action medical research for children

Research Review

Saving and changing children's lives | 2024

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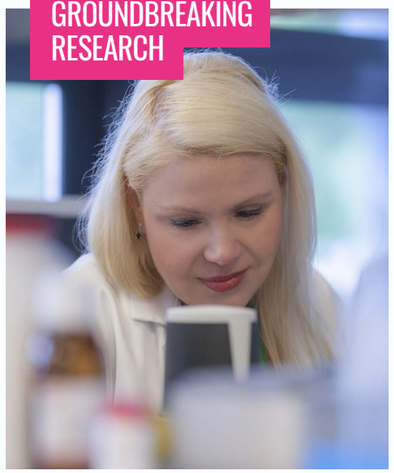
SAVING TINY LIVES



GROUNDBREAKING RESEARCH

MAKING A DIFFERENCE FOR CHILDREN

HELPING THE MOST VULNERABLE

action medical research for children

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WELCOME

At Action Medical Research, we are dedicated to saving and changing children's lives through groundbreaking medical research. The projects we fund lead to new treatments and breakthroughs, as you'll see in the pages that follow.

In 2024, we funded 14 new research projects across the UK, helping babies, children and young people affected by a range of conditions. These included epilepsy, premature birth, Duchenne muscular dystrophy, and rare neurodevelopmental disorders. This work offers real hope to many families.

Right now, we have over £11 million invested in the work of around 190 top researchers, supporting around 55 projects across the UK. Our partnerships with other charities continue to play a vital role, and we were delighted to fund five new projects in collaboration with LifeArc, focusing on rare diseases in children.

We also awarded three new Research Training Fellowships, through our scheme to support talented early-career doctors and researchers as future leaders in children's health. Their work includes studying brain development in preterm babies, reducing infection risks from breathing tubes, and exploring treatments for rare immune system disorders. We have now awarded 192 Fellowships to date.

Each year, we review the impact of previously funded research, and you'll find progress updates throughout this Research Review. One example, on page 5, shows how research we funded in 2016 has now led to clinical trials of two drugs with the potential to help children and young people with neurofibromatosis type 2.

Other exciting progress includes a simple play-based intervention, shown to improve learning and cognitive development in very premature babies; improved understanding of a rare type of anaemia, already enabling less painful, faster diagnosis for children; and a new blood test that could help prevent sudden deaths caused by a rare heart condition.

Action is truly proud to be saving and changing children's lives. None of this would be possible without the support of many companies, trusts, foundations, groups and individuals, as well as the expert guidance of our Scientific Advisory Panel. We remain ever grateful to all of our supporters, including our network of local committees and volunteers, as well as those who so generously remember Action with gifts 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.

Julie Buckler Chief Executive
Action Medical Research for children

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

FIGHTING FOR CHILDREN WITH 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.

PROGRESS MADE

DEVELOPING A NEW TREATMENT FOR NEUROFIBROMATOSIS TYPE 2

This rare condition causes multiple tumours, typically on nerves, the spine and around the brain. Helped by past Action funding, 2024 saw the start of an exciting clinical trial in patients, and a much-needed treatment option is now on the horizon.

Neurofibromatosis type 2 (NF2), also known as NF2-related schwannomatosis, is a genetic condition that causes slow-growing tumours in the nervous system. While these are not usually cancerous, their locations can cause serious symptoms and make them very difficult to treat.

NF2 can cause hearing loss, problems with balance and mobility, pain and paralysis. Sadly symptoms tend to worsen over time and life-threatening complications can develop.

There is currently no cure and treatments such as surgery or radiotherapy are often only partially effective, and tumours can recur. Cancer drugs may also help, but these can have serious side effects.

OUR IMPACT

In 2016, we awarded funding to a team led by Dr Sylwia Ammoun at the University of Plymouth. This was to continue work to explore the role certain virus proteins in the body played in the development of tumours linked to NF2, and to test if antiviral drugs could offer a new treatment option.

This Action-funded research confirmed that viral proteins do contribute to the growth of NF2 tumours and, excitingly, that existing drugs, already used to treat HIV, could reduce and slow the growth of these tumours in the laboratory.

"The grant from Action made all the difference," says Dr Ammoun.


Dr Sylwia Ammoun

"It allowed us to continue the research – and these promising results have now helped lead to a clinical trial to test if these drugs can be repurposed to treat NF2," explains Dr Ammoun.

This first trial involves patients over 18, with younger people to be involved in future tests. "NF2 develops in childhood, so it will be of great importance to test these drugs in children," concludes Dr Ammoun.

> **"The grant from Action allowed us to continue the research – and our promising results have now helped lead to a clinical trial in patients"** Dr Sylwia Ammoun

HOPE FOR CHILDREN LIKE OSCAR

Oscar is 13 and currently has 10 tumours caused by NF2. These include one in the brain, two growing on the hearing nerves, and several spinal tumours that have caused severe problems in his left leg and foot.

His mum Jo says: "Children with NF2 deserve a better future – one without fear of where the next tumour will pop up and which nerve or sense it will take. These new drugs could be a game-changer for families."

75%
OF RARE DISEASES AFFECT CHILDREN

PROGRESS MADE

TOWARDS NEW TREATMENTS FOR RARE ANAEMIA

Research that finished in 2024 has given important new insights into the underlying biology of a rare type of anaemia. This is set to improve diagnosis and treatment for affected families.

Congenital dyserythropoietic anaemia type 1 (CDA-1) affects the development of red blood cells and is usually diagnosed in childhood. It can cause weakness, shortness of breath and headaches, as well as organ damage leading to heart failure, diabetes and liver disease.

Treatment involves regular blood transfusions or injections with interferon. However, both can cause serious side effects – and interferon doesn't always work.

