action medical research for children

Research Review 2020

Saving and changing children's lives

Contents

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

Please send all communications to:
Action Medical Research, Vincent House,
Horsham, West Sussex RH12 2DP

T **01403 210406**
E **info@action.org.uk**
W **action.org.uk**

/actionmedres
@actionmedres
@actionmedres

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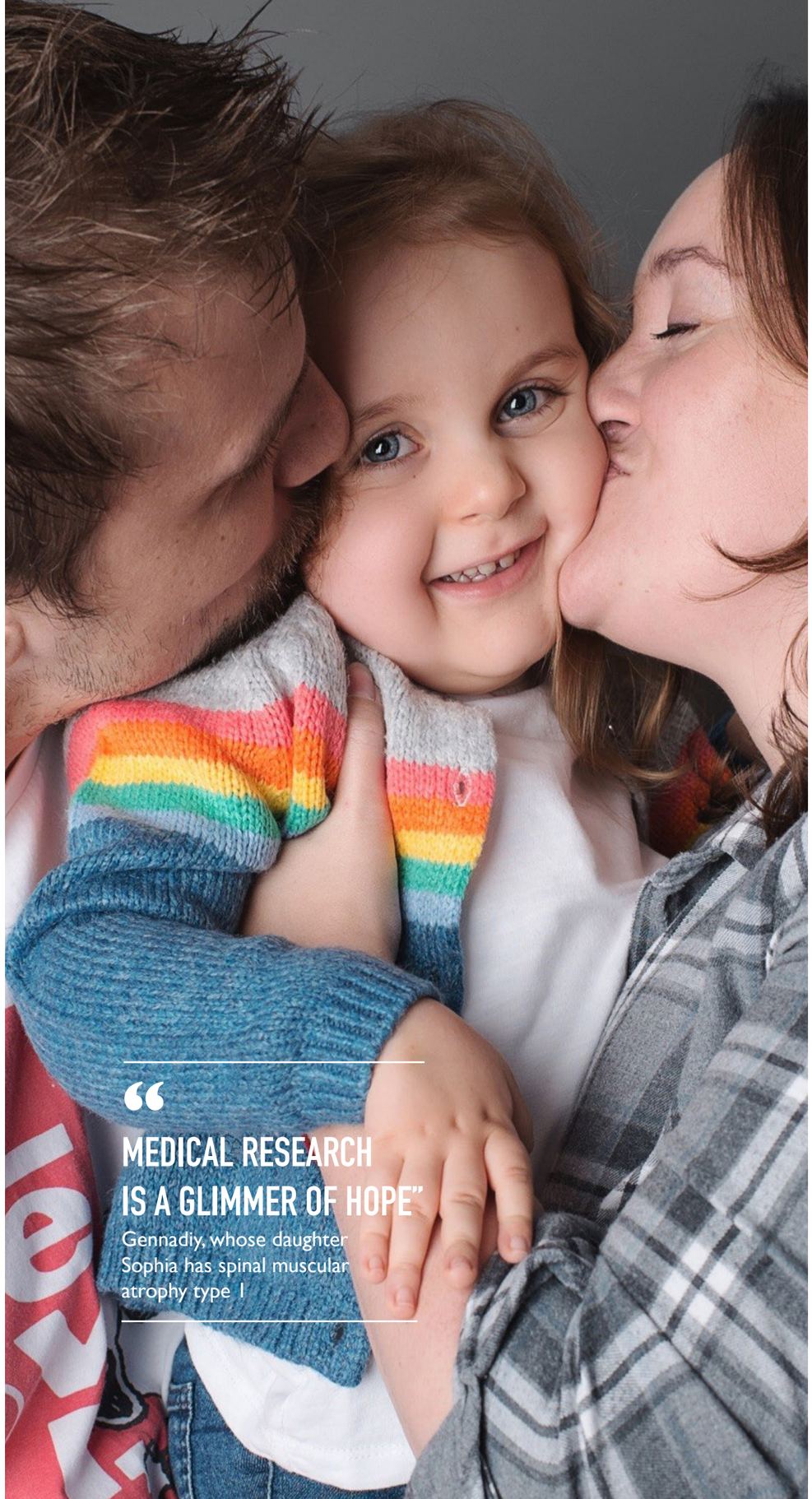


Welcome

The last year has been a stark reminder of the huge difference medical research can make. Right now the whole world is relying on breakthroughs to help us live alongside COVID-19. At the same time, many thousands of families in the UK are already living with other diseases and conditions, hoping that research will lead to the treatments they so desperately need to help their children.

Like everyone, at Action Medical Research we have been affected by the pandemic and our community stepped up magnificently. We worked hard to keep people safe, to regroup our fundraising to support research that was underway, and to fund more new research to help save and change children's lives.

