases are linked to faults in the *OPA1* gene, which is essential for healthy mitochondria – the parts of cells that produce energy.

When mitochondria do not function properly, energy-demanding cells like those in the optic nerve can die prematurely.

With Action funding, researchers at the UCL Institute of Ophthalmology are working to better understand why these nerve cells die. Using an innovative 'optic nerve in a dish' model, created from patient cells, they are testing thousands of genes to identify those involved in cell survival. The aim is to uncover new targets for drugs that could help to preserve and prolong vision in children with this condition.

Generously supported by the Albert Gubay Charitable Foundation.

Children with DOA can begin to lose vision before they are 10 years old

> "While severity varies, many individuals with DOA will eventually be registered as legally blind. Our goal is to identify genes that could be targeted to slow or prevent optic nerve cell death.
>
> Professor Mike Cheetham

TACKLING RARE DISEASES

Thousands of UK children are living with a rare disease for which there is no cure or few treatment options. Research we fund helps to unlock vital medical breakthroughs for these forgotten families.

Gene therapy gives hope to girls with Rett syndrome

Rett syndrome causes severe disability, but clinical trials are beginning to offer families new hope. Early Action funding helped pave the way to exciting progress now being made.

Rett syndrome is caused by a fault in a gene that's critical for normal brain functioning. It usually affects girls, with symptoms starting subtly, around six to 18 months after birth. Sadly, these worsen over time, leading to severe physical and learning disability.

In 2009, Action Medical Research awarded over £180,000 to support the search for new treatments. Professor Sir Adrian Bird and his team at the Wellcome Trust Centre, University of Edinburgh, had previously generated early evidence that symptoms of Rett syndrome might be reversed if healthy gene function could be restored.

With Action funding, they took the next step. They used viral vectors – harmless viruses modified to carry healthy genes – to deliver a corrected version of the faulty gene into brain cells, and showed that this could successfully reverse symptoms in the laboratory. In collaboration with researchers in the US, they then showed that injection of their vector into the bloodstream could give more effective results.

This work, published in 2013, generated initial proof of principle that gene therapy held promise to treat, and potentially even cure, Rett syndrome. It also laid the foundations for further advances, including a 'mini-gene' approach that was also effective and easier to deliver.

Excitingly, the first clinical trials of gene therapies in patients were launched in 2023 in the US and Canada. One of these has adopted the mini-gene approach pioneered by the Edinburgh team. Initial results show promise, bringing the possibility of life-changing treatment closer than ever before.

This research was supported by a generous grant from The R S Macdonald Charitable Trust.

> **"Action played a part in developing the story towards clinical trials, for which the charity deserves significant credit.**
>
> Professor Sir Adrian Bird

Molly, who has Rett Syndrome

PROGRESS MADE

Unlocking treatments for Vici syndrome

Researchers have made important findings to better understand this rare disease, opening up promising new pathways towards future treatments.

Vici syndrome is a very rare and severe genetic condition that affects many different parts of the body. There is currently no cure and sadly the condition is life-limiting.

With support from Action, researchers at University College London have recently made significant discoveries about how Vici syndrome affects cells in the body. Crucially, they have identified multiple biological pathways that could be targeted with drugs. Early laboratory studies have found that drugs aimed at these pathways could help to restore cell function. This has revealed new possibilities for treatment, opening the door to future clinical trials.

Professor Michael Duchen says: "Our findings have been very exciting, and could ultimately lead to new treatments to help reduce the impact of Vici syndrome, and related diseases."

Supported by a generous gift left in Joyce Edmund's will.

Helping future families like Emmy's

Emmy was diagnosed with Vici syndrome at 18 months old. She is blind, tube-fed and unable to walk or talk, but her personality shines through, says mum Ellie.

Ellie had feared there was something wrong from Emmy's earliest days. She'd struggled to feed, didn't track objects with her eyes, and showed little interaction. By the time she was 18 months old, the family knew Emmy faced serious health challenges. But they were 'completely blind-sided' by her devastating diagnosis.