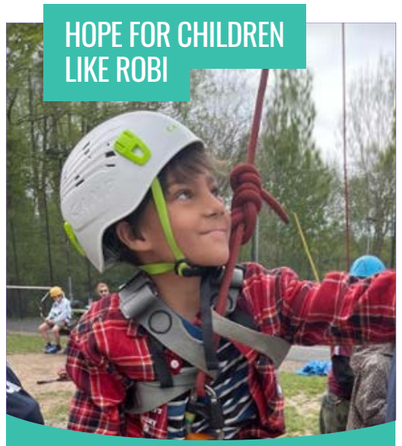
With Action funding, Dr Chris Babbs and his team at the University of Oxford studied the underlying cellular activity that causes this disease. They identified new biomarkers, meaning children can now be diagnosed more quickly and easily, reducing reliance on invasive bone marrow tests. These biomarkers could also help lead to the discovery of new and improved treatments.

The team also shed new light on how interferon treatment works, with their findings suggesting that lower doses could be effective and with fewer side effects.

This research was supported by a generous legacy gift from Joyce Edmunds.

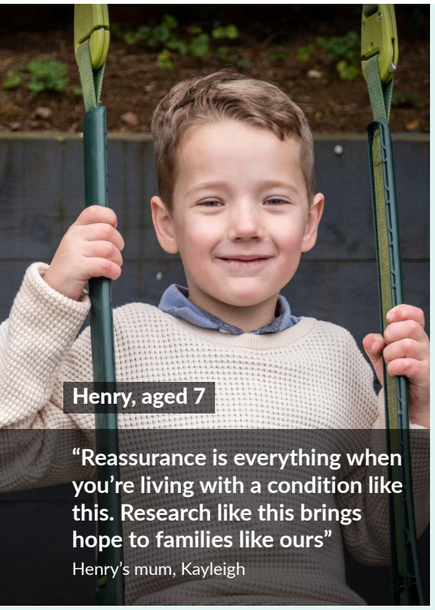
> "This funding was an amazing boost for an area where there is great unmet clinical need" **Dr Chris Babbs**

HOPE FOR CHILDREN LIKE ROBI



Robi was diagnosed with CDA-1 anaemia when he was just nine months old, following a bone marrow biopsy. Now 12, he has had blood transfusions or interferon injections his whole life. At times he has needed to switch treatments due to the side effects.

His mum, Resina, says: "This research gives us a lot of hope and could have a positive impact for Robi. We are very grateful for the work to learn more about this very rare condition and how it may be treated."



Henry, aged 7

"Reassurance is everything when you're living with a condition like this. Research like this brings hope to families like ours"
Henry's mum, Kayleigh

HEART DISEASE TEST BREAKTHROUGH

Action funding has supported the development of a new blood test that could help to prevent sudden death caused by a rare heart condition.

Hypertrophic cardiomyopathy causes thickening of the heart muscle. This can become life threatening – and it is sadly a leading cause of sudden death in young people. It is estimated to affect around 1,000 children in the UK, but there may be many more who are undiagnosed.

With funding from Action and LifeArc, Professor Juan Pablo Kaski and his team, based at University College London and Great Ormond Street Hospital, have shown that their test can successfully detect proteins in the blood that are a marker for this disease.

Crucially, the new test can also identify four specific proteins which may indicate cases where there is greater risk of sudden death. It is hoped this will now be developed for use across the NHS.

The results of this research were published in 2024 and featured on *Channel 4 News*.

NEW RESEARCH TO HELP CHILDREN WITH RARE DISEASES

Researchers are testing combining new gene therapies with existing medications to enhance their effectiveness. This could unlock their full potential, transforming treatment for this life-limiting disease.

Duchenne muscular dystrophy causes severe and progressive muscle weakness. It almost always affects boys and there is no cure. By their early teens, most will need to use a wheelchair – and sadly most only live into their 20s or 30s.

The condition is caused by a faulty gene, meaning the body doesn't make enough, or any, dystrophin, essential for keeping muscles healthy. New gene therapies aim to restore dystrophin, but their effectiveness has so far been limited.

Earlier research suggested that combining gene therapies with an existing type of drug could further boost dystrophin production. Co-funded by Action and LifeArc, researchers are now testing this approach on patient cells.

Led by Professor Francesco Muntoni at UCL Great Ormond Street Institute of Child Health, they are studying combinations to see which works best. If successful, this could transform the lives of affected children and their families.

This research is supported by a generous legacy gift from the family of Dr Daphne Sprague.

Around 100 BOYS ARE BORN WITH DUCHENNE MUSCULAR DYSTROPHY EACH YEAR IN THE UK

TESTING THERAPIES FOR NEURODEGENERATIVE DISEASES

Wolfram syndrome and Niemann-Pick type C1 are devastating conditions with no cures. New research could help slow the progression of symptoms.

These two conditions both involve the gradual loss of nerve cells, causing severe symptoms affecting the brain and central nervous system. Sadly there is no effective treatment for either, and affected children's lives are often cut short.

Both are linked to problems with the body's natural process for clearing cellular waste. When this malfunctions, unwanted materials build up and cause damage.

With funding from Action and LifeArc, Dr Sovan Sarkar and his team at the University of Birmingham are testing existing therapies, used for other conditions, that could help halt or slow the progression of symptoms.

They are using nerve cells and 'mini-brains', generated from patient skin cells grown in laboratory dishes, to assess two treatments.

HELPING CHILDREN LIKE TOM

Tom is 16 and has Wolfram syndrome. He is now visually impaired and has hearing loss. He is also autistic and has diabetes.

"Research is the only thing that keeps families going as they watch their child suffer – the hope that things will get better," says his mum, Andrea.

FINDING MEDICINES TO HELP CHILDREN LIKE TORA

Tora is 10 and lives with the rare disease neurofibromatosis type 1. This can cause a wide range of symptoms and treatment options are very limited. Children like Tora can face daily challenges and the future can feel very uncertain.