Despite the challenges, we were able to fund eight new research projects including three into the effects of COVID-19 in children, as well as three projects researching children's rare diseases together with our partner LifeArc. We also funded two new Research Training Fellowships, developing the research leaders of the future – and we are proud that a former Action Research Training Fellow, Professor Sir Andrew Pollard, is today one of the expert scientists who helped develop and test the COVID-19 Oxford/AstraZeneca vaccine.

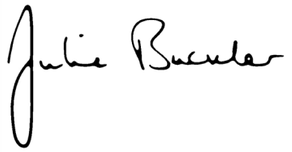
Exciting progress from research funded by Action included a new 3D ultrasound imaging technique to screen for hip problems in children with cerebral palsy – and moving a step closer to reducing the risk of preterm birth, with promising results and a full clinical trial planned in 2022.

A new report also demonstrated the impact we make, with research we funded showing the benefit of providing eyecare in special schools, increasing the number of children whose visual needs are met, improving their vision and unlocking potential in the classroom. This important research is now influencing in-school vision services for the 130,000 children in special education in the UK.

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

The extraordinary results for children and their families that we make possible can only happen with the support of many individuals, groups, companies, trusts and foundations. We are grateful to everyone in our community, including our network of hardworking local committees and volunteers, as well as those who generously remember Action with gifts in their wills.

Joining us helps make new research breakthroughs possible. Please help us fight for children's lives.



Julie Buckler
Chief Executive
Action Medical Research for children

New research in the fight against COVID-19

In 2020 a new health crisis emerged. In response to the pandemic, we launched our COVID-19 children's research appeal to fund research in this crucial new area.

Over the last year, life has changed in ways we could never have predicted as the world fights a new and devastating virus. Medical research has been thrown into the spotlight – and relied upon to deliver the life-saving breakthroughs needed to control and treat the virus.

But while investment in research to help understand the disease in adults was swift, there was a lack of work focusing on babies and children, and the impact of the pandemic on them.

> "CHILDREN HAVE BEEN THE QUIET, FORGOTTEN BYSTANDERS IN THE COVID-19 PANDEMIC"
>
> **Dr Barney Scholefield**

We moved quickly to help fill this gap and, thanks to the support of our COVID-19 children's research appeal, awarded three new projects to help better understand the impact of the pandemic on babies and children, with further research being supported in 2021.

This vital research could lead to new ways to prevent and treat severe illness in children as a result of COVID-19 – and shed light on why children are generally better protected from the virus than adults. It could provide vital information to help now and to fight future pandemics.

While it is well known that older adults are most likely to become seriously ill, some children are still vulnerable to severe illness as a result of COVID-19.

There is also much still unknown about the risks in pregnancy and to babies. And the wider impact of the pandemic on the physical and mental health of children will also be felt long into the future.

We brought together an expert advisory group of leading children's health researchers to help guide how we could best fund research to help.

As Dr Barney Scholefield, a member of this group and paediatric intensive care consultant and researcher, said: "Children have been the quiet, forgotten bystanders in the pandemic. It is essential we bring them to the forefront as they can help us all understand this disease better, and we must improve the way we care for them during these uncertain times."

In the first wave of the pandemic, from March to July 2020, more than 650 children in the UK were admitted to hospital with severe COVID-19 infection and in the second wave some doctors reported admissions of children to be around three times higher.

Why do children respond differently?

With Action funding, researchers hope to answer one of the biggest unknowns of the pandemic.

It quickly became apparent that children are generally less affected by the virus that causes COVID-19 than adults. Dr Marko Nikolić of University College London is teaming up with Dr Kerstin Meyer at the Wellcome Sanger Institute to uncover clues that could help explain this phenomenon – and explain why those children who are infected are more susceptible than others.

They are investigating whether children generally have lower amounts of a molecule in the lining of their nose that could make it harder for SARS-CoV-2, the virus that causes COVID-19, to infect their cells compared to adults. The researchers will also look for differences in how the immune system responds to the virus.

The results of this research could offer crucial insight into how to combat serious illness in children and adults, now and in future pandemics.

> "WE HOPE TO UNCOVER VITAL NEW CLUES THAT LEAD TO NEW APPROACHES TO PREVENT OR REDUCE SEVERE DISEASE"
>
> **Dr Marko Nikolić**

Does the virus affect brain development?

New research will assess whether being exposed to COVID-19 puts babies at increased risk of developmental issues.

Thankfully most newborn babies with COVID-19 have mild or no symptoms, but the virus can sometimes cause brain complications in adults. This raises the possibility of there being hidden effects on a child's developing brain.

At the University of Bristol Dr Ela Chakkarapani is assessing babies exposed to the virus, either in the womb or shortly after birth, against those who were not, checking for any early signs of developmental delay.

The findings could help identify children who may be at increased risk of behavioural or emotional problems and need additional support. Dr Chakkarapani also hopes to follow up with these babies later in childhood for future studies.

