action medical research for children

Research Review

Saving and changing children's lives | 2023

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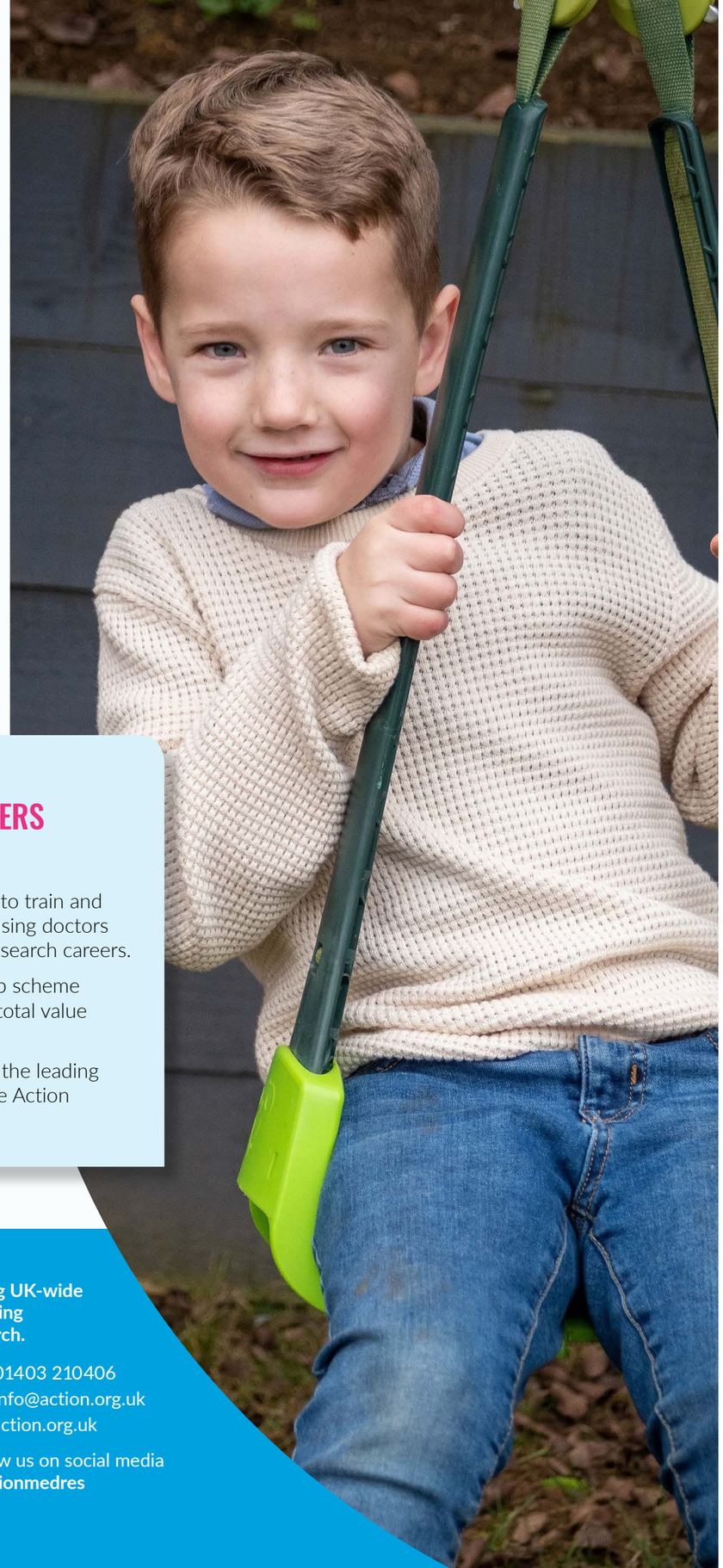

50 YEARS FUNDING RESEARCH LEADERS OF THE FUTURE

Since 1973, we've been helping to train and develop some of the most promising doctors and scientists early on in their research careers.

Our Research Training Fellowship scheme has supported 186 people, at a total value of over £15.5 million.

We are very proud that some of the leading children's researchers today were Action Research Training Fellows.

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

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researchreview 2023

WELCOME

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

In 2023 we funded 13 new research projects to help babies, children and young people. These projects, now under way, offer hope to families affected by conditions including brain cancer, epilepsy, liver disease and kidney disease.

We continued to work closely with our partners and were pleased to make two of our new awards with Borne, for research that aims to prevent preterm birth, as well as a grant together with the charity DEBRA, to help children with the skin condition epidermolysis bullosa. We also funded four new projects in our important partnership with LifeArc, researching rare diseases in children.

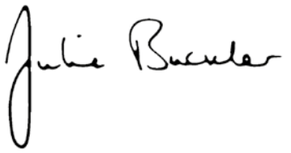
A new report in 2023 showed how our funding for research into asthma is now making a real difference for children and young people. On pages 4-5 you can read how life-changing care is being provided through personalised treatment, thanks to work we funded in 2017.