NF1 affects around 1 in 3,500 people. Symptoms can vary greatly and develop gradually. As children get older, they often develop multiple non-cancerous tumours growing on or under the skin and along nerves. These can cause serious complications and sadly in some cases become cancerous. Learning and developmental issues are also very common.

Tora was diagnosed when she was a year old. Her mum Lisa, a GP, had noticed a cafe au lait spot when she was just days old. These light brown skin patches can be a sign of NF1 – although Lisa admits that it was only her medical knowledge that made her consider this possibility. "If I was non-medical, the diagnosis would have probably taken many years," she explains.

Lisa was initially dismissed as an anxious first-time mum. But when another spot appeared, she strongly requested a referral for genetic tests. The results, when they finally came, confirmed her worst fears.

"I remember the phone call like it was yesterday," says Lisa. "As a doctor, I knew what NF1 was, but I was desperate for the test to be negative. After the call, I cried until my body ached. I didn't sleep properly for weeks."

Around 95% OF RARE DISEASES LACK AN EFFECTIVE TREATMENT

Thank you to charity Nerve Tumours UK for putting us in contact with Tora and Lisa.

As a toddler, Tora had to learn to cope with frequent distressing procedures, such as blood tests, cannulas and MRI scans. She was under nine different medical specialties.

Most frightening of all was the discovery of an optic pathway glioma – a slow-growing tumour occurring in or around the optic nerve. This was found when Tora was three and has thankfully so far remained stable.

"The biggest difficulty at the moment is how the condition affects her experiences in school," says Lisa. Tora has dyspraxia and hypermobility, which affects her strength, coordination and balance. As well as these physical challenges, she has significant dyslexia and dyscalculia. Managing the emotional pressures of living with NF1 has become a top priority.

Thinking about the future, Lisa worries about the risk of certain cancers. Her ultimate hope is for a cure, or to be able to predict and understand what could lead to worse symptoms.

In terms of the difference new treatments could make, she says: "It would be a huge weight off my shoulders."

Tora with mum Lisa

> **"Our fear is that treatment options are so limited. It's very difficult living in a situation where you have no idea how bad things could be and what would make a difference"** Tora's mum, Lisa

NEW RESEARCH

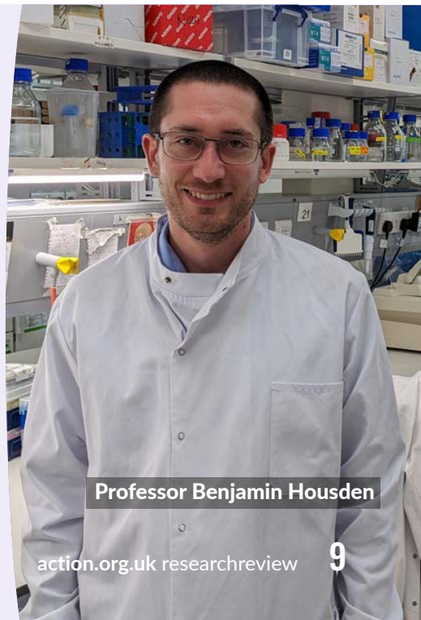
There is currently only one approved drug treatment for NF1, but it isn't always effective and can cause serious side effects.

With funding from Action Medical Research and LifeArc, Professor Benjamin Housden of the University of Exeter hopes to develop a new treatment option.

He is investigating the potential for re-purposing two drugs which are already used to treat other diseases.

These drugs have shown promise in killing NF1 tumour cells, but further work is needed.

"We hope our results will lay the foundations for clinical trials involving patients as soon as possible," says Professor Housden. "Since these drugs are already in use and their safety and dosage are established, this should help accelerate this process."



Professor Benjamin Housden

PREGNANCY AND CARING FOR 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.

PROGRESS MADE

PROTECTING SLEEP FOR PREMATURE BABIES

An Action Medical Research study that finished in 2024 has shown the importance of sleep cycles on brain development for babies born prematurely.

Advances in treatment have led to improved survival, but babies born prematurely still face an increased risk of long-term neurodevelopmental complications. There are many reasons for this, but evidence suggested an important relationship between babies' sleep and healthy brain development.

In the second half of pregnancy, a baby's brain undergoes rapid development, forming new connections and networks. At the same time, distinct sleep states emerge, with the baby cycling between so-called 'active' and 'quiet' sleep.

But babies born too soon are cared for in an environment that's very different to the womb. In neonatal intensive care there are bright lights and loud noises, and babies endure frequent, often painful, medical procedures.

All of this can interrupt natural sleep cycles – and this could have a significant impact on the development of healthy brain networks, ultimately affecting babies' long-term outcomes.

Supported by Action, researchers at Cambridge University Hospitals NHS Foundation Trust, developed a new way to study sleep cycles and their link to brain development in preterm and full-term babies.

Led by Professor Topun Austin, the team used a non-invasive imaging system, with a lightweight, flexible cap worn on the baby's head to track blood flow in the brain. This study was the first to demonstrate wearable technology for cot-side neuroimaging in newborn infants.

The team found significant differences in functional brain connectivity between neonatal sleep states. This has led to a follow-on study to continue this work, with the hope of developing a clinical system to promote sleep cycling and help brain development in these vulnerable babies.

"Sleep, although something all newborn babies spend a lot of time doing, is very under-researched. This could have a major beneficial impact on the way infants are cared for in neonatal units"

Professor Topun Austin

Around 55,000 BABIES ARE BORN PREMATURELY IN THE UK EACH YEAR

PROGRESS MADE

IMPROVING PREGNANCY CARE FOR CHILDHOOD CANCER SURVIVORS

Action-funded research has helped to better understand the risk of complications in some women treated for cancer in younger life. This is set to result in new guidelines for better care.

Thanks to advances in treatment, many children now survive a cancer diagnosis and eventually want to start families of their own. But it had been suspected that women who received a bone marrow transplant as a child or young adult were at greater risk of pregnancy complications.