"This study could help shape advice for pregnant women and new parents – and identify children who may need additional checks and support," he says. "The results could also stimulate research into new treatments to help reduce the long-term impact on children's lives."

Protecting babies from COVID-19

We are also funding a study that could inform how best to protect pregnant woman and their babies from any potential risks from COVID-19.

While certain bacterial or viral infections during pregnancy or soon after birth can cause harm for women and their babies, the potential risks of COVID-19 are so far unknown.

More than **700,000** babies are born in the UK each year

Thousands of pregnant women in the UK are likely to have been infected with SARS-CoV-2, the virus that causes COVID-19, many with no symptoms. Current evidence suggests only a small number become seriously ill. But there are many outstanding questions.

"THE PANDEMIC HAS RAISED MAJOR CONCERNS FOR PREGNANT WOMEN AND THEIR HEALTHCARE TEAMS"

Professor Kirsty Le Doare

Professor Kirsty Le Doare is leading a study that will screen women from hospitals across England to identify how many have been infected – and whether the virus or protective antibodies are passed from mother to baby during pregnancy, delivery or breastfeeding.

"We hope that our findings will help to reassure pregnant and breastfeeding women about how to safely care for their babies. It will also help inform future decision-making about vaccinating pregnant women to help protect them and their babies from infection," says Professor Le Doare.

Fighting for babies born too soon

Each year, around 60,000 babies are born prematurely in the UK and sadly more than 1,000 babies die as a result. Action funds vital research to try and prevent premature birth and provide the best possible care for these vulnerable infants.

Over the last two years we have been shining a spotlight on the impact of premature birth and raising vital funds for life-saving research in this area. We've now raised more than £600,000 towards our £1m target to help babies born too soon. We had aimed to reach this goal by the end of 2020 but, due to the challenges of the year, we will now continue until we reach our £1m target.

The earlier a baby is born, the higher their risk of death or complications that can leave them with lifelong disabilities, such as cerebral palsy, visual impairment or hearing loss.

We're delighted to share below progress reported back in 2020 from just one of the research projects we've supported in this area.

Steps Forward

TREATMENTS TO PREVENT PREMATURE BIRTH

Action funding has taken researchers a step closer to reducing the risk of preterm birth, by using two treatments in combination.

Treatment with a hormone called progesterone can reduce a woman's risk of giving birth early, but it doesn't work for everyone. So Professor Mark Johnson, of Imperial College London, has been investigating whether combining progesterone with the asthma drug aminophylline, already used to help premature babies' lungs develop, could be more effective.

This study, with generous support from The Albert Gubay Foundation, looked at the combination treatment in the laboratory and in a feasibility study in pregnant women who were at high risk of early labour. The promising results have meant the work is now moving towards a full clinical trial, with the ultimate goal to stop babies from being born prematurely, save lives and improve their health.

The research team is now planning this larger clinical trial, which they hope will start in 2022.

Little Luka's toughest of starts

As our focus on premature birth continues, Luka's story highlights the challenges faced by babies born too soon. Arriving 10 weeks early, he spent his first two months in hospital and needed life-saving surgery.

With her first pregnancy progressing smoothly, Lucy and her husband Kyle had every reason to feel happy and confident as they prepared to welcome their baby. But one night, two-and-a-half months before her due date, Lucy started to feel uncomfortable. Realising the sensation was coming in waves, she called the hospital. "We hadn't even finished antenatal classes," she recalls.

Lucy was given drugs to try and delay labour but they didn't work. Baby Luka arrived weighing just 3lbs 9oz and was immediately taken to the Neonatal Intensive Care Unit (NICU).

Doctors had reassured Lucy and Kyle that babies born at 30 weeks were far less vulnerable than those born even earlier, and initially Luka did well. "We just assumed everything was going to be alright. We thought Luka would eat, grow and be home in a few weeks," says Lucy. "But after three or four days, it hit really hard. Then on day five Luka became very ill. It was the worst day of my life."

Little Luka had an infection and was given a range of antibiotics. He was ventilated, given morphine and thankfully started to recover.

Globally, premature birth is the biggest killer of children under 5

Lucy and Kyle began to relax. But three weeks later, they got a phone call in the middle of the night. Luka had been very sick, his tummy was extended, and doctors felt they must operate straight away to find out what was wrong.

The surgeon detected the life-threatening bowel disease necrotising enterocolitis (NEC). 10cm of Luka's bowel was removed, and a stoma was formed to take care of his body's waste.

To Lucy and Kyle's immeasurable relief, the medical team was confident that Luka would make a good recovery. After eight weeks in hospital, Luka finally came home where, thankfully, he's continued to thrive. Further surgery to reverse his stoma was required, but all went well.

> "IT WAS A JOURNEY THROUGH HELL BUT WE WERE LUCKY ENOUGH TO BRING OUR BABY HOME. RESEARCH INTO PREMATURITY AND ITS COMPLICATIONS IS VITAL"
>
> **Luka's mum, Lucy**

Luka is now a happy, lively and healthy little boy. He's had regular check-ups with a paediatrician to check his development but is doing well with no concerns raised. But Lucy and Kyle are very aware that not all families have such a positive outcome.