Other exciting progress included researchers moving a step closer to developing new drugs to treat children with Duchenne muscular dystrophy, and a common diabetes medicine shown in the laboratory to be effective in protecting babies from brain damage at birth.

It was also a special year, as we celebrated 50 years of our Research Training Fellowship scheme. This develops doctors and researchers early in their careers, helping to develop future leaders in children's research. Throughout this Research Review, you can see just some of the people we've supported in the past who have gone on to achieve research success. We also awarded two new fellowships in 2023, with great potential to make a difference for children through research.

Action is proud to be saving and changing children's lives and this is thanks to the support of many trusts, foundations, companies, individuals and groups, as well as our expert scientific panel. We remain deeply grateful to all of our supporters, including our network of volunteers and local committees, and those who so generously remember Action with gifts in their wills.

But there are still hundreds of thousands of sick and disabled babies and children in the UK who need our help. Please help us fight for little lives.



Julie Buckler
Chief Executive
Action Medical Research for children

Ian, on the front cover, is enjoying the positive effects of Frame Running. You can read more about the success of this research, and how it's also helped another child, Poppy, on page 19.

IMPACT FOR CHILDREN

A GAME-CHANGING NEW APPROACH TO TREATING ASTHMA

We fund medical research to make a real difference to children's lives. An exciting example is funding that supported a pioneering clinical trial, showing that personalising medication, according to genetic make-up, can improve treatment for children and young people with asthma.

Asthma causes coughing, wheezing and difficulty breathing, and is a leading cause of emergency hospital admission in children and young people.

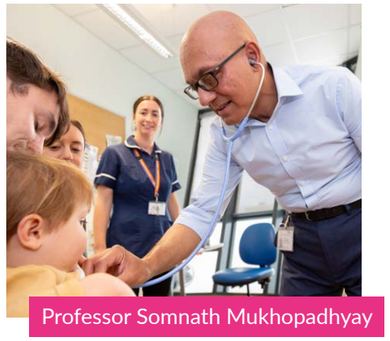
When the condition is managed well, children can lead a full and active life. But when it's not, it can severely limit their ability to play, run and be active, and affect attendance at school.

Evidence had been emerging that one of the most commonly used asthma controller medications, called salmeterol, was not equally effective in all children. This means that some do not see the expected benefits and continue to experience symptoms, including asthma attacks.

Supported by Action funding of more than £270,000, awarded in 2017, an expert team, led by Professor Somnath Mukhopadhyay at Brighton's Royal Alexandra Children's Hospital, ran an exciting clinical trial.

The first of its kind, this trial involved 240 children aged 12 or over and tested prescribing treatment based on genetics. This is an approach known as personalised medicine.

> "Action's funding was critical in supporting the first-ever clinical trial of this kind of precision medicine for children's asthma"
>
> Professor Somnath Mukhopadhyay



Professor Somnath Mukhopadhyay

A cheap, simple saliva test was used to determine children's genetic make-up. They were then prescribed either salmeterol or another drug called montelukast, according to the results of the test.

The research showed that a personalised approach could significantly improve children's response to treatment and therefore their quality of life, compared to standard care.

"This work is having much positive impact in the UK, and across the world," says Professor Mukhopadhyay.

In the UK alone, an estimated 150,000 children with asthma have the genetic make-up that means salmeterol is likely to be less effective. As a result of this research, life-changing care is now being provided for some of these children. The hope is this will extend to help many more, worldwide, in the near future.

We are grateful to the many trusts and foundations who helped us support this research, including The Henry Smith Charity.

IN THE UK ALONE, AN ESTIMATED

150,000

CHILDREN COULD BENEFIT

A LIFE-CHANGING EFFECT

Christian was one of the children who took part in this clinical trial. Changing his treatment, based on his genetic profile, has finally brought his asthma under control, giving him freedom to enjoy life with his friends.

Christian had struggled with severe asthma for many years and the highest doses of medication didn't seem to help. His mum, Catherine, said it felt like they'd run out of options. "We were at the doctors' surgery all the time," she says.

For Christian, his asthma limited his ability to take part in sports, even though he really wanted to. "It made it very difficult – I'd try but it wouldn't be long before I'd start to cough and feel breathless," he says.

It turned out that Christian had the genetic change that meant he wouldn't respond as well to salmeterol. "His inhaler was changed and bingo!" says Catherine.

Christian says his new treatment has made a big difference.

"I can now participate in sports that I previously struggled with, like football, badminton and going to the gym."

"It's allowed me to do lots of things with my friends that I would have struggled with before."

"It's been life-changing," adds Catherine. "We are so grateful to all the charity supporters who helped make this work possible and feel so lucky that we've been able to benefit from it."

> "He can just get on with life now. It's had such an amazing impact"
>
> Christian's mum, Catherine

FIGHTING RARE DISEASES

Families facing rare diseases can experience some of the toughest fights imaginable. Diagnosis can all too often be a very long and painful journey. But this is only the start.