With funding from Action and Borne, Dr Melanie Griffin and her team have worked to better understand this.

Their study confirmed that those who had bone marrow transplants, particularly when combined with total body irradiation, were at higher risk than usual of giving birth very early and having smaller babies.

The team are now working to share their findings so that national guidelines can be updated and those affected can access more specialist support.

"We are now advising that these women are seen in specialist preterm birth prevention services, which prior to this work hasn't been routinely happening," says Dr Griffin.

This project was generously supported by The James Tudor Foundation.

> **"Patient and parent groups we spoke to were really grateful that we have done this work"**
>
> **Dr Melanie Griffin**

More than 850 GIRLS ARE DIAGNOSED WITH CANCER EACH YEAR IN THE UK

A HELPING HAND FOR VERY PRETERM BABIES

Action funding has shown how a simple play intervention, using sticky mittens with babies born prematurely, boosts learning and cognitive development.

Babies born very prematurely, before 32 weeks of pregnancy, are at high risk of experiencing learning difficulties. Early intervention during infancy, when the brain is rapidly developing, can help.

Previous research had shown that full-term babies benefit from play using Velcro-covered mittens. But the effects on very premature infants were unknown.

Dr Ruth Ford from Anglia Ruskin University led a study to test this. Families used sticky mittens for five minutes daily over three weeks. Compared to a control group, the babies using sticky mittens showed significant improvements in exploratory behaviours that are fundamental to learning.

This could go on to influence UK policy on post-discharge care for very premature infants, with this simple and home-based intervention being promoted to parents.

NEW RESEARCH TO HELP PREMATURE BABIES

REDUCING INFECTIONS FROM BREATHING TUBES

Preterm babies are especially vulnerable to infections, which are a major cause of death and serious illness. These can also cause long-term problems for babies who survive.

Infections can often occur while babies are in intensive care units, and some are linked to microbes that normally live on the skin entering the body from life-saving breathing tubes.

With Action funding Dr Shin Tan, a new Action Research Training Fellow, based at the University of Nottingham, aims to incorporate newly developed antimicrobial coatings onto breathing tubes used for babies' ventilation.

Her research will develop and test suitable candidate materials in the laboratory. She will then conduct a small clinical study to determine the effectiveness of the coating on tubes used in newborn babies.

"This study will lay the foundations for a future larger clinical trial to evaluate if these coatings are effective at reducing infections and improving long-term outcomes in preterm babies," says Dr Tan.

This research is being generously supported by the Peter Dixon Charitable Trust.

SOOTHING PAIN TO PROTECT THE BRAIN

Babies born early will experience at least 10 painful procedures every day during their time in neonatal intensive care.

Treating pain in tiny babies is a challenge, with very few methods approved for use. But untreated repetitive pain can have lasting effects, including on brain development.

Supported by Action, Dr Heather Kitt, at the University of Oxford, is leading a clinical trial to test whether kangaroo mother care – using skin-to-skin contact combined with gentle stroking – can effectively reduce pain responses in premature babies during essential blood tests.

Dr Kitt, who is also a new Action Research Training Fellow, will compare this with standard care, measuring brain activity and responses such as heart rate and facial expression. She will also look at neurodevelopmental outcomes of babies.

If proven safe and effective, this simple, low-cost method could be quickly adopted into clinical practice, improving care for preterm babies and their families.

> **"These vulnerable babies urgently need more effective methods of pain relief"**
>
> **Dr Heather Kitt**

IMPROVING CARE FOR BABIES LIKE ELSIE

Elsie was born more than three months early. Tiny and incredibly fragile, she weighed just 600 grams and spent a total of 211 days in neonatal intensive care.

Around 23 weeks into her pregnancy, Elsie's mum Debi's waters broke. Within a few days, Debi was found to have an infection and it quickly became evident that Elsie needed to be delivered immediately.

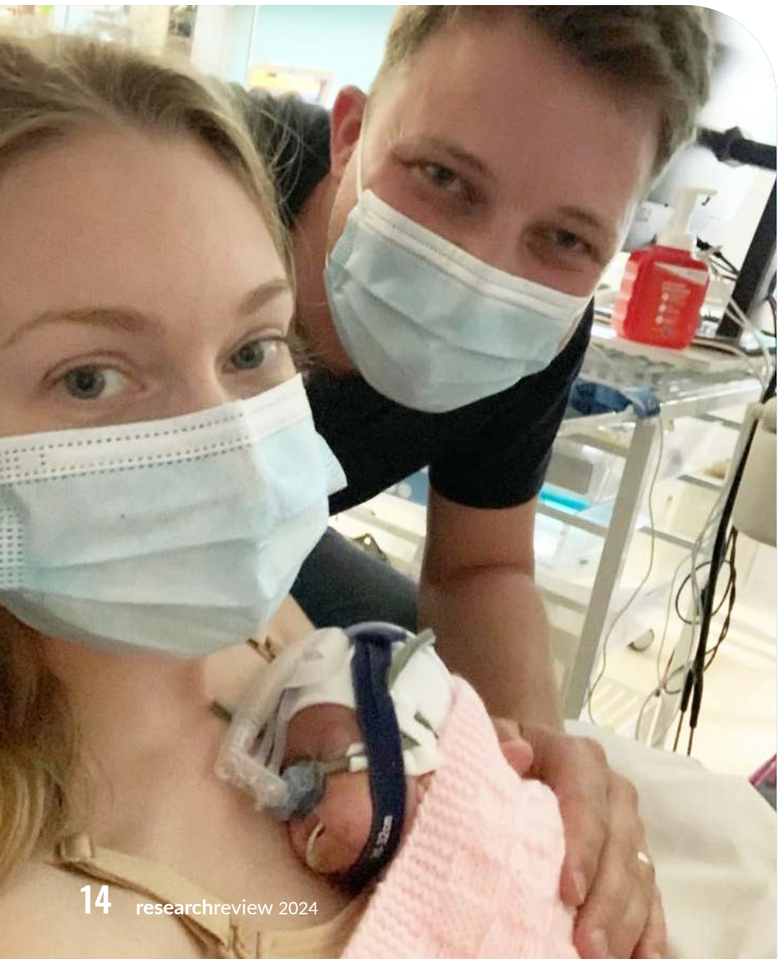
Born by emergency caesarean at just 23 weeks and five days, Elsie weighed less than a bag of sugar. The medical team offered little hope for her survival. "We were told that her chances were 1,000 to one," says her dad, Chris.