"Premature birth can happen to anyone – there's just not enough research, yet it's one of the biggest killers of babies in the world," says Lucy. "When Luka was in hospital there were just so many mums who never got an answer. It does make me afraid to become pregnant again. Premature birth affects the whole family, grandparents too."

Research to fight rare diseases

Thousands of families across the UK are dealing with the fact that their child has a rare disease for which there is no cure, and no or limited treatments.

We fund groundbreaking medical research to develop new treatments and give hope to these forgotten families. Hope for new treatments and cures. Hope that in the future other families will not have to go through what is all too often a heartbreaking journey.

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

In 2020 we funded three new projects as part of our ongoing partnership with British medical research charity LifeArc. Together we have now invested more than £1m into vital research in the area of children's rare diseases since this joint funding initiative was set up in 2019.

Treating a rare form of epilepsy

Researchers aim to develop a new drug to prevent the delayed development and learning disabilities that children with pyridoxine-dependent epilepsy face.

Pyridoxine-dependent epilepsy typically becomes apparent shortly after birth. It's caused by a faulty gene, which in turn causes the build-up of toxic chemicals in the child's body. The resulting chemical imbalances lead to seizures, delayed development and learning disabilities.

Taking lifelong large daily supplements of pyridoxine can usually control the seizures. But this doesn't address long-term neurological symptoms.

Led by Professor Wyatt Yue, this research aims to develop a new treatment to prevent the build-up of the chemicals which cause these debilitating symptoms.

"We have previously identified several potential drug compounds, and we will now make tiny changes to their chemical structures and evaluate their properties in the laboratory," he explains.

The team will then test the effects of the most promising drug candidates in cell studies, selecting the best ones for future development for patients.

"If we can identify a drug that tackles the root cause of the neurological symptoms, it will greatly improve children's quality of life," says Professor Yue.

Preventing a deadly heart condition

Hypertrophic cardiomyopathy is a leading cause of sudden cardiac death in young people. New research aims to improve diagnosis and monitoring.

Hypertrophic cardiomyopathy is an inherited condition that causes thickening of the heart muscle. Although it is rare, and may cause no symptoms, sadly it can cause heart failure and sudden death.

Current treatments can help manage the condition but do not target the underlying cause, or prevent it developing in those known to be at risk.

Dr Juan Pablo Kaski and his team, based at University College London, want to gain a better understanding of the early features of the disease and how it progresses over time. They have recently developed a simple blood test to diagnose the condition in adults and measure disease severity. They will now see if this test also gives accurate results in children, including those who are known to be at risk but have not yet shown symptoms.

The researchers also aim to identify new biomarkers in the blood and urine that are specific to children, and correlate these with disease severity and progression.

This could result in faster and cheaper diagnostic tests, new tools to monitor the disease and ultimately new treatments.

> "OUR ULTIMATE GOAL IS TO MAKE IT POSSIBLE TO PREVENT THE DISEASE FROM PROGRESSING"
>
> **Dr Juan Pablo Kaski**

Steps Forward

IMPROVING DIAGNOSIS OF PERRAULT SYNDROME

With Action funding, researchers have now identified new genetic changes that cause this rare inherited condition.

Perrault syndrome causes severe, progressive hearing loss during childhood, and girls can grow up to have fertility problems that affect their chances of becoming pregnant. It is under-recognised, particularly in boys, where the cause of their hearing loss is often undiagnosed.

Changes in several different genes have already been found to cause the condition, but they don't explain all cases.

With Action funding, researchers, led by Professor Bill Newman at the University of Manchester, have discovered that changes in five new genes can also be responsible. Importantly, they have also identified a more severe childhood-onset form of the syndrome, and are now working to understand this in more detail.

This improved genetic testing helps children and their families understand their difficulties and health problems, and helps doctors provide the most appropriate treatment and therapies. It also allows for the counselling of at-risk family members, which helps when planning for a baby.

This work was assisted by a grant from The Peter Stebbings Memorial Charity.

Hopes of a new treatment for Duchenne

Duchenne muscular dystrophy is a rare and ultimately fatal disease that causes muscle weakness and a relentless deterioration in physical abilities. Boys like Tom eventually become paralysed, needing round-the-clock care.

Tom was diagnosed with Duchenne when he was four, but his parents were concerned about him from his earliest days. "At the baby clinic, the health visitor would stress the importance of 'tummy time' to help Tom lift his head, but he couldn't do it. He just screamed," his mum, Rebecca, remembers. "He was slow to reach milestones like crawling and walking, too."

Tom could not bounce on a trampoline like other toddlers, he fell over a lot and he walked with an awkward gait. By the time he was due to start school, Rebecca had insisted on a referral to a paediatrician.