"We really didn't think there could be anything so catastrophically wrong," says Ellie. "Now, we take each day as it comes. Moments of love and resilience keep us going."

> **"We are grateful for the research teams investigating Vici syndrome and other rare and debilitating conditions. This research could be incredibly significant.**
>
> Emmy's mum, Ellie

Emmy

NEW RESEARCH

Investigating Rasmussen's encephalitis

This rare disease typically strikes previously healthy children under 10, causing severe, progressive inflammation in one side of the brain.

Seizures are usually the first sign of Rasmussen's encephalitis and become increasingly frequent. Over time, children develop worsening learning difficulties and weakness on one side of the body that can lead to complete paralysis. There is no cure – and by the time most children are diagnosed, irreversible brain damage has already occurred.

Current treatment focuses on controlling seizures and reducing inflammation. In many cases, the only way to stop seizures and prevent further cognitive decline is a major operation to disconnect the affected half of the brain. But this can have life-changing consequences, such as the permanent loss of vision or speech.

Dr Eva Ioannidou is investigating the underlying biological mechanisms driving this devastating condition. Her aim is to enable earlier diagnosis and reveal new treatment targets.

Dr Ioannidou is an Action and British Paediatric Neurology Association Research Training Fellow

Making discoveries to help children like Lilly-May

Lilly-May was an active, healthy little girl until the summer of 2018, when everything changed almost overnight. Within just a few months, she needed life-saving brain surgery.

Then aged three, Lilly-May had suddenly started to suffer severe seizures. She was repeatedly admitted to hospital, but doctors struggled to work out what was wrong, and different medications didn't seem to help.

Lilly-May deteriorated frighteningly quickly. Soon she was unable to eat, drink, walk or talk independently, and needed round-the-clock care. Following a brain biopsy, she was finally diagnosed with Rasmussen's encephalitis. Doctors hoped to be able to delay major surgery, but she became critically ill. "We were told she had 24 hours to live," recalls her mum, Carly.

A complex, 18-hour operation saw surgeons disconnect the two halves of Lilly-May's brain to stop the disease spreading to the healthy side. This saved her life, but it was only the beginning of a long recovery.

Now 11, Lilly-May still lives with the effects of the disease and surgery. She has weakness on one side of her body, wears a leg splint and has a visual impairment. She also has limited use of her hand, and still has seizures, though these are controlled with medication. Carly says her daughter's strength continues to inspire those around her.

Lilly-May

> **"If doctors can understand why this disease happens and how to treat it earlier, it could drastically change the future for children like Lilly-May.**
>
> Lilly-May's mum, Carly

FIGHTING CHILDHOOD CANCER

Thanks to medical advances, most children with cancer can be cured. But treatment is usually long and intensive, and some cancers remain difficult to treat. We fund research to develop more effective, kinder treatments — to give every child the best possible chance of a healthy future.

Action funding helps advance leukaemia treatment to clinical trial

Researchers have moved a vital step closer to a new treatment for children fighting drug-resistant T-cell acute lymphoblastic leukaemia.

Leukaemia is the most common cancer affecting children and young people, impacting around 700 families in the UK each year. Nearly 100 of these children are diagnosed with T-ALL, an aggressive form of the disease that can stop responding to treatment.

While most children with T-ALL will be cured, sadly some don't respond to treatment or find their cancer returns. There are very limited options for those with drug-resistant or relapsed T-ALL, and these children often lose their lives.

In 2018, Action awarded funding of more than £200,000 to Dr Frederik van Delft and his team, based in Newcastle and Glasgow.