Despite the grim odds, Elsie proved resilient. She would go on to spend seven months in neonatal intensive care, each day fraught with uncertainty. "We were told several times that we could lose her, but each time she pulled through," says Chris.

Being born so early meant Elsie faced many serious setbacks. At just 10 days old she needed an emergency operation after a feeding tube perforated her stomach. This was only the beginning of a series of challenges.

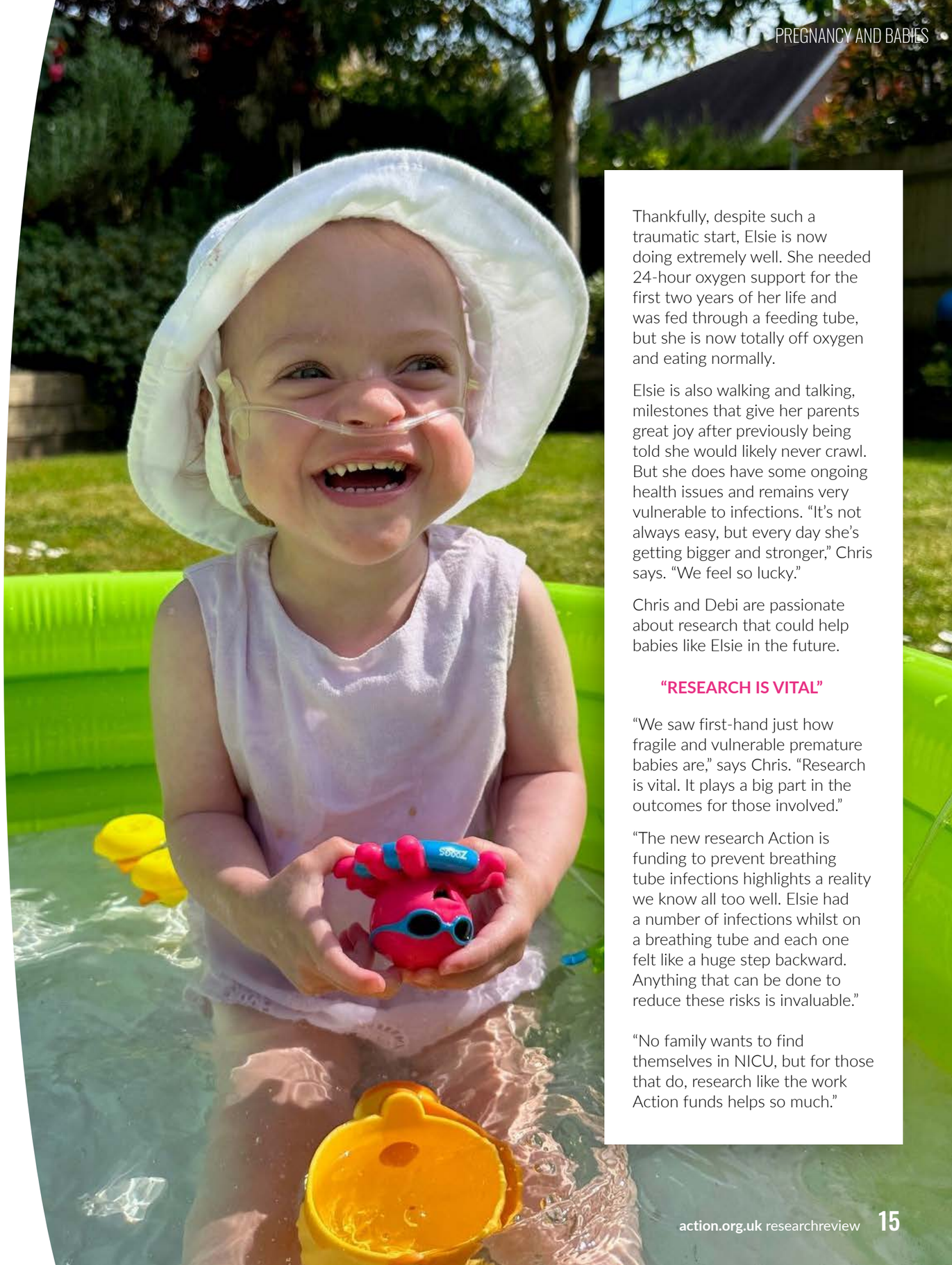
One of her most significant struggles was with severe chronic lung disease, common in premature babies whose lungs are underdeveloped. Elsie also developed retinopathy of prematurity, a serious eye condition that required surgery.

These complex conditions demanded round-the-clock care and constant adjustments to her treatment plan. "The NICU became our new world," says Chris. "Elsie was so fragile; we needed to be constantly vigilant. We practically lived at the hospital during this time – our lives were completely flipped upside down."



"Every infection, every challenge, feels like an impossible battle. As parents you cling to anything that might improve their chances"

Elsie's dad, Chris



Thankfully, despite such a traumatic start, Elsie is now doing extremely well. She needed 24-hour oxygen support for the first two years of her life and was fed through a feeding tube, but she is now totally off oxygen and eating normally.

Elsie is also walking and talking, milestones that give her parents great joy after previously being told she would likely never crawl. But she does have some ongoing health issues and remains very vulnerable to infections. "It's not always easy, but every day she's getting bigger and stronger," Chris says. "We feel so lucky."