The paediatrician carried out a full neurological assessment and asked to do a blood test 'to rule out a couple of rare conditions'. He phoned the next day with devastating news: Tom had muscular dystrophy.

Tom's little sister was just seven days old at the time. "I was breastfeeding when I answered the phone and remember my tears streaming down onto her head," says Rebecca.

"It was such a shock. It seemed so improbable that my gorgeous son would eventually become locked in a body he could not move and die."

Sadly, further tests revealed that Tom has the severe form of the disease known as Duchenne muscular dystrophy. Most children with Duchenne need to use a wheelchair by their early teens and, although life expectancy is increasing, most don't live beyond their forties.

At the time of Tom's diagnosis, the family had just built their dream home, but it had stairs.

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

Advised to be proactive, they bought a bungalow which they adapted for Tom's future needs.

Now 13 years old, physically Tom is following the disease's expected progression. He can no longer walk or sit up from lying down and uses a wheelchair. Rebecca notes that his arms are starting to weaken but his heart and breathing muscles remain normal.

Tom does not have the serious learning difficulties and autism which can affect children with Duchenne, and he copes well at his mainstream school. His mum says he faces life with a 'you just have to get on with it' attitude and inspires his family every day.

In 2020 Action and LifeArc awarded funding for new research which aims to identify a drug compound with the potential to become an effective new treatment for Duchenne.

The research team, led by Professor Angela Russell, are investigating drugs that can boost utrophin, a protein that is found naturally in muscles and has the potential to act as a substitute to dystrophin. They hope to identify a drug that can be taken into clinical development, with the hope of becoming an effective new treatment that's suitable for all affected children.

"Potential treatments mean everything to families like ours," says Rebecca. "Too many parents have lost their children to this horrendous condition."

> "IT SEEMED SO IMPROBABLE THAT MY GORGEOUS SON WOULD EVENTUALLY BECOME LOCKED IN A BODY HE COULD NOT MOVE AND DIE"
>
> **Tom's mum, Rebecca**

Bringing eyecare into special schools

We're delighted to have funded research that has successfully shown the benefits of delivering comprehensive eyecare in the familiar setting of a child's school – paving the way for services to be offered in schools across the UK.

Children with learning disabilities are 28 times more likely to have a serious sight problem than other children, but nearly 40 per cent have never had an eye test or any eyecare. So sight problems often remain undiagnosed and therefore untreated, preventing children from reaching their full potential.

Access to eyecare can be very challenging for children with developmental disabilities and their families. These children may also be less able to express, or even recognise, that they have sight problems – and adults may assume that their behaviour is just part of their disability, without exploring other possible factors.

In 2016, Action awarded almost £190,000 to a team led by Professor Kathryn Saunders at Ulster University. Called the Special Education Eyecare project, or SEE for short, two hundred children and young people from Northern Ireland's biggest special school took part.

Nearly two thirds of the children involved in the study were found to have at least one significant eye or vision problem. Nearly half also had at least one unmet visual need, like no glasses or needed large print learning materials.

The research proved that providing full eyecare in the familiar school setting – with glasses dispensed on-site and written information and advice shared with teachers and parents – had a positive impact.

Around **130,000** children attend special schools across the UK

"Our study demonstrates, for the first time, measurable benefits to children and young people. These were apparent in both children's vision and behaviour," says Professor Saunders.

> "THE SUPPORT FROM ACTION WAS PIVOTAL"
>
> **Professor Kathryn Saunders**

Matthew's story

Matthew was one of the children who took part in the SEE project. He has autism and his mum Julie says that being seen at school, a setting he's more comfortable in, made a crucial difference.

"It was fantastic for an autistic child, as Matthew can be very fearful. We had both previously found eye tests very stressful. This really helped take that pressure off," she explains. "Although we knew Matthew had vision problems from an early age, it wasn't until the SEE project that we got a really accurate prescription for him and he started to wear his glasses regularly," adds Julie.

Matthew had previously been very reluctant to wear his glasses and would cry when his teachers asked him to. But following the detailed advice provided, school staff worked as a team to encourage him. They developed a simple daily routine, where they would hold Matthew's hand, so he couldn't take his glasses off, and take him for a walk up and down the corridor. He now looks for his glasses as soon as he wakes up.

"This extra support has been great and wearing his glasses means he now concentrates better as he can see what he needs to without squinting."

> "IT WAS INVALUABLE FOR US. IT WILL BE FANTASTIC TO SEE IT EXPANDED MORE WIDELY FOR OTHER CHILDREN "
>
> **Matthew's mum, Julie**

Steps Forward

PERSONALISING CHILDREN'S ASTHMA TREAMENT

Findings from an Action funded study have demonstrated how personalised treatment can improve the quality of life for children and young people with asthma.

Asthma causes coughing, wheezing and difficulty breathing – and in the UK it results in one child being admitted to hospital every 18 minutes. But when the condition is managed well, children can lead a full and active life.