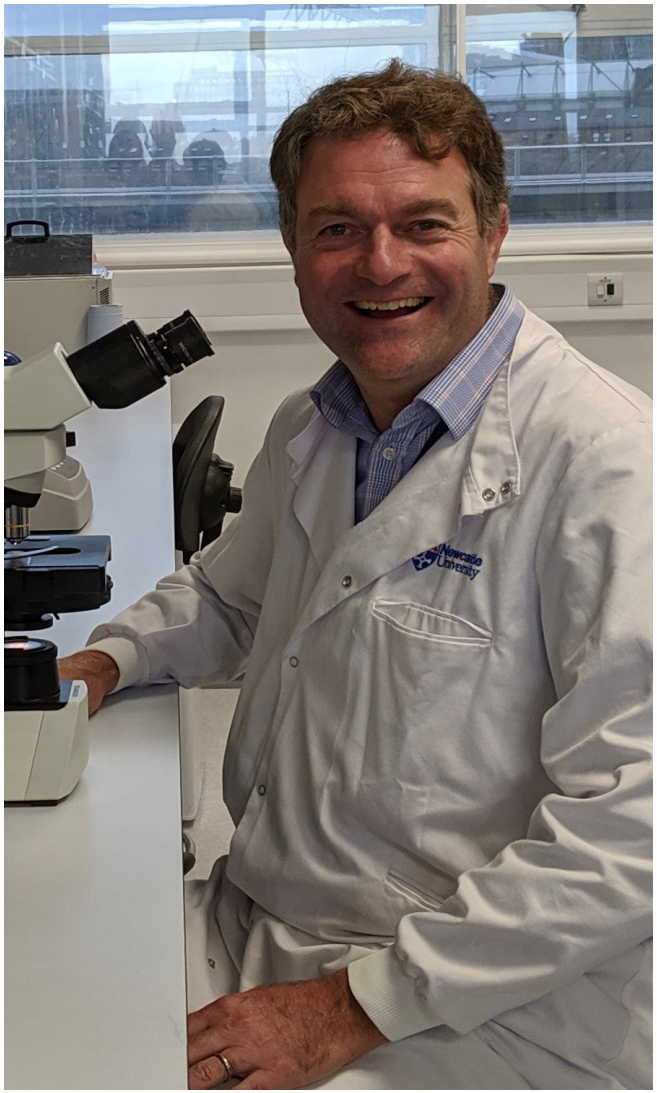
They had early laboratory evidence that combining two existing drugs could overcome treatment resistance and kill T-ALL cancer cells.

With Action's support, the team were able to confirm that their drug combination was more effective than either drug used alone.

This research, alongside work by other groups in the field, has now led to the new combination being included in an international trial of different drugs to treat children with resistant or relapsed T-ALL. Five hospitals across the UK will take part in delivering this trial, due to start in 2026.

As part of the project, the team also developed a new laboratory system to test how different drug combinations affect leukaemia cells taken from patients. This means researchers can focus more quickly on the most promising treatments – helping to speed up progress towards better outcomes for children.

We are grateful to the Team Lewis Trust and other charitable trusts who generously supported this research.



> **"The Action-funded work confirmed that our drug combination looked very promising. A new clinical trial will now test how well it works in patients.**
>
> Dr Frederik van Delft

Testing drugs that block cancer cells

Funded again by Action, Professor Frederik van Delft's latest research continues to seek effective new treatment options for children with T-cell acute lymphoblastic leukaemia.

Using higher doses of chemotherapy could potentially help cure more children with T-cell acute lymphoblastic leukaemia (T-ALL), but this greatly increases the risk of life-threatening side effects. An alternative approach is to develop new, targeted drugs, such as those that work by blocking key proteins that help cancer cells survive treatment.

Unfortunately, a drug that targets these survival proteins and had shown promise in clinical trials is no longer available for children with T-ALL. This leaves doctors with even fewer treatment options and has slowed progress in developing new therapies.

In this new research, Professor van Delft and his team aim to quickly identify a suitable replacement. They have already identified some existing drugs that work in a similar way, but these require further testing to confirm their safety and effectiveness.

"Ultimately, this research could help lead to safe and effective new treatment options for children with T-ALL and help prevent more young lives from being cut tragically short," says Dr van Delft.