Chris and Debi are passionate about research that could help babies like Elsie in the future.

"RESEARCH IS VITAL"

"We saw first-hand just how fragile and vulnerable premature babies are," says Chris. "Research is vital. It plays a big part in the outcomes for those involved."

"The new research Action is funding to prevent breathing tube infections highlights a reality we know all too well. Elsie had a number of infections whilst on a breathing tube and each one felt like a huge step backward. Anything that can be done to reduce these risks is invaluable."

"No family wants to find themselves in NICU, but for those that do, research like the work Action funds helps so much."

HELPING CHILDREN WITH DISABLING 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 epilepsy.

PROGRESS MADE

NATIONAL SURGERY STUDY SET TO HELP CHILDREN WITH CEREBRAL PALSY

Action supported the first ever UK-wide study of orthopaedic surgery for children with cerebral palsy who are able to walk. Finishing in 2024, this has shown that it is better for them to undergo a type of treatment called Single-Event Multi-Level Surgery than not.

Cerebral palsy is the most common serious physical disability in childhood and often affects how children move due to problems with bones, muscles, and joints. Single-Event Multi-Level Surgery (SEMLS) is widely used to correct these issues, but there was limited data about its effectiveness.

Led by surgeon Mr Tim Theologis, researchers collected information on 203 children who were offered SEMLS at 22 centres across the UK. They tracked their progress over two years, using feedback from parents and surgeons, and computerised gait analysis.

The results showed that SEMLS improved how children walked, though it did not lead to major improvements in how far or fast they could walk. In contrast, children who did not have surgery saw their walking worsen over time, and parents also noticed this decline.

Mr Theologis, based at Oxford University Hospitals NHS Foundation Trust, says: “This is set to help establish the first national benchmark for the expected outcomes from SEMLS in the UK. We also identified areas for further research, particularly to address the geographical variations in clinical practice and help improve outcomes for all children with cerebral palsy, regardless of where they live.”

This research was generously supported by The Rooney Foundation and The Linder Foundation

Around 2,000

BABIES ARE DIAGNOSED WITH CEREBRAL PALSY EACH YEAR IN THE UK

HELPING CHILDREN LIKE LEO

Leo was born nearly three months early and suffered a bleed in the brain, which led to a diagnosis of cerebral palsy. As he got older, muscle tightness and weakness became increasingly apparent, especially affecting his left leg, which rotated inwards.

“He has always been very active and independent, but by secondary school Leo needed to walk with a stick,” says his mum, Nancy. “He didn’t want to use a wheelchair, but it became clear that things were getting more difficult for him.”

Leo had SEMLS when he was 14, and Nancy says it has made a big difference, helping his mobility and giving him freedom to do the things he loves.

“He is much more stable now and has less pain. He’s safe on his stick. You can see physically he is upright and strong. He’s very sporty but he can only do that because he’s had the benefit of this corrective surgery,” she says.

“It’s given Leo freedom of movement and freedom to do what he wants to do”

Leo’s mum, Nancy

NEW RESEARCH

HELPING CHILDREN WITH EARLY-ONSET EPILEPSY

Action funding is supporting a study to help better understand the connection between early-life epilepsy and learning and behavioural challenges in preschool children.

Epilepsy causes recurring, unprovoked seizures. Children with epilepsy are also more likely to have neurodevelopmental conditions, learning disabilities, autism and attention-deficient hyperactivity disorder.

Dr Charlotte Tye, from King's College London, has been leading a study to track how epilepsy in infants affects brain development, behaviour and learning during the first two years of life.

With Action support, this work is now being extended to include children between the ages of three and five. The aim is to identify early brain changes that can predict later outcomes, and explore whether these changes explain the relationship between early-onset epilepsy and neurodevelopmental challenges.

This research will pave the way for future early interventions to help improve the lives of children and their families.

ADVANCED SCANNING TO BETTER UNDERSTAND AUTISM

Professor Tomoki Arichi and his team are studying brain activity during social behaviour. They hope their findings will lead to more effective support for children.

Every autistic child is different, but many struggle with social interactions. This can affect their relationships, education and wellbeing as they grow up.

Supported by Action, researchers at King's College London are combining virtual reality, especially designed for young children, with advanced MRI scanning. Their system creates an interactive, engaging experience, enabling them to study brain activity in a way that's fun and more feasible for young children.

By comparing autistic and neurotypical children, the team hopes to uncover differences in social brain processing.

"Our goal is to identify patterns of brain activity during the critical period when conditions like autism first start to emerge," says Professor Arichi. "Being able to identify social processing differences early – and to predict future outcomes – would enable more targeted support to help children to navigate the social world more effectively."

> **"This could lead to earlier diagnosis and help us to understand how we can support children more effectively"**
>
> **Professor Tomoki Arichi**

More than 1 in 100 PEOPLE ARE AUTISTIC

FIGHTING INFECTIONS INCLUDING COVID-19

Starting with polio in the 1950s, we have a long and proud history of fighting serious infections that can devastate children's lives.

MAKING AN IMPACT TACKLING COVID-19

RESEARCH TO HELP BABIES AND CHILDREN DURING THE PANDEMIC AND BEYOND

Action has always risen to the medical research challenges of the day – and in 2020 a new health crisis emerged. In response, we launched our COVID-19 children's research appeal and funded research in this crucial new area.

In spring 2020, medical research was thrown firmly into the spotlight – and relied upon to deliver the life-saving breakthroughs needed to control and treat the new virus that was causing COVID-19. But while the global research effort was unprecedented, there was a lack of work focusing on babies, children and young people, and the impact of the pandemic on them.

Our broad remit, funding research across a wide range of diseases and conditions, allowed us to respond quickly.

During 2021 and 2022 Action invested more than £1.1 million in seven new studies.

These would investigate the impact of COVID-19 on pregnant women, babies and children, and help young people suffering from the debilitating effects of long COVID.