Professor Somnath Mukhopadhyay, of Brighton's Royal Alexandra Children's Hospital and Sussex Medical School, has uncovered evidence that a commonly used asthma medicine, salmeterol, may actually offer little benefit to some of the children taking it – with genetic differences effecting how well the medicine works.

In a trial that was the first of its kind, Professor Mukhopadhyay and his team used an inexpensive saliva test to determine differences in children's genetic make-up. They then prescribed either salmeterol or another drug, montelukast, according to the children's genetic make-up. Those who received the personalised treatment showed greater improvements to their quality of life.

"THESE RESULTS SHOW THAT IT COULD BE BENEFICIAL TO TEST FOR CERTAIN GENETIC DIFFERENCES AND SELECT MEDICATION ACCORDINGLY"

Professor Somnath Mukhopadhyay

Around **1.1 million** children in the UK have asthma

We are grateful to the many trusts and foundations who helped support this project including The Henry Smith Charity

Almost **2,000** babies born each year in the UK are diagnosed with cerebral palsy

Steps Forward

NEW SCANS FOR CHILDREN WITH CEREBRAL PALSY

With Action funding, researchers have developed a 3D ultrasound scanning technique to screen for hip problems in children with cerebral palsy.

Children with cerebral palsy face lifelong difficulties with movement and coordination, and up to 40 per cent start to have problems with their hips during childhood. This can be very painful and lead to hip dislocation. It can make it hard for children to stand, walk or even sit comfortably and cause other problems, such as fractures and skin ulcers. Early and accurate prediction of hip problems, allowing prompt treatment, is therefore vital.

X-rays are currently used for screening but they cannot be carried out often due to exposure to radiation, and they only produce 2D images which may not always reveal problems.

Dr Adam Shortland and his team at Guy's Hospital, London, have trialled a new technique which is safe to use more regularly and provides detailed 3D images.

"The technique is really easy to use. You can take as many pictures as you like, with the hip in different positions, and it is a far less difficult experience for children, and their parents, to cope with," says Dr Shortland.

The team's research also suggests that sonographers or outpatient clinicians would be able to carry out these scans after just a few hours of training, making it time and cost-effective.

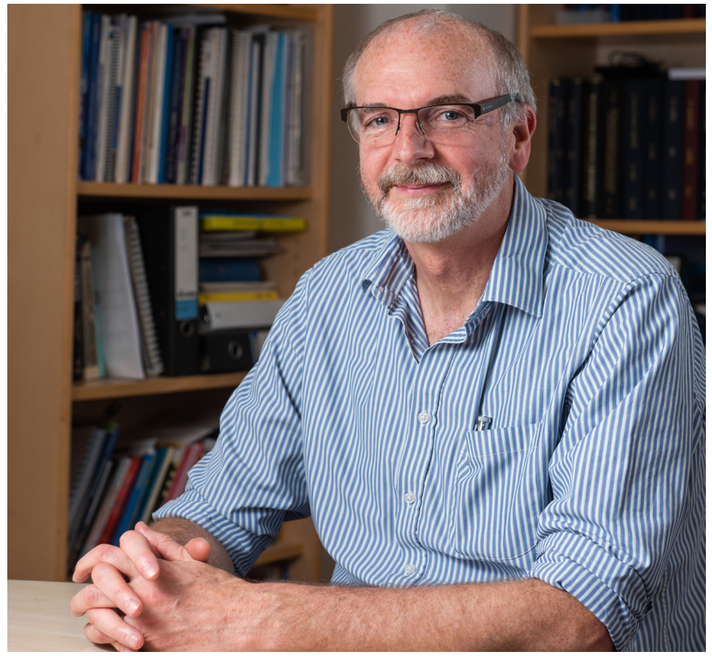
The researchers hope more children will start to benefit from the new technique within the next three years.

"WE'RE EXCITED ABOUT OUR FINDINGS WHICH WE FEEL OFFER REAL CLINICAL POTENTIAL"

Dr Adam Shortland

Funding future leaders in medical research

Our Research Training Fellowship scheme supports some of the UK's most gifted doctors and scientists early on in their research careers. We're incredibly proud that one former Fellow is Professor Sir Andrew Pollard, who's led the trial to test the Oxford/AstraZeneca vaccine for COVID-19.



The rollout of vaccines has been a hugely significant step in the fight against COVID-19. As Director of the Oxford Vaccine Group, Professor Pollard has played a leading role – recently recognised with a knighthood for his services to public health. But while his current focus has been on a vaccine developed primarily to protect adults, he is also a Professor of Paediatric Infection and Immunity at the University of Oxford and an Honorary Consultant Paediatrician, with most of his career being focused on preventing and treating diseases that threaten children.

Action is delighted to have played a part in supporting Professor Pollard, funding his Research Training Fellowship back in 1995. This enabled him to complete his PhD and, he says, played a key role in his development to become a leading authority in paediatric immunity and vaccinology.

