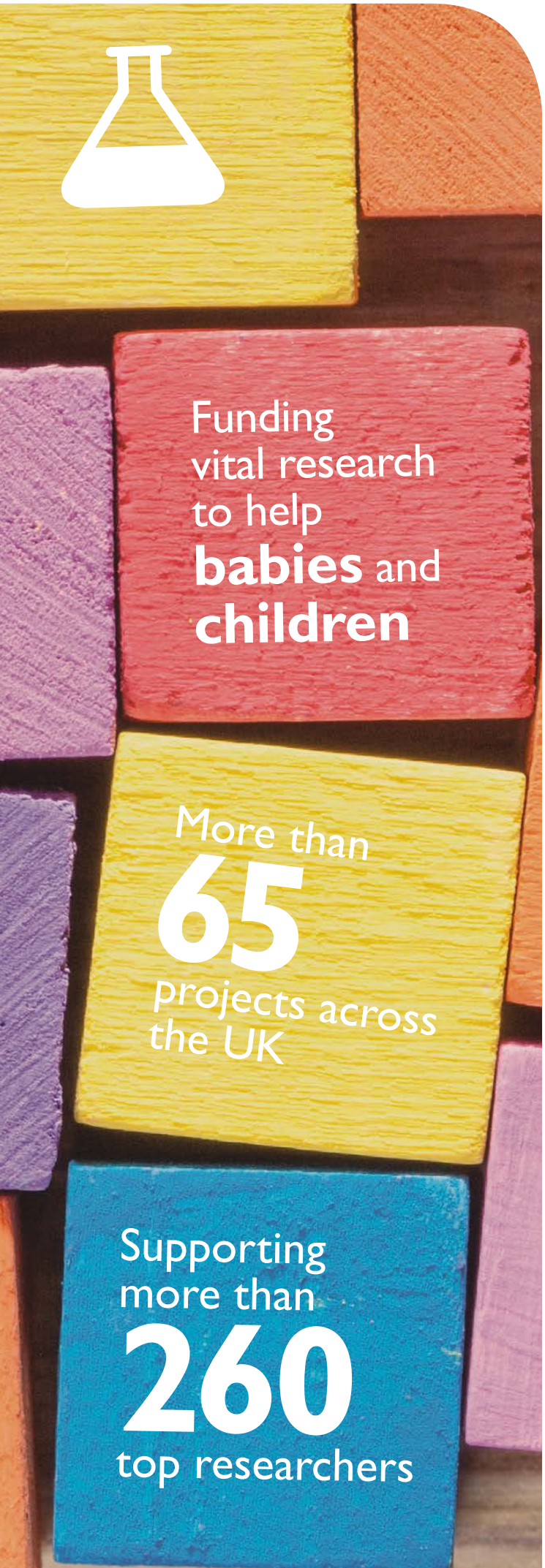
action medical research for children

Research Review 2017

Saving and changing children's lives

Contents



Welcome

In 2017 we celebrated 65 years of funding extraordinary medical breakthroughs. We are proud that since our charity began in 1952 we have invested over £110 million in research, some £318 million in today's terms.

Building on our successes in helping to beat polio in the UK, fighting meningitis and developing ultrasound scanning in pregnancy, last year we saw the impact of our funding to develop and test a new way of using electrical stimulation to help children with dropped foot walk more easily.

Exciting progress in 2017 resulting from our funding also included new drug combinations to make treatment more effective for the childhood cancer neuroblastoma – and a test that could be used in early pregnancy to identify women at high risk of going into labour too soon.

We funded 17 brand new research projects to help babies, children and young people, all identified through our world-class peer review system. We also continued working in partnership with other charities and funded the first of our projects together with Borne to prevent premature birth.

Such achievements are only possible thanks to the support of many wonderful people and organisations. We are grateful to our network of local committees and volunteers who work so very hard, as well as to those people who thoughtfully remember Action with gifts in their wills. We greatly appreciate support from many charitable trusts and especially the Exilarch's Foundation.

At Action, we're on a mission to save and change children's lives through medical research. Please join us and help fund more research that could make such a difference.

Julie Buckler
Chief Executive
Action Medical Research

Saving tiny lives

Preventing premature birth

Being born too soon can have a life-changing impact and sadly some babies don't survive. We're funding research which aims to reduce the number of babies born prematurely and give the best possible outlook for affected families like Lucy and Olivia's.