Even when parents find out why their child is so unwell, there may be few answers and very limited options. Sadly, the vast majority of rare diseases – around 95 per cent – have no affective treatment or cure. Many are also life-limiting.

For families facing a life-changing diagnosis, medical research gives hope – hope for new treatments, hope for a cure and hope that in the future no family will have to go through the heartbreak of losing a child to a rare disease.

Our rare disease research campaign continues to raise awareness and funds for vital research into conditions that can cause so much suffering for children and their families. Thanks to supporters, we have raised almost £600,000. But so much more needs to be done. While individually rare diseases may affect a very small number of people, collectively they affect many thousands in the UK alone – and 70 per cent of rare diseases begin in childhood.

With your help, we can do more to fight rare diseases. Our support of early-stage research is especially important. This is often the starting point that leads to new discoveries and, eventually, new treatments, bringing hope to families.

AROUND
95%
OF RARE DISEASES LACK AN EFFECTIVE TREATMENT

50 YEARS FUNDING RESEARCH LEADERS OF THE FUTURE

Pioneers in treating rare diseases

Some researchers we supported early in their careers as Research Training Fellows have gone on to make major achievements to help children with rare diseases.

In 1975, the late Professor Roland Levinsky received one of Action's first fellowships, training in immunology of childhood diseases. Shortly after completing his fellowship, he performed the first successful bone marrow transplant in a child at Great Ormond Street Hospital.

By 1983 he was one of only two paediatric immunologists in the UK.

Professor Levinsky's later work helped to discover the genetic basis of several rare diseases and contributed to the development of gene therapies to treat rare, previously fatal, immunodeficiency conditions.

He would also teach Professor Bobby Gaspar (then Dr Gaspar), pictured, who received a fellowship in 1993. Today, Professor Gaspar continues to develop life-saving gene therapies which can cure children with very severe rare diseases.

"Each new development brings real hope – and that is worth fighting for, so that families in the future don't have to feel that the bottom is dropping out of their world"

Sally, mum of Danny, pictured, who has Hunter syndrome

HELPING CHILDREN LIKE EVA

Three-year-old Eva has a very rare liver disease that causes severe and progressive damage. Treatment options are extremely limited, with affected children eventually needing a transplant.

Eva is a very chatty little girl, who loves the outdoors and going to forest school. “She’s a real bright spark,” says her mum, Sophie. “She goes to nursery and is a happy little thing.”

After a very tough start in life, Eva has had a period of better health, something her parents are incredibly grateful for. But unfortunately, the family has no certainty as to how long it will last. “It feels a bit like a ticking time bomb,” says Sophie. “At the moment, we’re enjoying having her well enough to live relatively normally, but we know there will come a time when her health degrades significantly again.”

When she was nine months old, Eva was diagnosed with progressive familial intrahepatic cholestasis type three or PFIC3. This is caused by faults in a gene called *ABCB4*, which provides the instructions to make a protein needed for healthy liver function. The condition causes damage and scarring which eventually leads to liver failure.

As a baby Eva was extremely thin, struggled to feed or sleep and had very elevated liver enzymes. She’d been re-admitted to hospital, following a seizure, when she was 10 days old and spent most of the first months of her life there.

“She was classed as failing to thrive,” explains Sophie. “She had constant sickness and diarrhoea, and was very, very thin and very distressed. She wasn’t really growing and was still in tiny or premature baby clothes for a long time. We were very worried. But because the disease is so rare, it was really difficult to get a diagnosis and to understand why she was so poorly.”

In the end it was Sophie, a biologist by background, who’d asked for Eva to be tested for PFIC. “I did loads of online research and have been told that we probably wouldn’t have had a diagnosis when we did if I hadn’t been pushing for it,” she says.

Since being diagnosed, Eva has been taking a liver medication and for the last two years has been generally well. But she already has scarring to her liver and recently her liver enzyme levels have begun to rise again. She is also developing nutrient deficiencies such as anaemia.

“We’re very concerned about the long-term impact of these deficiencies,” says Sophie. “Although this is a liver disease, it can affect the whole body, including the brain, eyesight and growth.”

70%
OF RARE DISEASES START IN CHILDHOOD

FIGHTING RARE DISEASES

> “We know there will come a time when her health degrades significantly”
>
> Eva’s mum, Sophie

“The longer Eva remains well, the better in terms of her development and growth,” says Sophie. “But also, the more time there is for research. There are so few treatment options – really, it’s just the medication she’s on now or a liver transplant.”

“The drug Eva takes is not a cure. It just slows things down and buys us time, which is the best we can hope for at the moment.”

NEW RESEARCH

Action Medical Research is funding a project to develop gene therapy for this rare liver disease.

Led by Professor Paul Gissen at the UCL Great Ormond Street Institute of Child Health, researchers plan to use a harmless virus to deliver a working copy of the faulty gene that causes PFIC3 into liver cells. This type of gene therapy has already been used to treat other genetic diseases.