"It was critical because it was my research training. It put me in a laboratory and gave me a broad range of techniques I needed to apply to my research and a very detailed understanding of the challenges – how to effectively control experiments and how to collect and work with data directly from patients," says Professor Pollard.

> "MY FELLOWSHIP GAVE ME A PORTFOLIO OF SKILLS WHICH I CONTINUE TO USE TODAY"
>
> **Professor Sir Andrew Pollard**

"It was an amazing all-round experience and gave me a portfolio of understanding and skills which I continue to use today. This includes both general skills around analysis but also some very specific techniques that are still used today and mean I can now supervise others to work on vaccine development."

This early work, funded by Action, saw the then Dr Pollard study how children of different ages responded to meningococcal meningitis and septicaemia – helping to demonstrate that the immune response in young children is different to older ones. Understanding this was very important.

"At that time there had been a huge increase in the number of cases and severe disease in the UK," he reflects. "The understanding gained could then be applied to understanding immune responses to vaccines. Vaccination has since had a huge impact on the particular type of meningitis which was so horrifying."

Findings from his research were incorporated into guidelines for the management of meningococcal disease. And for many years, Professor Pollard continued to work in the field of meningitis research, with two further Action projects in this area.

With fellow Oxford University researchers, Professor Pollard played a significant role in the work that would eventually lead to the Bexsero vaccine against MenB, introduced as a routine immunisation for babies in the UK in 2015. This has seen cases fall by almost two thirds.

Prior to the pandemic, Professor Pollard and his team were working on other vaccines, including those to protect children from typhoid and plague. These infections are thankfully very rare in the UK but are still a threat to millions of children around the world.

Action is honoured to have played a role in supporting Professor Sir Andrew Pollard, whose work has helped save millions of lives.

Over 45 years, Action Medical Research has funded more than 180 Research Training Fellowships at a total value of over £14 million.

Research Training Fellows 2020

Our latest Fellows aim to help children with a rare brain disease and to improve treatment for peanut allergy.

Dr Michael Eyre, of King's College London, is investigating if advanced scans, taken early on in treatment, can identify clues that could help doctors to personalise treatment for NMDAR-antibody encephalitis – a disease caused by the immune system mistakenly attacking the brain. With prompt treatment, most children survive but they face a slow recovery, and many are left with long-lasting difficulties.

Dr Eyre hopes his research will lead to earlier symptom control, shorter hospital stays and reduced long-term effects. This Fellowship is jointly funded with the BPNA.

Meanwhile, at Imperial College London, Action and Rosetrees Trust are together funding a further Fellowship. Dr Sharanya Nagendran hopes to help children with peanut allergy, a condition that has been increasing in recent decades.

A new type of treatment, called oral immunotherapy, can be beneficial but not all children can tolerate its current form. Dr Nagendran is testing an alternative approach that hopes to boost the safety and long-term effectiveness of treatment, helping more families live without fear of a life-threatening reaction.

Research grants awarded in 2020

Action Medical Research is currently funding around 60 projects across the UK, eight of them awarded in 2020.

The next medical breakthrough could be on your doorstep.

COVID-19 – does exposure early in life affect development?

Dr E Chakkarapani
University of Bristol, University of Oxford, University of Leicester, Imperial College London

COVID-19 – understanding SARS-CoV-2 infection in pregnant women and their babies

Professor K Le Doare
St George's University of London, St George's University Hospitals NHS Foundation Trust, Public Health England, London

COVID-19 – why do children respond differently to infection compared with adults?

Dr M Nikolić
University College London, Wellcome Sanger Institute, Cambridge

Duchenne muscular dystrophy – finding new drug treatments *

Professor A Russell
University of Oxford

Epilepsy – developing new drug treatments for pyridoxine dependent epilepsy *

Professor W Yue
Newcastle University, University of Oxford

Hypertrophic cardiomyopathy in children – improving diagnosis and monitoring *

Dr JP Kaski
University College London, UCL Great Ormond Street Institute of Child Health, Great Ormond Street Hospital for Children

NMDAR-antibody encephalitis – using cutting-edge brain scanning techniques to personalise treatment for a rare brain disease **

Dr M Eyre
St Thomas' Hospital, King's College London, Evelina London Children's Hospital, Guy's and St Thomas' NHS Foundation Trust

Peanut allergy – improving the safety and effectiveness of a new treatment

Dr S Nagendran
Imperial College London, Imperial College Healthcare Trust

* Jointly funded with LifeArc
** Co-funded with the BPNA (British Paediatric Neurology Association).

Locations of current research funded by Action

Thank you

We are always hugely grateful to the many individuals, companies, trusts and foundations who so generously donate to the vital work supported by Action Medical Research. In 2020, with so many of our face-to-face fundraising events cancelled or postponed, this support was more important than ever.