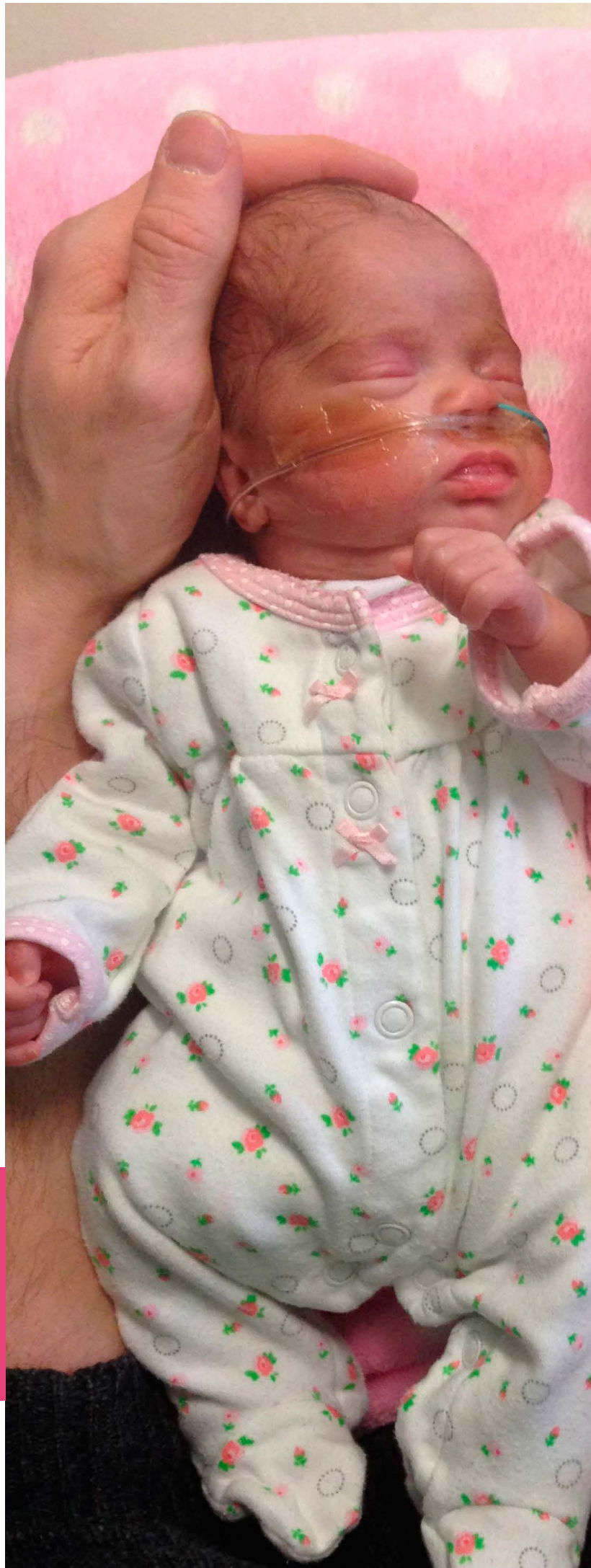
Twins Lucy and Olivia were born almost four months before their due date and, more than four years on, the trauma of giving birth so very early will, says their mum Gemma, stay with the family forever.