Fighting cancer in children

Action has a proud history of supporting research to advance the development of new and kinder treatments to fight childhood cancers.

Almost 4,000 children and young people under 25 are diagnosed with cancer each year in the UK. That's around 11 families a day receiving devastating news.

A cancer diagnosis is upsetting at any age but is especially so when the patient is very young. Sadly there are many different types of cancer that can affect children – and childhood cancers are not the same as adult cancers. The type of cancer, how advanced it is and how best to treat it can differ between children and adults. So it's important that we invest in research to help children with cancer.

There are also many types of cancer treatment, such as surgery, chemotherapy, radiation therapy, immunotherapy, and stem cell transplant. Often these treatments can have serious side effects.

So research is vital for identifying which children need intensive treatment, whilst sparing others from invasive treatments that can have life-changing side effects.

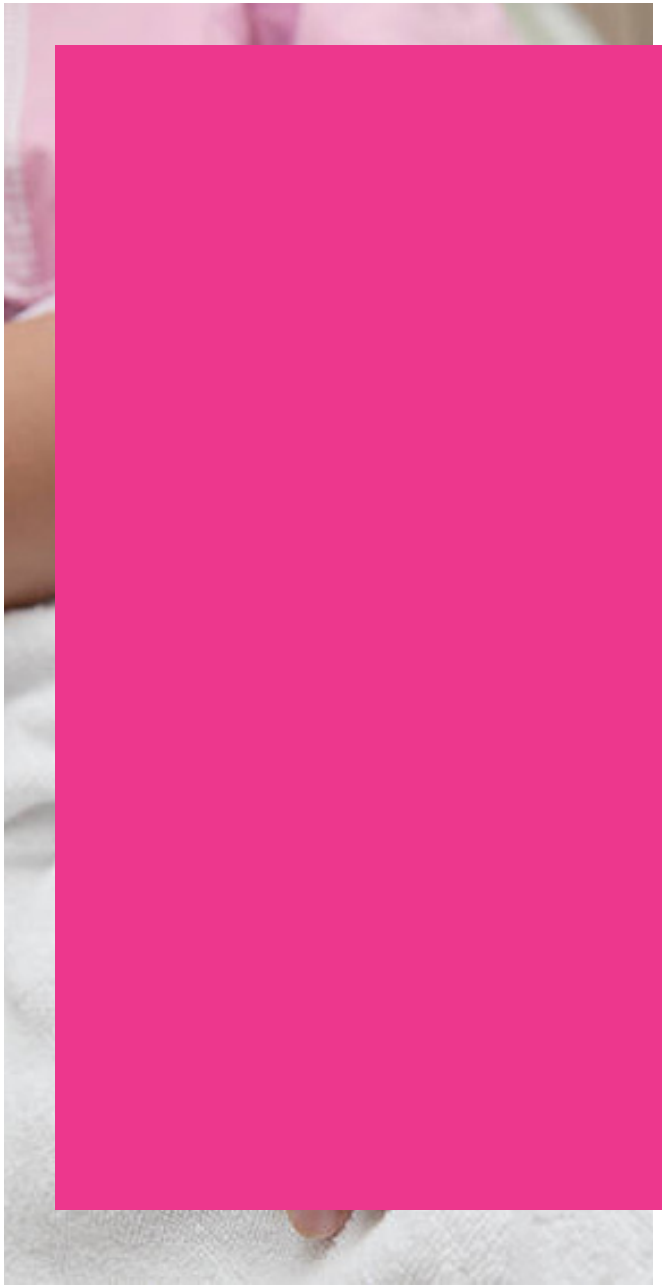
As well as funding work to find new treatments for leukaemia, we are also currently supporting research to develop an urgently needed new approach to fight the most lethal form of childhood brain cancer, diffuse midline glioma.

Our research successes in fighting cancer include:

- Finding new ways to diagnose and assess brain tumours in children. This work identified markers of tumour aggressiveness and developed new ways to assess these using non-invasive MRI scans. It means doctors can now tailor treatments for each child and reduce the risk of toxic side effects.