These projects were designed to provide vital information that could have an immediate impact, as well as help to fight future pandemics. They enhanced our understanding of the virus, identified risk factors for severe disease, and supported the development of new guidance.

> **Action funding was critical during an unprecedented time in medical science"**
>
> **Professor Kirsty Le Doare**

SEVEN COVID-19 PROJECTS FOR BABIES, CHILDREN AND YOUNG PEOPLE

This research has now helped to:

- Understand differences in immune responses between children and adults.
- Understand infection in pregnant women and their babies, including advice for vaccination.
- Identify risk factors and develop specialist tests to help prevent severe forms of COVID-19 that can, sadly, affect some children.
- Assess the impact of the pandemic on children with a newly diagnosed brain tumour, with recommendations made for managing cancer services during any future periods of disruption.
- Identify young children who may be at risk of delayed social or emotional development following early-life exposure to COVID-19.
- Develop a new online treatment programme to tackle long COVID in young people.
- Improve understanding of how long COVID affects the brains of children with ongoing symptoms.

More than
£1.1 million
INVESTED IN SEVEN STUDIES

INVESTIGATING IMMUNE RESPONSE

Children with COVID-19 are usually much less likely than adults to become seriously ill – but in 2020, nobody knew why. Funding from Action helped to answer this key question.

Researchers, led by Dr Marko Nikolić, Dr Kerstin Meyer and Professor Sarah Teichmann, found that children have a much stronger immune response in the nose, helping to fight off the virus straight away, before it spreads to other body parts. They also have a weaker immune response in the bloodstream compared to adults – protecting them from immune-related damage to organs, as seen in severe disease.

This research, and follow-on work that the team has carried out since, has given crucial insights into how the immune system fights COVID-19. It has also helped to identify potential avenues for preventing and treating severe cases.

> **Our findings could be used to help identify which children and adults are at greater risk of severe illness"**
>
> **Dr Marko Nikolić**

HELPING MUMS AND THEIR BABIES

At a time of such great uncertainty, Action-funded research also led to much-needed guidance and reassurance for pregnant women.

Women and their babies are especially vulnerable to infections during pregnancy and the first weeks after birth. So when COVID-19 arrived, there were significant concerns.

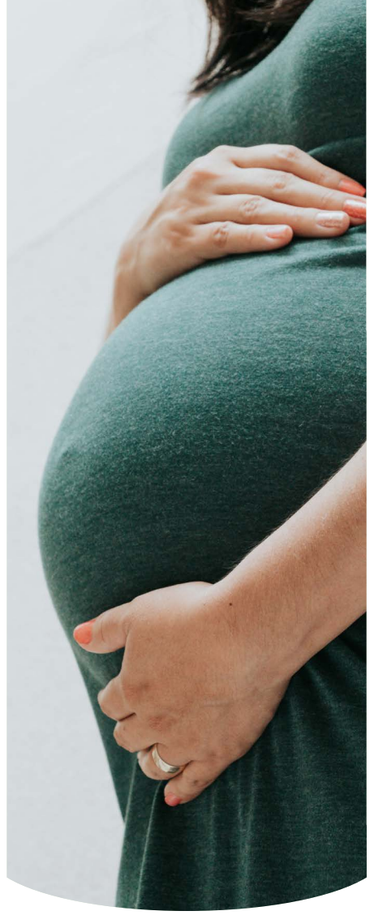
Led by Professor Kirsty Le Doare, this work aimed to determine how much of a risk COVID-19 posed for pregnant women and their babies. It saw women screened at 10 different hospitals across England.

It found that the virus itself did not appear to be transferred to babies during pregnancy or breastfeeding.

It also showed that antibodies against COVID-19 could pass from mother to baby, offering a level of protection against the virus to newborn babies, whether through vaccination or by natural infection.

These findings fed into guidance for pregnant women and new parents, published by the UK Health Security Agency and the NHS.

"The Action funding was crucial during an unprecedented time in medical science," says Professor Le Doare. "It helped us to produce high quality evidence for a population that had previously been underserved by COVID-19 studies and vaccine trials."



HELPING CHILDREN SUFFERING WITH LONG COVID

Action funding helped to develop online support for affected young people.

Long COVID can leave children with ongoing breathlessness and feelings of anxiety or low mood many months after their initial infection.

Dr Samatha Sonnappa and her team at the Royal Brompton Hospital in London set out to develop an online programme to help.

With input from young people suffering with long COVID, they developed modules including breathing techniques and coping skills, along with bespoke videos. These are now available as an online resource.

"The grant from Action allowed us to bring together a team of specialists, alongside teenagers, to design and then deliver a new intervention," says Dr Sonnappa.

> **"This work simply would not have happened without this vital funding"**
>
> **Dr Samatha Sonnappa**

NEW RESEARCH TO PREVENT AND TREAT INFECTIONS

A BREATH TEST TO DIAGNOSE CRITICALLY ILL CHILDREN

Every year, millions of children worldwide are hospitalised for chest infections. In the UK alone, nearly 6,000 children with life-threatening chest infections are admitted to intensive care units.

While these infections are frequently caused by viruses, most critically ill children are initially treated with antibiotics. This is because routine tests to diagnose bacterial infections have a low pick-up rate and take days to come back. Unfortunately, unnecessary antibiotic use can lead to unpleasant side effects and contributes to the development of antibiotic resistance.

Professor Padmanabhan Ramnarayan, and a team of world-leading scientists at Imperial College London, aims to develop a new breath test that can rapidly and accurately identify bacterial infections. This would be quick, safe and painless for seriously unwell children. It could also be repeated frequently to track whether an infection is improving, helping to guide treatment.

This could transform care, ensuring sick children receive the best possible treatment while reducing antibiotic use and unnecessary side effects.