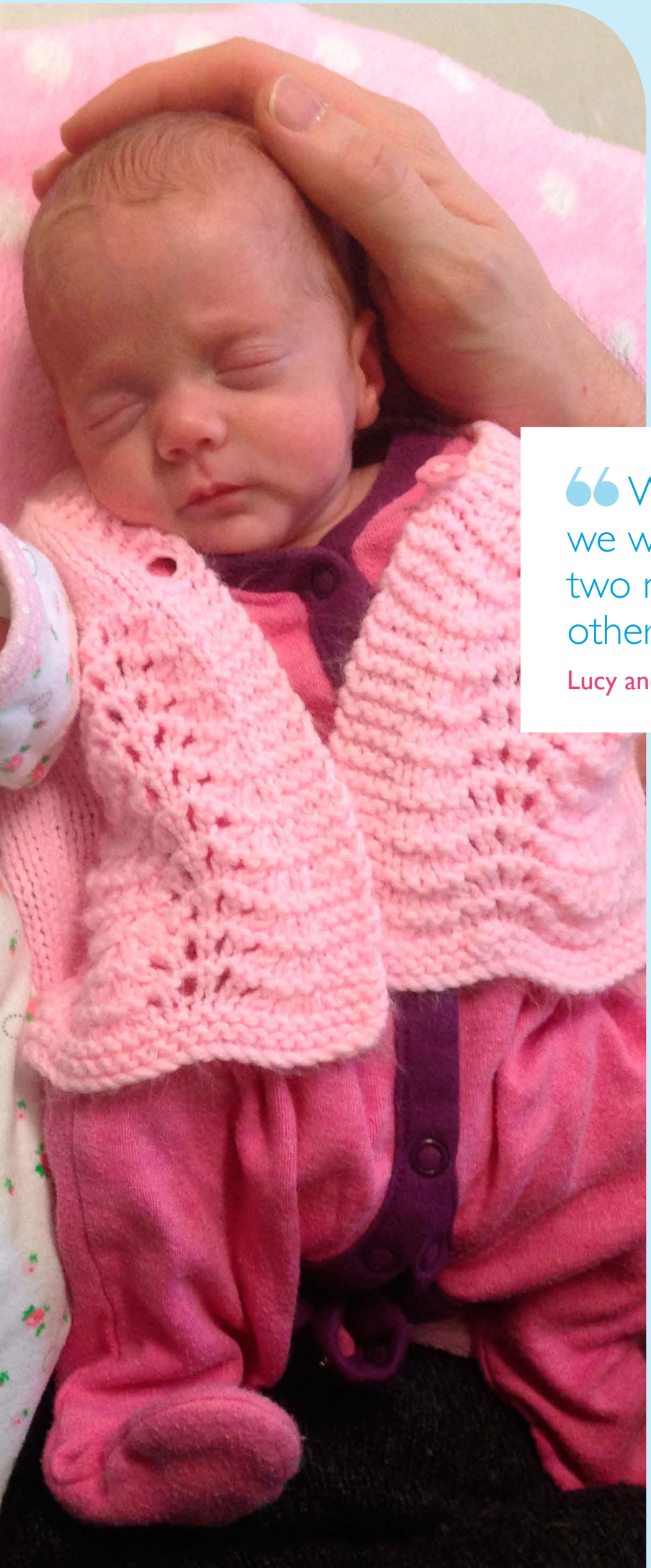
"My girls were much-wanted IVF babies and my pregnancy was totally normal," says Gemma. "I felt well, and expected everything to be fine."
But after experiencing a little blood loss 25 weeks into her pregnancy, Gemma was admitted to hospital. With her babies not due for 15 weeks, Gemma was bewildered when medics asked if she was feeling any tightening sensations.

Fearing that labour was beginning, doctors gave her medicines to try and slow things down. The treatment initially seemed effective but worked only for a few days. Lucy and Olivia were born weighing just 1lb 12oz and 1lb 9oz.

The following hours, days and weeks were a rollercoaster of fear and worry. Gemma and her husband Will did not know from one hour to the next whether breathing difficulties or infection might claim their tiny, fragile daughters' lives. While Lucy was able to breathe on her own after just one month, Olivia stopped breathing several times and needed life-saving treatment.

Around **61,000**
babies are born prematurely every year in the UK





Gemma describes their months in hospital as a time of 'long, drawn out fear'. Even when the girls were eventually allowed home, weighing just 4lbs, family life was dominated by anxiety. "Olivia was still on oxygen," she explains. "So we had canisters fitted at home, and portable canisters for when we went out. Taking newborn twins out is hard at the best of times, but for us it was so frightening."

> "We are really aware that we were lucky. During our two months in NICU, five other babies died"
>
> **Lucy and Olivia's mum, Gemma**

"When babies are born extremely prematurely, all the lovely bits of having a baby are taken away. You feel very far removed from all of your friends," adds Gemma.

The outlook for the girls' long-term wellbeing was uncertain, with doctors unable to give any reassurance. "It was totally unclear if they would walk, talk or even smile," says Gemma. Thankfully, both are smiling, laughing, talking and walking, but anxiety about their wellbeing still casts a deep shadow. "The fear of them dying hasn't gone," says Gemma. "I didn't go to baby groups because I was scared of them getting infections. And I'm terrified of them starting school."