“If successful, this could help to transform the lives of children, sparing them from the long waits and complications often associated with liver transplants,” says Professor Gissen. “It could also pave the way to the development of similar gene therapies for children with other severe liver diseases in the future.”

TREATING RARE HEART CONDITIONS

Arrhythmogenic cardiomyopathy describes a group of very rare, inherited heart conditions that can cause sudden cardiac arrest. Dr Sara Moscatelli, a new Action Research Training Fellow, aims to improve understanding of how it develops in children, to improve treatment and identify those at greatest risk.

Arrhythmogenic cardiomyopathy is a condition where the heart muscle cells don't stick together properly. This can cause the walls of the heart to become weak, meaning blood won't be pumped as well as it should.

Symptoms can include abnormal heart rhythms, fainting, breathlessness and chest pain. Some children show no symptoms at all but can still be at risk of life-threatening complications.

While there's no cure, treatments can help to reduce and control the symptoms and risks. But current approaches are determined by data from affected adults, not children.

Dr Moscatelli, of Great Ormond Street Hospital, is studying a group of children who are either already affected or carry the faulty gene that causes the condition. She aims to identify early signs that can help predict how the disease will develop. This could lead to personalised approaches to treatment, helping to prevent sudden death in children and young people.

> "Early diagnosis and treatment could help save lives"
>
> Dr Sara Moscatelli

> Steps forward

IMPROVING TREATMENT FOR DUCHENNE MUSCULAR DYSTROPHY

With Action funding, researchers have moved a step closer to developing new drugs to treat children with this progressive muscle-wasting disease.

Duchenne muscular dystrophy causes muscle weakness and wasting that gradually worsens over time. It almost always affects boys and sadly there is no cure. Although life expectancy is increasing, most will develop heart and breathing problems that lead to life-threatening complications.

In the UK, about 100 boys are born with Duchenne each year. It's caused by a faulty gene which means the body doesn't make enough, or any, of the protein dystrophin, which protects muscles. While gene therapies are showing exciting progress, they are not suitable for all patients.

With joint funding from Action and LifeArc in 2020, Professor Angie Russell and her team aimed to identify drug compounds that could become a much-needed new treatment for all.

> "A big advantage of utrophin replacement therapy is that it is potentially suitable for all patients"
>
> Professor Angie Russell

The team, based at the University of Oxford, focused on drugs that would boost utrophin, another protein found in muscles that could act as a substitute for dystrophin. They successfully identified a number of potential new drug compounds and now understand how these work within cells to increase utrophin levels.

Professor Russell says: "These findings have allowed us to secure a further £2.2m in funding from the Medical Research Council to now develop these promising new molecules into an effective and long-lasting utrophin replacement therapy for the benefit of all children with Duchenne."

FIGHTING FATAL BRAIN CANCER

New research aims to develop an urgently needed new approach to treat rare and incurable childhood brain tumours.

Diffuse midline glioma is the most lethal form of childhood brain cancer. These tumours are highly aggressive, growing in the midline between the two halves of the brain. Unfortunately, they cannot be removed with surgery and although radiotherapy can temporarily slow down their growth, this is not a cure.

"The outlook for children is currently very poor," says Dr Antonis Pouliopoulos. "Sadly, their lives will be cut short – with an average survival of less than a year."

With co-funding from Action and LifeArc, Dr Pouliopoulos and his team at King's College London aim to develop an innovative and non-invasive approach. This will use gene therapy combined with focused ultrasound to deliver highly targeted treatment, that is personalised to each patient's tumour.

"We hope our results will help pave the way for a future clinical trial in children with diffuse midline gliomas – offering new hope for these desperately ill children and their families," says Dr Pouliopoulos.

SAVING TINY LIVES

Action Medical Research has a long and proud history of playing a role in landmark medical breakthroughs that have gone on to become part of routine pregnancy or neonatal care.

These include helping to fund research that discovered the importance of taking folic acid before and during pregnancy to prevent spina bifida, and work that helped introduce ultrasound scanning in pregnancy.

We continue to fund vital new research that aims to give all babies the best possible start in life. With current Action funding, researchers are striving 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.

OVER
90,000
BABIES ARE CARED FOR IN NEONATAL UNITS IN THE UK EVERY YEAR

50 YEARS FUNDING RESEARCH LEADERS OF THE FUTURE

Preventing vitamin K deficiency

One of the earliest Research Training Fellows, Dr John Tripp was awarded a grant in 1974. His work would later lead to guidelines on how to treat vitamin K deficiency in newborn babies.

All babies are born with low levels of vitamin K, which is vital for the production of blood clotting factors. This puts them at risk of developing vitamin K deficiency bleeding, previously known as haemorrhagic disease of the newborn. This can be life-threatening or cause brain damage.

Thankfully, it is now far less common than it was four decades ago due, in part, to routine guidance on the administration of vitamin K to all newborn babies, to which Dr Tripp contributed. Preventative treatment is now recommended and offered to all babies born in the UK.