- Supporting work to develop and refine CAR T cell therapy for high-risk neuroblastoma. This is a promising form of immunotherapy that uses a patient's immune system to kill cancer cells.

- Developing guidelines to improve pregnancy care for childhood cancer survivors, who may be at greater risk of complications or a premature birth.



> **"We're blessed that we are a complete family and truly hope that further research will continue to help more families like ours to stay complete.**
>
> Kathryn, mum of Joey, who was successfully treated for a brain tumour

OUR 2025 NEED TO KNOW

Useful information about our funded research, legacies, grant listings, who's who and a thank you to our supporters.

Finding and funding high quality research

At Action Medical Research, we are committed to fighting for answers that can lead to breakthroughs, treatments and cures for some of the toughest fights children can face. We have over £11m invested in the work of around 200 top researchers, working on around 50 projects across the UK.

Our aim is always to fund only high quality research that is most likely to deliver real benefit for babies, children or young people. Our rigorous, gold standard scientific review process ensures that the charity funds some of the best doctors and researchers in children's hospitals, specialist units and universities across the UK.

Each year, our grants are awarded based on the recommendations of a Scientific Advisory Panel of world-class medical researchers, who also consider further opinions from UK and international experts in the field. This expert panel, supported by our peer reviewers, ensures we fund research that is judged most likely to make a difference. We are very grateful to all those who share their time and expertise as part of this important process.

Action is a founder member of the Association of Medical Research Charities (AMRC), the membership organisation of leading medical and health research charities in the UK. The AMRC assesses our peer review processes every five years and has awarded Action Medical Research a certificate of 'Best practice in medical and health research peer review'.

A lasting legacy

Gifts in wills are a vital source of income and we are incredibly grateful to those people who choose to show their support in this special way. A gift in your will could unlock a future medical breakthrough. It could help us find new treatments and cures that could change children's lives for years to come.

Thank you to those who have made provision for Action in their will. We would like to gratefully acknowledge the gifts in wills we received in 2025 from the following supporters.

Mary Black
Sally Blake
Elizabeth Brown
Moira Carle
Maureen Chapman
Alan Chisholm
Geraldine Comba
Madeleine Daly
Jean Daniel
Olga Dixon
Peter (Clifford) Draper
Annie Fletcher
Gwyneth Garner
Angela Griffiths
Frances Hill
Anna Jackson
Jill Kearley
Beryl Rowe
Pat Schwarzenbach
Margaret Williams
James Wilson

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

Research grants awarded in 2025

Action Medical Research is currently funding around 50 projects across the UK, 15 of them awarded in 2025. The next medical breakthrough could be on your doorstep.

Brain injury at birth – can melatonin or cooling therapy help in cases of mild newborn brain injury?

Dr Raymand Pang

University College London

Brain injury in newborn babies – improving prediction and diagnosis through a new cot-side scanner

Professor Topun Austin

University of Cambridge, Cambridge University Hospitals NHS Foundation Trust, Universisty College London

Cystic fibrosis – predicting long-term health outcomes for children with an inconclusive diagnosis*

Dr Rebecca Dobra

Imperial College London, Royal Brompton Hospital, Institute of Child Health, University College London, King's College Hospital, Royal London Hospital

Cystic fibrosis – developing new drug treatments to help improve the lives of children*

Dr Guy Moss

University College London, University of Bristol

Duchenne muscular dystrophy – developing a risk assessment tool to help predict spine fractures in young people

Dr Sarah McCarrison

Royal Hospital for Children Glasgow, University of Glasgow, Birmingham Women's and Children's Hospital, Newcastle University

Dominant optic atrophy – searching for potential treatment approaches to help prevent sight loss in children**

Professor Mike Cheetham

UCL Institute of Ophthalmology, University of Cambridge

Improving pain assessment in babies to support their care after surgery

Dr Roshni Mansfield

University of Oxford

Non-Ketotic Hyperglycinemia (NKH) – developing new treatments for life-limiting inherited metabolic disease

Professor Nick Greene

UCL Great Ormond Street Institute of Child Health

Polycystic ovary syndrome (PCOS) – could a food supplement help relieve symptoms for teenagers?