For Gemma and Will research into the causes of premature birth, like that funded by Action, is incredibly important. "It means everything," she says. "We need to know more, to stop premature birth happening in the first place. Without more research, people will end up coming home without their babies."

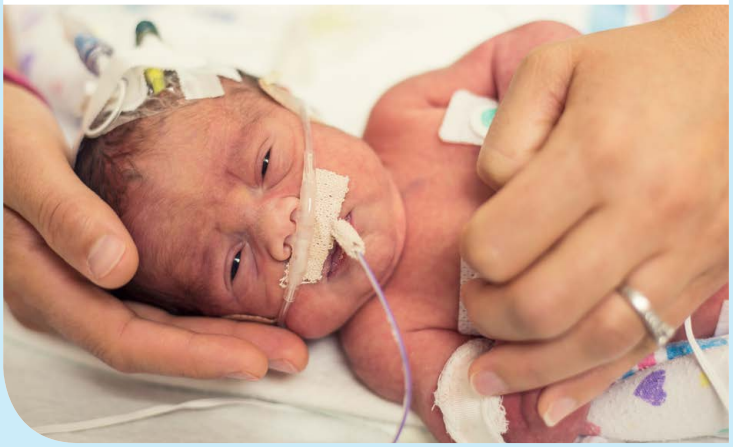
New research to tackle premature birth

Premature birth and its complications are a leading cause of death and disability in babies and children across the world. The reasons why some women go into labour early are still not fully understood but medical research is making progress. In 2017 Action, working in partnership with the charity Borne, funded two important new pieces of work looking at the role infection can play.

Women who experience very early preterm births often have a mild vaginal infection. However, many mums-to-be with similar infections go on to have uncomplicated pregnancies. This suggests that the way in which a woman's body fights infection is significant.

At King's College London, Dr Rachel Tribe and her team hope to find a specific biological 'finger-print' or biomarker to identify who is at most risk of early delivery. They aim to develop a screening test that can be carried out in early pregnancy. Those who are found to be at high risk could then receive appropriate care to protect their babies from being born too soon.

> "Our hope is it could reduce the numbers of premature births, as well as reduce the risk of brain damage and its impact on children's lives"
>
> Professor Donald Peebles



At University College London, Professor Donald Peebles and his team are also looking at how infection can trigger premature birth – as well as damage the developing baby's brain. He is testing a potential new treatment that is designed to boost a woman's natural defences and help prevent bacteria from getting into the womb.

Steps Forward

Reducing the risk of stillbirth

Stillbirth is devastating, with long-lasting effects on bereaved parents. Tragically, around 9 babies are stillborn every day in the UK. Parents want to know why their baby has died, whether it might happen again if they try for another baby and what they can do to avoid further stillbirth.

Action, along with research partners Cure Kids and Sands, has supported a three-year study led by Professor Alexander Heazell looking specifically at mothers' sleep positions in late pregnancy in relation to the risk of stillbirth.

This work, completed in 2017, confirmed findings from earlier studies in New Zealand and Australia that, in the third trimester (after 28 weeks of pregnancy), pregnant women who go to sleep on their back are more likely to have a stillbirth.

Supported by NHS England, a public health campaign has now been launched advising women to go to sleep on their side in the third trimester of pregnancy. The 'Sleep on Side' campaign has received wide media coverage. It is estimated that if all pregnant women in the UK followed this advice it could save the lives of around 130 babies a year.

This new advice could save up to **100,000** babies a year internationally

Steps Forward

A test to predict risk of early labour

Research funded by Action in 2014 has made important steps towards developing a blood test that could be used in early pregnancy to identify women who are at high risk of going into labour too soon.

Research Training Fellow Dr Joanna Cook investigated the role of naturally occurring substances called microRNAs, which seem to be involved in controlling when a woman goes into labour. These can be detected in the blood and, importantly, their levels have been found to be different in women who go on to develop cervical weakness – a known cause of premature birth. If diagnosed early enough cervical weakness can be treated and pregnancy prolonged.

Dr Cook says: "In our clinic we often see women who have already had very premature babies, but didn't receive special monitoring in their first pregnancies because we had no way of knowing they were at risk."

These promising results will now be tested in a larger group of women. If successful it is hoped that a commercially available test would be ready in around five years.