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

Thank you too to those who have made provision for Action in their will and to those who left us a legacy gift in 2020.

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The Hospital Saturday Fund
Humanimal Trust
Liberty Specialty Markets
The Linder Foundation
Milton Damerel Trust
Next Retail
Oso Foundation
Price Bailey
Reed Foundation
Roche Products
Rouleur
Rusk & Rusk
Sir William Coxen Trust Fund
Willmott Dixon

Legacy gifts

Jean Allen
Megan Andrew
Lady Ashtown
Pam Brindley
Mary Essame
Jack Greenall
Shirley Gristwood
Doreen Kirby
Winifred Pickersgill-Brown
Elisabeth Spaven
Margaret Teague
Patricia Tuckman
Margaret Turner
Sheila Wheeler
Dinah Wigan

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

Summarised financial statements

for year ended 31 December 2020

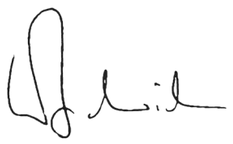
Report by the trustees on the summarised financial statements

The summarised financial statements below are extracted from the full trustees' annual report and financial statements, which were approved by the trustees and signed on their behalf on 28 April 2021. The full financial statements, on which the auditor, Buzzacott LLP, gave an unqualified audit report on 29 April 2021, was submitted to the relevant statutory bodies, including the registrar of companies, on 30 April 2021.

The auditor has confirmed to the trustees that, in their opinion, the summarised financial statements are consistent with the full financial statements for the year ended 31 December 2020.

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

Signed on behalf of the trustees



Luke Bordewich Chair

Income and expenditure	2020	2019
	£000s	£000s
Net incoming resources		
Donations and legacies	1,417	2,728
Investments	(75)	(2)
Other income	152	0
Total net incoming resources	1,494	2,726
Outgoing resources		
Medical research projects	1,912	2,598
Medical dissemination	676	748
Net outgoing resources	(1,094)	(620)
Net (losses) gain on investments	1,691	901
Net movements in funds	**597**	**281**

Balance sheet	31 Dec 20	31 Dec 19
	£000s	£000s
Fixed assets		
Tangible	295	311
Investments	11,952	11,656
Total fixed assets	12,247	11,967
Current assets	1,520	1,382
Current liabilities within one year	(5,237)	(5,293)
Liabilities falling due after one year	(1,886)	(2,009)
Total net assets	6,644	6,047
Representing:		
Unrestricted funds	6,644	6,047
Restricted funds	0	0
Total funds	**6,644**	**6,047**

Who's who 2020

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

President
Field Marshal The Lord Guthrie GCB LVO OBE DL

Vice Presidents
Stephen May
Richard Price
The Duchess of Northumberland

Trustees
Phil Hodkinson, Chair – retired December 2020, Vice President from January 2021
Luke Bordewich, Honorary Treasurer – until December 2020, Chair from January 2021
Richard Wild, Honorary Treasurer – from January 2021
Esther Alderson
Professor Sarah Bray BA MPhil PhD FMedSci
Professor David Edwards MA MBBS DSc MRCR FRCP FRCPCH FMedSci
Kathy Harvey
Karen Last
Professor David Rowitch MD PhD ScD
Richard Stoneham-Buck

Scientific Advisory Panel
Professor David Rowitch MD PhD ScD, Chair
Dr Claire Booth MBBS MSc PhD MRCPCH
Professor Clare Bryant BSc PhD BVetMed – retired November 2020
Professor Yanick Crow MBBS MRCP CCST PhD FMedSci
Professor Inderjeet Dokal MBChB MD FRCP FRCPCH FRCPath FMedSci – retired March 2020
Professor Alicia El Haj FREng FRSB FEAMBES
Professor Jonathan Grigg BSc MBBS MD FRCPCH
Professor Catherine Hawrylowicz PhD
Professor Mark Johnson PhD MRCP MRCOG – retired July 2020
Professor Samantha Johnson PhD CPsychol AFBPsS
Professor Andrea Nemeth BSc MBBS DPhil (Oxon) FRCP
Dr Adam Shortland BSc PhD MIPEM CSci
Professor Shiranee Sriskandan FRCP PhD
Professor Michael Taggart BSc PhD

Remembering our Patron

It was with great sadness that we marked the passing of HRH The Duke of Edinburgh in April 2021, Patron of our charity since 1955. He was a fantastic supporter of our work for over 60 years, generously hosting a number of receptions for the charity and taking great interest in the medical research we fund.

His passion for science and helping young children always shone through, and we are immensely grateful for his commitment and enthusiasm. We were very fortunate to have had his support for so many years to help generate funds for our life-saving work.

Thank you

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

A member of the:

amrc
ASSOCIATION OF MEDICAL RESEARCH CHARITIES

Registered with FUNDRAISING REGULATOR

FSC
www.fsc.org
MIX
Paper from responsible sources

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