> Steps forward

PROTECTING NEWBORN BABIES FROM BRAIN DAMAGE

Researchers have shown that a commonly used diabetes medicine can protect the brain from damage following complications at birth. Importantly, this work has now led to further funding to allow progress towards a clinical trial.

Hypoxic-ischemic encephalopathy, or HIE, can cause lasting damage to a baby's brain. It's usually caused by a lack of oxygen at or around birth and estimates suggest it affects over 2,000 babies in the UK each year. Sadly some die, and many others develop irreversible and lifelong learning and physical disabilities, including cerebral palsy. The emotional and financial cost of caring for affected children can be enormous.

Cooling therapy, developed with previous Action funding, can help some babies, but over half of those who develop HIE still cannot be saved or develop serious disabilities. So, a new treatment that can be combined with cooling to enhance its effectiveness is needed.

Led by Professor Ahad Rahim, researchers have now tested a drug called exendin-4 to see whether it could help to protect a baby's brain – and shown it to be highly effective.

The team also showed that it can be combined with cooling to improve its effectiveness even further. The drug is already used as a treatment for adults with other conditions, so it is hoped it can be quickly developed to treat babies.

This study, funded by Action in 2016, has now enabled the researchers to secure further government funding of £1.5 million from the Medical Research Council. This will allow the work to progress forwards, to improve treatments for babies shortly after birth.

“Action funding was very important in helping us move this work further towards the clinic”

Professor Ahad Rahim

Aiden developed HIE following a very traumatic birth

PREVENTING PREMATURE BIRTH

Sam was born extremely prematurely, during the height of the COVID-19 pandemic in 2020. He weighed just 790g, less than a bag of sugar, and spent almost four and a half months in the neonatal unit.

Sam's mum, Zalena, had been enjoying a problem-free first pregnancy, with no complications – until the week that she went into premature labour. She was just 24 weeks pregnant, meaning her baby would be born more than three months early.

With Sam's arrival imminent, Zalena was given drugs to try and prepare for a premature birth. These included magnesium sulfate to try and protect Sam's brain and steroids to help strengthen his lungs. When he was born, tiny Sam was immediately put on a ventilator, to help him breathe, and taken to the neonatal intensive care unit.

Zalena describes the first few months of Sam's life as a rollercoaster. "We felt bewildered, shocked, scared, devastated…" she says. "But also happy and proud of him. We took it one day at a time. At times we took it one minute or one hour at a time. We agreed to only focus on the information in front of us and not the what ifs because it was just too overwhelming."

The earlier a baby is born, the greater their risk of serious complications and Sam was to fight numerous battles. At five days old he developed necrotising enterocolitis (NEC), a life-threatening condition where tissue in the gut becomes inflamed. This saw him have emergency surgery to remove part of his bowel. He also suffered a collapsed lung, needed a temporary lung, had kidney failure, and suffered a series of infections.

"At one stage it was feared that he might lose his sight," says Zalena. "He developed the most aggressive form of retinopathy of prematurity, a condition when abnormal blood vessels grow in the retina. He needed three lots of injections and laser surgery to save it."

Sam would spend a total of 139 days in neonatal intensive care. "The staff saved his life on countless occasions and became our extended family," says Zalena. "Because he was born early on in the pandemic, we were not able to see our friends and family. So our support network became the doctors and nurses."

Sam is now three years old and is thankfully a happy little boy who's doing well. He still has some health issues as a result of his early birth, but Zalena describes him as 'a little miracle'.

Zalena says her experience of premature birth changed her forever. "I am not the same person. You cannot go through this without being affected," she says.

AROUND
55,000
BABIES ARE BORN PREMATURELY EVERY YEAR IN THE UK

"It's shocking that thousands of families go through this every year. This is why we need more research"

Sam's mum, Zalena

"Before this happened to us, we had no idea of what it meant to have a baby at such extreme prematurity. The journey is volatile and hugely emotional. It's shocking that thousands of families go through this every year. This is why we need more research."

NEW RESEARCH

The causes of preterm birth remain poorly understood but an infection may be a trigger in around 40% of women who experience an unexpected early labour.

With co-funding from Action and Borne, researchers are aiming to develop a treatment to boost the body's natural defences in women who are known to be at higher risk of premature birth.

As well as providing a physical barrier to the womb, the cervix also produces specialised antimicrobial proteins to help fight infection. Researchers at University College London aim to boost this effect in cervical cells, which they hope will help to stop bacteria from entering the womb.

This could lead to a new treatment to reduce the risk of early labour, helping to save tiny lives and prevent the serious health issues that prematurity can cause.

TACKLING INFECTIONS

A TEST TO PROTECT BABIES

Cytomegalovirus, or CMV, is a common and usually harmless virus. But it can sometimes cause serious disabilities, such as sight or hearing loss, in babies who contract it during pregnancy.

Early detection and prompt treatment of congenital CMV helps to improve outcomes. But there is currently no routine screening for newborn babies, partly because of the lack of a suitable diagnostic device.