> "We hope that this exciting finding will enable us to develop a new way to screen pregnant women so doctors can identify, and help, women who are at risk of going into labour too soon"
>
> Dr Joanna Cook

Crohn's disease

Looking for causes

Up to one third of people are under 21 years old when diagnosed with Crohn's disease and the number of children and young people affected is rising.

Crohn's disease primarily affects the gut, causing inflammation and in turn distressing symptoms such as diarrhoea, abdominal pain and tiredness. While there are treatments that can help, there is currently no cure and many children undergo surgery to remove damaged parts of the bowel within 10 years of diagnosis.

In 2017 Dr James Ashton, based at the University of Southampton and Southampton Children's Hospital, was awarded an Action Research Training Fellowship to investigate what triggers the disease in children and causes sudden flare-ups.

"Children with Crohn's tend to have more severe symptoms than adults and it can have a significant impact on their lives – their growth, schooling and psychological wellbeing can all be affected," explains Dr Ashton.

"A lack of understanding of what causes the disease is hindering efforts to develop better treatments and to use those we have effectively," he adds.

Working with patients, Dr Ashton is studying the interaction between three different factors over time – bacteria living in the gut, the children's genetic make-up and the activity of their immune systems.

Crohn's disease affects at least **115,000** people in the UK

> "We hope that our findings will help develop better ways to predict disease severity, as well as new and improved treatments"
>
> Dr James Ashton

Cystic fibrosis

Fighting life-threatening lung infections

Action funding is helping researchers develop new ways to tackle hard-to-treat infections in children with cystic fibrosis.

Cystic fibrosis is the UK's most common life-threatening inherited disease. Thanks to medical progress already made, life expectancy is improving. But sadly the condition still claims the lives of two people every week in the UK. This is often due to progressive damage to their lungs, caused by bacterial infections that are hard to treat.

Children with cystic fibrosis experience a range of symptoms, including a build-up of sticky mucus in their lungs and airways which makes them very susceptible to chest infections.

Infections caused by *Pseudomonas aeruginosa* are a major threat and are the focus of research being led by Dr Tanmay Bharat at the University of Oxford, funded in partnership with the Cystic Fibrosis Trust.

These bacteria are notoriously difficult to defeat – they form a biofilm that coats the lining of the airways, acting as a barrier to stop antibiotics and the body's own immune cells from destroying them. The World Health Organisation lists it as the highest priority for the development of new antibiotic treatments.

"If we could identify new ways to wipe out these bacteria, it would help children with cystic fibrosis live longer, better lives"

Dr Tanmay Bharat

Steps Forward

A new treatment for cystic fibrosis

Thanks to Action funding awarded in 2014, in partnership with the Cystic Fibrosis Trust, researchers have moved closer to developing a new type of treatment to protect the lungs of children with cystic fibrosis. The inhaled medicine aims to target an underlying cause of symptoms rather than just alleviating the symptoms themselves.

The surface of the lungs becomes dehydrated in children with cystic fibrosis and researchers, led by Professor Steve Hart at the UCL Great Ormond Street Institute of Child Health, believe this is a major cause of symptoms – causing the production of thick, sticky mucus.

"Our new medicine is designed to combat this," says Professor Hart. "By keeping the surfaces moist, we hope this will reduce the thickness of the mucus, leading to fewer chest infections, improved breathing and reduced coughing."

But a big challenge is getting the new medicine to penetrate through existing mucus and into the lung cells where it can get to work.

"During this study, we developed a formulation that can deliver the medicine to where it's needed," says Professor Hart.

"We're excited about the progress we're making with this new medicine, which we hope will one day dramatically improve the lives of children with cystic fibrosis."

The team estimates trials in patients could happen within three years.

More than **4,000** UK children have cystic fibrosis

Rare diseases

Improving surgery for children with craniosynostosis

When Monty was just over a year old surgeons painstakingly reconstructed the front of his skull. With Action funding specialists are testing a new treatment approach which could in future improve surgery for children like Monty.

Three-year old Monty is full of energy and always has a smile on his face. "You wouldn't know he'd had major head surgery if you looked at him now," says his mum Lucy. But Monty was born with craniosynostosis, a rare condition that causes the bones in the skull to fuse together too soon.