Dr Pallavi Latthe

Birmingham Women's and Children's Hospital, University of Birmingham, Aston University

Preterm birth – developing a new cell therapy to help prevent babies from being born too soon***

Professor Rachel Tribe

King's College London, St Thomas' Hospital

Preterm birth – investigating the causes to identify ways to prevent it***

Professor Andrew Copp

UCL Great Ormond Street Institute of Child Health

Preventing vision loss by testing strategies to improve glasses wear in children with learning disabilities

Dr Emma McConnell

Queen's University Belfast, Ulster University, Royal Victoria Hospital, Belfast

Rasmussen's encephalitis – investigating the underlying causes of this rare childhood brain inflammation to improve diagnosis and treatment****

Dr Eva Ioannidou

UCL Great Ormond Street Institute of Child Health, Great Ormond Street Hospital

T-cell Acute Lymphoblastic Leukaemia – laboratory testing of new blockers of T-ALL survival

Dr Frederik van Delft

Newcastle University

Viral respiratory infections – finding effective treatment for vulnerable children

Dr Claire Smith

UCL Great Ormond Street Institute of Child Health, Great Ormond Street Hospital, Great North Children's Hospital

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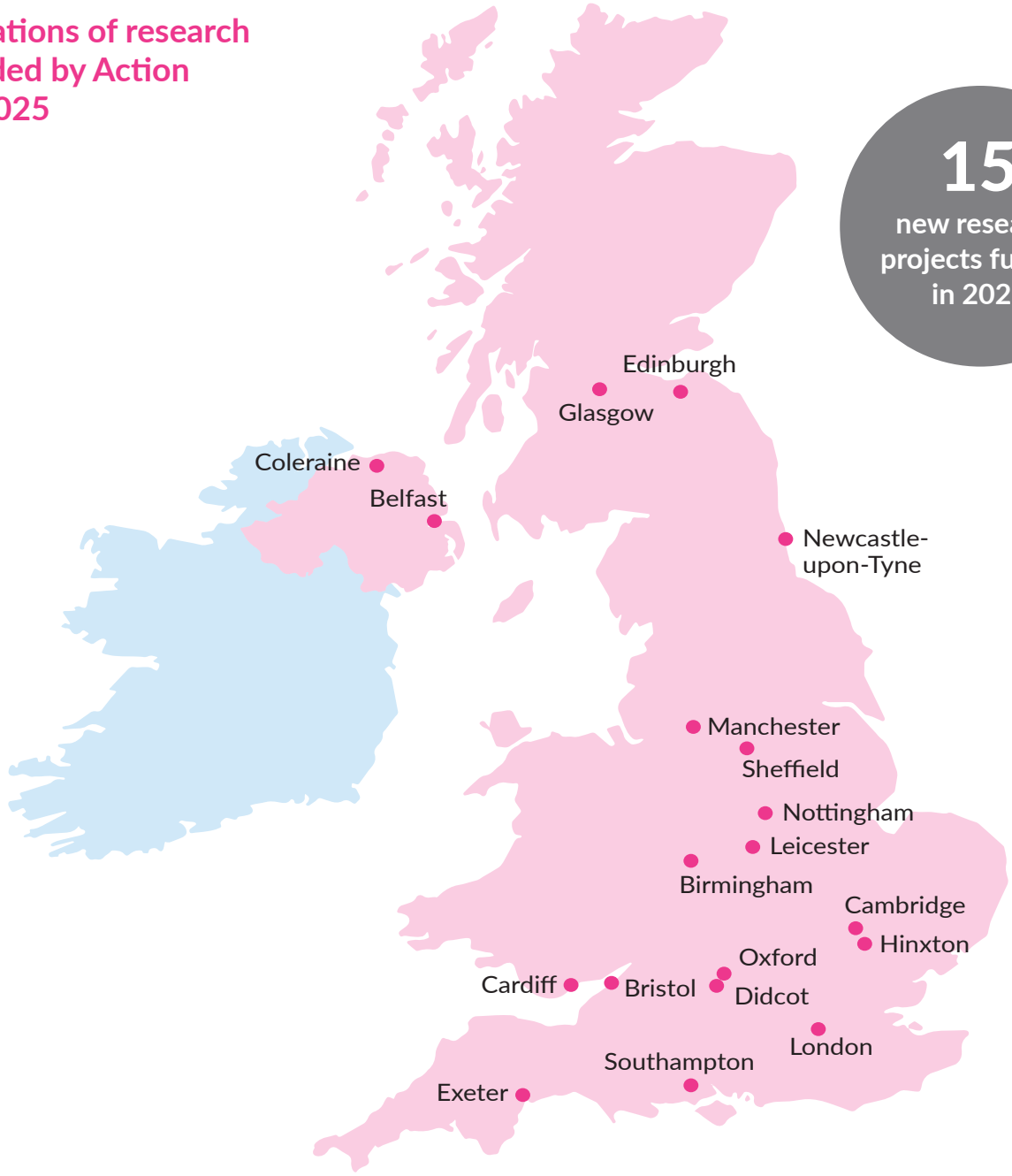
* *Jointly funded with Cystic Fibrosis Trust*
** *With generous support from The Albert Gubay Charitable Foundation*
*** *Jointly funded with Borne*
**** *Jointly funded with the British Paediatric Neurology Association (BPNA)*