With Action funding, Professor Vincent Teng and his team at Swansea University want to change this. They are developing a new, low-cost, highly-sensitive device that could give rapid results, enabling timely treatment to help more babies.

Such a device could, in the future, make a difference for children like Mylo.

> "If we had known Mylo had CMV at birth, he could have had immediate treatment"
>
> Mylo's mum, Ruth

MYLO'S STORY

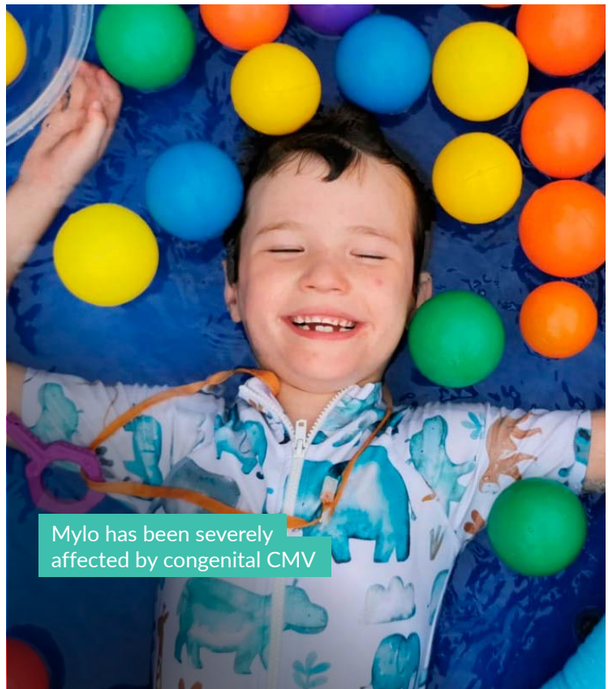
When Mylo was born, his parents Ruth and Adam already knew there was potentially something wrong. A routine scan at 36 weeks had shown excess fluid on his brain, but the cause was unknown.

Mylo failed his newborn hearing test and was soon missing early milestones. When he was three months old, he was finally diagnosed with congenital CMV.

"We hoped that the antiviral drugs he was given would be a miracle treatment," says Ruth. "But as time went on his complex needs emerged, seemingly by the day."

Mylo, now eight, 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.

"Had we known at birth that Mylo had CMV, he could have had immediate treatment, which may have made it more successful," says Ruth. "We were told that ideally it should be administered within six weeks to maximise the effect. That Mylo has been affected so severely is still a hard pill to swallow."



Mylo has been severely affected by congenital CMV

> Steps forward

FIGHTING NEW STRAINS OF MENINGITIS

With Action funding, researchers have identified new and emerging strains of bacteria that are now responsible for most cases of pneumococcal meningitis in young children. This will inform vaccine policy and treatment in the near future.

Pneumococcal meningitis mostly affects babies and toddlers and can have devastating consequences. Up to one in seven lose their lives and a quarter are left with severe after-effects, such as sight or hearing loss.

In the UK and Ireland, vaccines now protect against the most common strains of pneumococcus bacteria. But little was known about the risk, severity and outcomes of meningitis caused by new and emerging strains.

With funding awarded in 2018, Dr Godwin Oligbu and his team at Public Health England collected and analysed data and biological samples from children to identify the bacterial strains involved.

He says: "Our work will inform vaccine policy makers of the predominant types of bacteria now causing meningitis and fatalities. It will also provide guidance for paediatricians to better manage the condition."

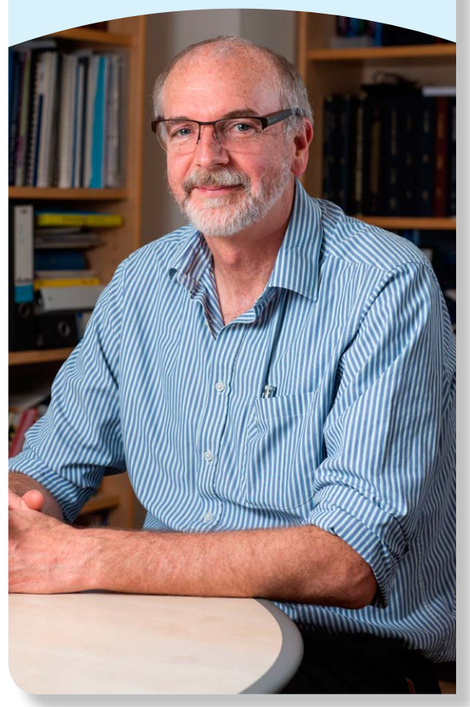
50 YEARS FUNDING RESEARCH LEADERS OF THE FUTURE

Developing life-saving vaccines

Professor Sir Andrew Pollard has spent much of his career preventing and treating infections that threaten children, especially meningitis.

Action supported him with a Research Training Fellowship back in 1995. This enabled him to complete his PhD and, he says, played a key role in his development to becoming a leading authority in paediatric immunity and vaccinology.