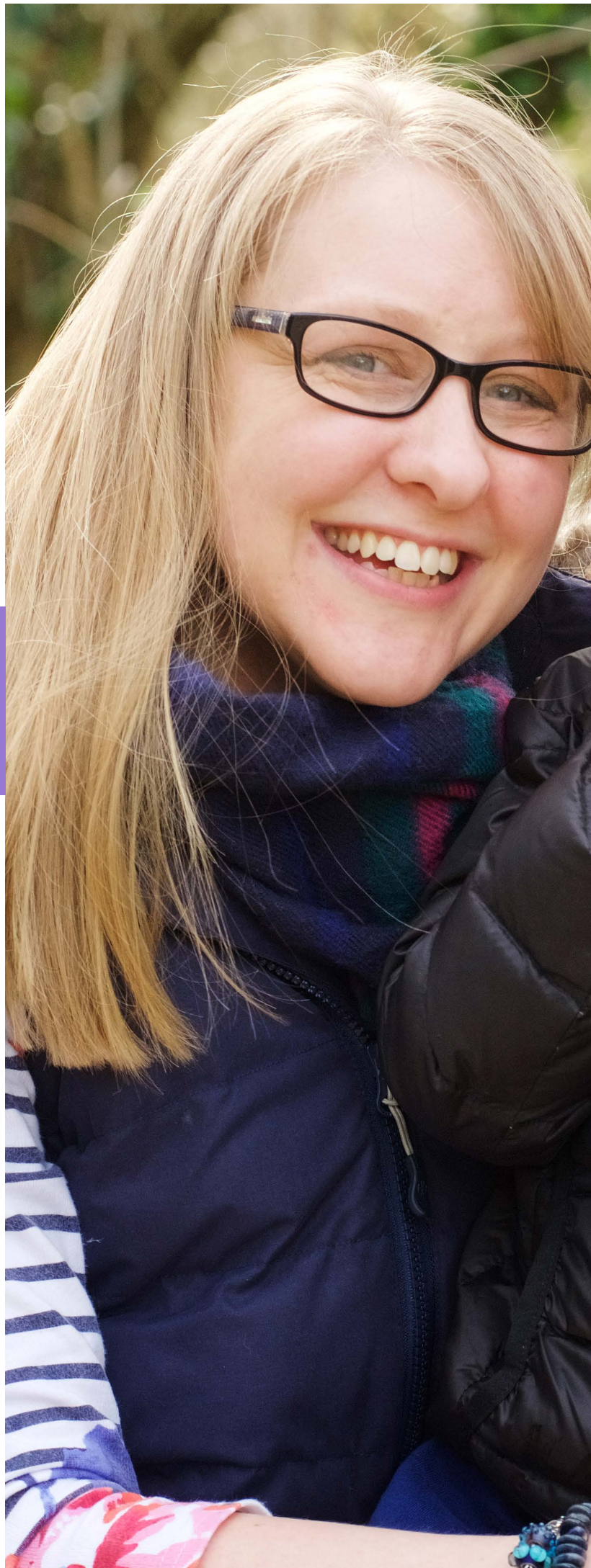
Around 350 children are born with craniosynostosis every year in the UK