Locations of research funded by Action in 2025



Who's who 2025

President

Field Marshal The Lord Guthrie GCB LVO OBE DL (deceased)

Vice Presidents

Phil Hodkinson

Stephen May

Richard Price

The Duchess of Northumberland

Trustees

Luke Bordewich, *Chair*

Richard Wild, *Honorary Treasurer*

Professor David Edwards MA MBBS DSc MRCR FRCP FRCPCH FMedSci

Clare Ferguson

Kathy Harvey

Karen Last

Bhavin Patel CFA *joined January 2025*

Professor David Rowitch MD PhD ScD

Professor Stephanie Schorge PhD

Richard Stoneham-Buck

Scientific Advisory Panel

Professor Stephanie Schorge PhD *(Chair)*

Professor Simon Bailey MBChB MRCP PhD

Professor Diana Baralle MBBS MD FRCP

Professor Joanne Blair MBChB MRCP MRCPCH MD

Professor James Boardman MBBS MSc PhD FRCPCH

Professor Adnan Custovic MD PhD FRCP FMedSci

Professor Deborah Gill PhD

Professor Ed Johnstone MBChB PhD MRCOG

Professor Juan Kaski MD(Res) FRCP FESC

Professor Pablo Lamata PhD

Professor Robert McFarland MA MBBS PhD MRCP FRCPCH

Associate Professor Nazima Pathan PhD FRCP

Professor Padmanabhan Ramnarayan MD FRCPCH FFICM

Professor Felicity Rose PhD FHEA

Dr Claire Thornton PhD

Professor Brigitte Vollmer Dr.med PhD FRCPCH

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.