He played a significant role in work that led to the Bexsero vaccine against meningitis B, now a routine immunisation for babies in the UK. He has also led global teams working on other vaccines, such as those to protect children from typhoid, and was also at the forefront of vaccine development during the COVID-19 pandemic.



HELPING CHILDREN WITH DISABLING CONDITIONS

NEW RESEARCH TO TREAT RECURRENT WHEEZING

Our research strives to help children affected by physical disability or chronic conditions that impact their quality of life. One of our new 2023 Research Training Fellows, Dr Shalini Hillson aims to improve treatment of wheezing in pre-school children.

Wheezing is common in babies and young children. It results from a narrowing of the airways, making it harder to breathe. While many pre-school children outgrow their symptoms, one in three will go on to develop asthma or poorer lung function later in life.

Unfortunately, the protocol for treating early wheezing hasn't really changed in more than 20 years and is limited in its effectiveness.

Its 'one-size-fits-all' approach relies heavily on inhaled steroids. But research suggests this often has no major effect.

"We urgently need a step-change in the way we treat young children with wheeze that accounts for its different underlying causes," says Dr Hillson.

She is now testing and assessing the feasibility of a set of simple bedside tests. These would be used to distinguish between different types of wheezing and help match the best treatment to each child.

This Research Training Fellowship is supported by The Masonic Charitable Foundation.

> Steps forward

A NEW SPORT FOR CHILDREN WITH REDUCED MOBILITY

Action funding has helped to develop Frame Running for young people with cerebral palsy and other conditions that limit mobility. This is already having a positive impact for children like Poppy, pictured right.

Poppy has a rare neuromuscular condition called acute flaccid myelitis, which developed suddenly following a virus when she was six years old. This caused inflammation and damage to her spinal cord, leaving her with paralysis and weakness in her upper body and core. She has had to learn to sit, stand and walk again, and now uses a wheelchair.

The opportunity to try Frame Running proved to be a positive turning point for Poppy. The activity involves propelling a three-wheeled running frame, with a saddle, body support and no pedals.

Poppy was one of the children who took part in a pilot study of the sport, funded by Action Medical Research and the Chartered Society of Physiotherapy Charitable Trust in 2018. This study, led by Dr Marietta van der Linden and Dr Jennifer Ryan, assessed if Frame Running could encourage children and young people to increase their physical activity, and if it had a lasting positive effect on their health and wellbeing.

Before her illness, Poppy was a very sporty child says her mum, Laura, and she had become very disheartened with the challenges she now faced. "Being a Frame Running athlete does a huge amount for Poppy's mental and physical health," says Laura. "It has been an amazing opportunity for her to work on her fitness and strength, while also having fun and feeling accomplished."

"Poppy left one of the early Frame Running sessions saying Mummy I'm so happy there is a sport I can do"

Poppy's mum, Laura

RESEARCH GRANTS AWARDED IN 2023

Action Medical Research is currently funding around 45 projects across the UK, 13 of them awarded in 2023. **The next medical breakthrough could be on your doorstep.**

Developing a cutting-edge treatment for a severe form of epilepsy
Professor Cathy Abbott
University of Edinburgh, University of Oxford

Searching for a new drug treatment for a rare debilitating skin condition [1]
Professor John Connelly
Queen Mary University of London

Developing a new gene therapy for children with a rare severe liver disease
Professor Paul Gissen
UCL Great Ormond Street Institute of Child Health, University College London

Personalising treatment for pre-school children with recurrent wheezing to reduce the long-term impact on their lung health [2]
Dr Shalini Hillson
Imperial College London, Royal Brompton Hospital, London

Arrhythmogenic cardiomyopathy – personalising treatment for these rare inherited heart conditions
Dr Sara Moscatelli
Great Ormond Street Hospital, London, UCL Great Ormond Street Institute of Child Health, St George's University of London

Craniofacial differences – designing customised face masks to help children on ventilation to breathe [3]
Dr Connor Myant
Imperial College London, Sheffield Children's NHS Foundation Trust

Developing a new approach to treat rare and incurable brain tumours [4]
Dr Antonis Pouliopoulos
King's College London

Developing a new drug treatment for a rare inherited kidney condition [4]
Professor Moin Saleem
University of Bristol

Preterm birth – investigating a surgical procedure to help reduce the risk after previous emergency caesarean section [5]
Professor Andy Shennan
St Thomas' Hospital, King's College London

Developing a diagnostic device to screen newborn babies for cytomegalovirus
Professor Kar Seng (Vincent) Teng
Swansea University, Cardiff University

Understanding the role of vitamin B6 in epilepsy to help improve treatment
Dr Karin Tuschl
UCL Great Ormond Street Institute of Child Health, University College London

Developing an antimicrobial therapy to help prevent preterm labour in women at high risk [5]
Professor Simon Waddington
University College London

Krabbe disease – developing a potential new drug treatment [4]
Dr Helen Waller-Evans
Cardiff University

[1] Jointly funded with DEBRA UK

[2] This Research Training Fellowship is generously supported by The Masonic Charitable Foundation

[3] Jointly funded with LifeArc and supported by a generous grant from the VTCT Foundation

[4] Jointly funded with LifeArc

[5] Jointly funded with Borne

Locations of research funded by Action

A LASTING LEGACY

Gifts in wills are a vital source of income to us 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.