FIGHTING VENTILATOR-ASSOCIATED PNEUMONIA IN CHILDREN

This life-threatening lung infection affects children who are already seriously ill. Dr Nazima Pathan aims to improve early detection and treatment. This could save lives and shorten hospital stays.

Ventilator-associated pneumonia affects up to one in five children admitted to paediatric intensive care units in the UK.

"These vulnerable children are at risk of dying not only from their critical illness but also from this hospital-acquired infection, which is caused by harmful bacteria entering their weakened lungs," says Dr Pathan.

Diagnosis is challenging because current tests don't provide clear results, making it difficult to choose the best treatment.

With Action funding, Dr Pathan and her team aim to deepen understanding of how these infections develop. Based at the University of Cambridge, they hope to identify potential markers in the body and lungs that signal the early stages of infection. This could lead to new diagnostic tools and enable doctors to provide more targeted, effective treatment.

Each year over
18,000
CHILDREN ARE ADMITTED TO PAEDIATRIC INTENSIVE CARE UNITS IN THE UK

RESEARCH GRANTS AWARDED IN 2024

Action Medical Research is currently funding around 55 projects across the UK, 14 of them awarded in 2024. **The next medical breakthrough could be on your doorstep.**

Childhood-onset lupus – developing a blood test to help prevent heart disease in children most at risk*
Professor Coziana Ciurtin
University College London

Combining virtual reality and brain scans to improve understanding of social brain processing in autistic children
Professor Tomoki Arichi
King's College London

Cystinosis – improving medicines to help children follow their treatment plan*
Dr Oisín Kavanagh
Newcastle University

Developing a non-invasive breath test to improve the diagnosis of lung infections in critically ill children
Professor Padmanabhan Ramnarayan
Imperial College London

Duchenne muscular dystrophy – boosting the effectiveness of cutting-edge gene-based therapies*
Professor Francesco Muntoni
UCL Great Ormond Street Institute of Child Health

Epilepsy – identifying early-life predictors of neurodevelopmental outcomes
Dr Charlotte Tye
King's College London, Evelina London Children's Hospital, Birkbeck, University of London

Neurofibromatosis type 1 – repurposing existing drugs for treating young people with this rare condition*
Professor Benjamin Housden
University of Exeter

Personalising treatment for children with rare inherited conditions affecting their immune system
Dr Thomas Altmann
Newcastle University, UCL Great Ormond Street Institute of Child Health, University of Manchester

Preterm birth – investigating whether a new drug could help prevent brain injury in babies born too soon
Dr Helen Stolp
Royal Veterinary College

Reducing pain and improving brain development for preterm babies
Dr Heather Kitt
University of Oxford

Reducing the risk of infections from breathing tubes in preterm babies
Dr Shin Tan
University of Nottingham

Searching for cutting-edge gene therapies to help children with a rare severe type of epilepsy
Dr Amy McTague
UCL Great Ormond Street Institute of Child Health

Testing a new treatment strategy that could help children with two rare neurodegenerative diseases*
Dr Sovan Sarkar
University of Birmingham, Birmingham Women's and Children's Hospital

Ventilator-associated pneumonia – developing new tools to predict serious lung infections in critically ill children
Dr Nazima Pathan
University of Cambridge, plus various children's hospitals

* Jointly funded with LifeArc

Locations of research funded by Action

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 already made provision for Action in their will and to those who left us a legacy gift in 2024.

Lorna Allatt
Joyce Appleton
Brenda Bond
Harry Brookes
Kenneth Brooks
Leonard Coffey
Raymond Coveney
Rosa Griffiths
Doris Hoar
Rosemary Horton
Lesley McLeish
Gillian Murray
Elisabeth Norton
Myra Phillips
Daphne Sprague
Ann Squire
Wendy Talfourd-Cook
Izabela Tolowinska
Elaine Veilands

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**

David Fern – legacy pledger

David, one of Action's cycling volunteers, has pledged to leave a gift in his will to Action. Having cycled competitively and worked on major events, he brought his expertise to one of our earliest bike rides, the Action 100. He had personal experience of neo-natal death and wanted to help raise funds and awareness. **"It's not about what I did in the past. Or what I can do today. But how I can continue to support important research into the future – and for this reason I decided to leave a legacy in my will."** says David.

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 190 top researchers, working on around 55 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'.

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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Professor Stephanie Schorge PhD (Chair)
Professor Simon Bailey MBChB MRCP PhD joined May 2024
Professor Diana Baralle MBBS MD FRCP (on sabbatical)
Professor Joanne Blair MBChB MRCP MRCPCH MD joined May 2024
Professor James Boardman MBBS MSc PhD FRCPCH
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Professor Deborah Gill PhD
Professor Ed Johnstone MBChB PhD MRCOG
Professor Juan Kaski MD(Res) FRCP FESC joined July 2024
Professor Pablo Lamata PhD
Professor Robert McFarland MA MBBS PhD MRCP FRCPCH
Associate Professor Nazima Pathan PhD FRCP joined August 2024
Professor Padmanabhan Ramnarayan MD FRCPCH FFICM joined July 2024
Dr Claire Thornton PhD joined August 2024
Professor Brigitte Vollmer Dr.med PhD FRCPCH joined August 2024

Scientific Advisory Panel for joint Action Medical Research/LifeArc project grant applications on rare disease research
Professor Stephanie Schorge PhD (Chair)
Professor Simon Bailey MBChB MRCP PhD joined May 2024
Professor Diana Baralle MBBS MD FRCP (on sabbatical)
Professor Joanne Blair MBChB MRCP MRCPCH MD joined May 2024
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Dr Thorsten Forster PhD
Professor Deborah Gill PhD
Professor Pablo Lamata PhD
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Dr Andy Merritt PhD CChem
Professor Padmanabhan Ramnarayan MD FRCPCH FFICM joined August 2024
Professor Felicity Rose PhD FHEA
Professor Brigitte Vollmer Dr.med PhD FRCPCH joined August 2024

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