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

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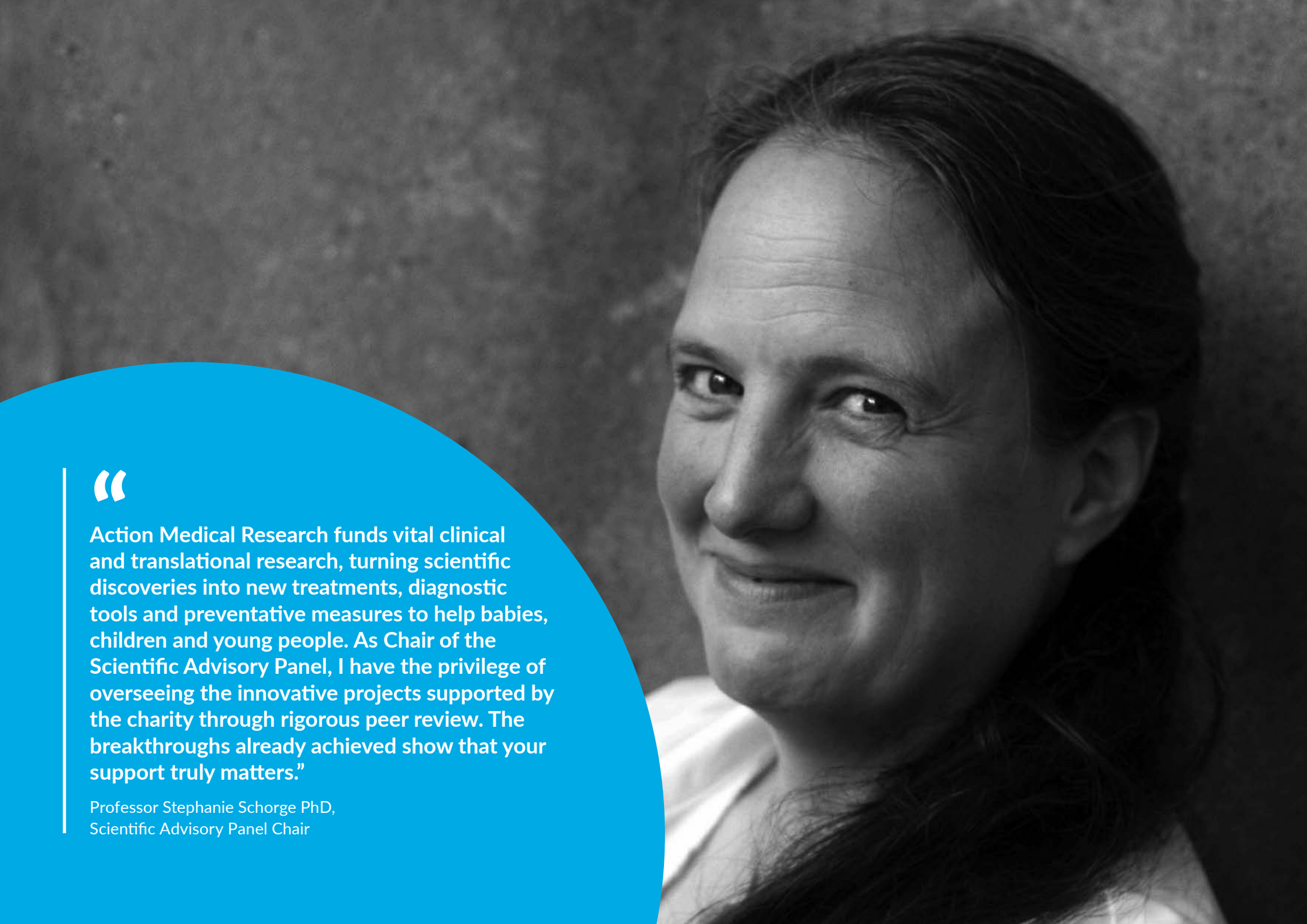

“Action Medical Research funds vital clinical and translational research, turning scientific discoveries into new treatments, diagnostic tools and preventative measures to help babies, children and young people. As Chair of the Scientific Advisory Panel, I have the privilege of overseeing the innovative projects supported by the charity through rigorous peer review. The breakthroughs already achieved show that your support truly matters.”

Professor Stephanie Schorge PhD,
Scientific Advisory Panel Chair

action medical research for children

We're the leading UK-wide charity dedicated to funding vital research to help sick babies and children.

Cover photo: Ben Rector
Photo page 4 OndroM / page 14 background to photo triocean / page 19 Lifestyle Travel Photo

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