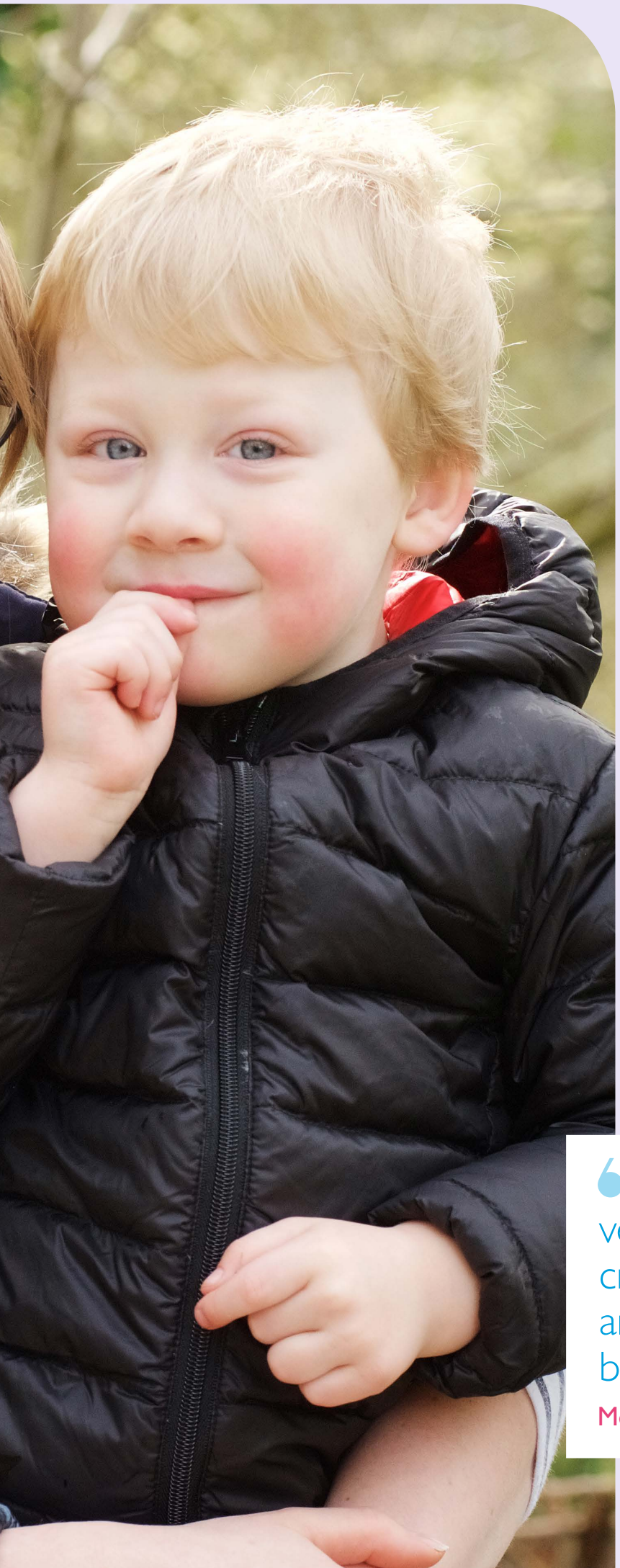
It affects the shape of the head, restricting room for the brain to grow, and can lead to pressure within the skull. This can affect development, cause problems with vision and hearing, and give children persistent headaches.

Monty was born with the two plates at the front of his skull already fused, creating a very triangular shaped forehead. When he was just four months old Lucy and husband Kris were advised that he should have surgery to correct this – a daunting prospect for any parent. The optimal time for this, they were told, would be just after his first birthday.

"The specialists were very clear that Monty's craniosynostosis was severe and it was not natural for the brain to be in that shape," recalls Lucy. "So it could impact on his long-term development. Nobody would wish that level of surgery on their child but we had to balance up the benefits and long-term implications."

When the day of the operation came, Monty was in theatre for almost eight hours while surgeons removed the front half of his skull – keeping his brain covered in liquid – and reconstructed his forehead. It was a tough





time for the family. "But you have to put your faith and trust in these amazing professionals," says Lucy.

Thankfully Monty, who is now three, coped amazingly well and after the operation his development seemed to rocket, says Lucy. "Things like his speech and suddenly being able to walk seemed to develop enormously," she says. "It could have happened anyway but it seemed very coincidental."

Monty's head remained swollen for several months but the plastic surgeon did an amazing job with his scar. "Hopefully one day Monty will understand how lucky he is to have such an amazing NHS that gave him the support he needed," says Lucy.

Monty will continue to be monitored at least once a year until he is fully grown, as there remains a chance that further intervention may be needed.

With Action funding, researchers hope to spare very young children like Monty – some of whom have further medical issues in addition to craniosynostosis – from such complex and lengthy operations by making less invasive treatment options work better than they currently do.

Lead researcher Dr Dagan Jenkins, at the UCL Great Ormond Street Institute of Child Health, says: "Coping with the news that your baby has such a serious condition is difficult enough, so we really want to improve current treatments so that they are both more effective and reduce the impact on these young lives. This would allow affected babies to look forward to a more typical childhood."

> "The specialists were very clear that Monty's craniosynostosis was severe and it was not natural for the brain to be in that shape"
>
> Monty's mum, Lucy

Treating cystic kidney disease

Cystic kidney disease is a common symptom of a group of rare, inherited disorders known as ciliopathies. New treatments are desperately needed to stop or slow down damage.

Inherited ciliopathies are a group of often chronically disabling, sometimes life-threatening, conditions. Children can experience a variety of symptoms and most will develop small, fluid-filled sacs, called cysts, in their kidneys. As these grow larger, they stop the kidneys from working properly.