We'd like to remember these kind supporters whose legacy gifts were received in 2023:

Pat Bowman
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Mary Hughes
Patricia Jeffreys
Grace Jones
Mary Mahy
Andrew Middlemiss
David Norris
Gertrude Palmer
Colin Reed
Maureen Rhodes
Diana Webber
Jane Wightman

"The impact that research can have on people's lives cannot be underestimated"

Anne and Stuart Simpson's son Paul had spina bifida and was the youngest person in the UK to benefit from a revolutionary walking device developed with Action funding. Paul sadly died when he was 14, due to complications associated with his condition. His legacy lives on in many ways. One of these is in his parents' decision to leave a gift in their wills to Action.

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**

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 around £9m invested in the work of more than 180 top researchers, working on around 45 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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WHO'S WHO 2023

Former Royal Patron
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President
Field Marshal The Lord Guthrie GCB LVO OBE DL

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Clare Ferguson joined July 2023
Kathy Harvey
Karen Last
Professor David Rowitch MD PhD ScD
Professor Stephanie Schorge PhD
Rajat Sharma
Richard Stoneham-Buck

Scientific Advisory Panel
Professor Stephanie Schorge PhD, Chair
Professor Diana Baralle MBBS MD FRCP joined August 2023
Professor James Boardman MBBS, MSc, PhD joined March 2023
Professor Claire Booth MBBS MSc PhD MRCPCH retired August 2023
Professor Adnan Custovic MD PhD FRCP FMedSci joined November 2023
Professor Alicia El Haj FREng FRSB FEAMBES retired May 2023
Professor Deborah Gill PhD joined November 2023
Professor Muzlifah Haniffa BSc MBBCh MRCP PhD FMedSci retired November 2023
Professor Ed Johnstone MBChB PhD MRCOG
Professor Pablo Lamata PhD joined February 2023
Professor Robert McFarland MA MBBS PhD MRCP FRCPCH joined July 2023
Professor Andrea Nemeth BSc MBBS DPhil (Oxon) FRCP retired May 2023
Professor Shiranee Sriskandan FRCP PhD retired May 2023

Scientific Advisory Panel for joint Action Medical Research/LifeArc project grant applications on rare disease research
Professor Stephanie Schorge PhD, Chair
Professor Diana Baralle MBBS MD FRCP joined August 2023
Professor James Boardman MBBS MSc PhD joined March 2023
Professor Claire Booth MBBS MSc PhD MRCPCH retired August 2023
Professor Paul Brennan PhD retired August 2023
Professor Adnan Custovic MD PhD FRCP FMedSci joined November 2023
Professor Alicia El Haj PhD FREng FRSB FEAMBES retired May 2023
Dr Thorsten Forster PhD
Professor Deborah Gill PhD joined November 2023
Professor Muzlifah Haniffa BSc MBBCh MRCP PhD FMedSci retired November 2023
Professor Pablo Lamata PhD joined February 2023
Professor Robert McFarland MA MBBS PhD MRCP FRCPCH joined July 2023
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Professor Andrea Nemeth BSc MBBS DPhil (Oxon) FRCP retired May 2023
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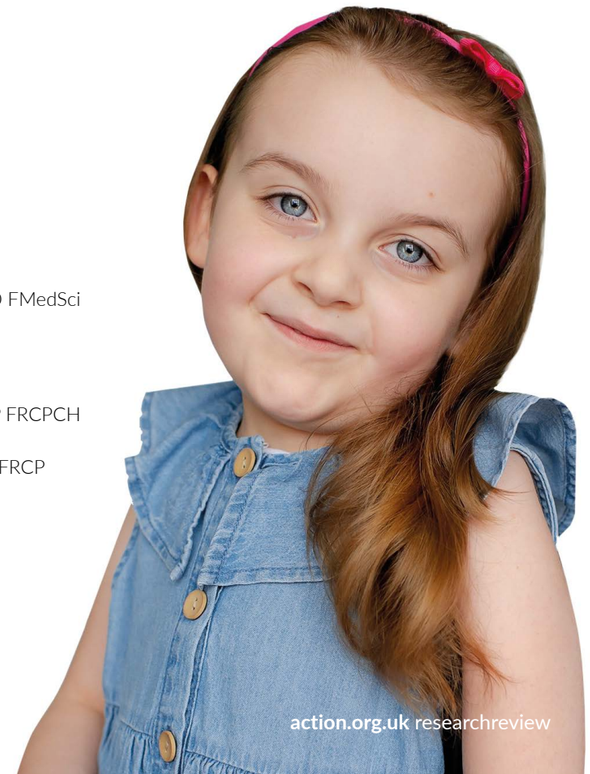

THANK YOU

action medical research for children

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Front cover photo from the family