One such condition is autosomal recessive polycystic kidney disease – more than half of all children who survive the early stages of this disease eventually experience kidney failure by the time they're 15 to 20 years old.

At the root of all these conditions are problems in tiny hair-like structures on cells, called cilia. These behave like antennae, helping cells to communicate with each other. When things go wrong with cilia, because of faulty genes, problems arise.

With Action funding Professor Colin Johnson, at the University of Leeds, is leading the search for much-needed drugs that could help preserve kidney function.

The team will be testing existing medicines that are already used to treat other conditions, as well as other chemical compounds that could be developed into new medicines to improve the function of cilia. These could improve and lengthen the lives of children with ciliopathies, sparing them from the need for dialysis or kidney transplant.

Today around **5,000** UK patients are receiving either dialysis or transplant because of an inherited kidney disease

Steps Forward

Fighting neuroblastoma

Around 100 young children a year are diagnosed with a rare cancer called neuroblastoma. Sadly some, like Felix, don't survive. Research funded by Action has successfully tested new drug combinations aimed at making treatment more effective, to save more lives.

Felix was just four years old when a GP discovered a mass in the little boy's abdomen. His parents, Matt and Colleen, were advised to take him straight to the Royal Marsden hospital. Here he was diagnosed with high-risk, stage four neuroblastoma, a cancer that starts in the nerve cells and can spread rapidly. Most affected children are very young – less than five years old – and, sadly, high-risk neuroblastoma can be very difficult to treat.

Felix endured two years of gruelling treatment, including chemotherapy, radiotherapy, operations and a stem cell transplant. But throughout his illness he remained cheerful, unaware of the gravity of the disease. "Felix's fortitude gave us strength," says Matt. He lost his fight, aged only six.

In 2014 Action awarded funding to Professor Robert Mairs and his team at Glasgow's Institute of Cancer Sciences. The aim was to enhance a treatment known as targeted molecular radiotherapy. This approach sees radioactive drugs, which seek and destroy cancer cells, injected into the bloodstream.

The team investigated using additional drugs that are designed to make cancer cells even more susceptible to radiation-induced damage. Two of these were found to work well. "This holds promise as another component in the arsenal for the treatment of high-risk neuroblastoma," says Professor Mairs, who anticipates clinical trials in children with the disease will happen within two years.

Matt says: "Leukaemia was a major killer 20 years ago and, due to investment in research, the prognosis for many is now good. Our hope is that knowledge and research is now invested into neuroblastoma."

This research was funded together with Neuroblastoma UK.

> Seeing the word 'cancer' written on the hospital signs was a devastating indication of the journey to come. Our life completely changed that night

Felix's Dad, Matt

Achieving potential

Tackling learning difficulties

Children naturally develop at different rates but missed milestones can be an early sign of problems. Action-funded research aims to make it easier to spot developmental delay and learning difficulties so children can get help sooner.

Parents know their children best and often notice if their little one doesn't reach a developmental milestone as expected. Their child may seem late to walk or talk. Or they may notice differences in the way they play, learn, speak or move. But since all children develop differently, it can be hard to know if professional help is needed.

Questionnaires completed by parents are often used to spot children who might need extra help and Action funding is now supporting researchers, led by Dr Samantha Johnson at the University of Leicester, who are adapting an existing questionnaire, currently used to identify problems in children who were born prematurely, to make it suitable for use with all young children. This will then be made available free of charge online, so health professionals and researchers worldwide can use it with parents of all young children.

> "What happens in early childhood can affect a child's health and wellbeing throughout their whole life"
>
> Dr Samantha Johnson

Steps Forward

A new, drug-free way to treat ADHD

Attention Deficit Hyperactivity Disorder (ADHD) can seriously affect children's lives both at school and at home. Medication can help, but may have side effects – and, since these drugs only work while you're taking them, the benefits can only be short term.

Now, a new drug-free approach to treatment for ADHD is showing promising, and longer lasting, results.

With funding from Action, researchers at King's College London have tested a technique called neurofeedback. This harnesses children's natural enjoyment of play and the power of technology to stimulate regions of the brain responsible for self-control.

A fun, space-themed video game is connected to a brain scanner. As children activate the correct part of the brain, a space rocket soars as a reward. They learn, through playing the game, how to control their brain activity themselves. Children who took part saw improvements in their symptoms comparable to using medication – and the improvements were still evident almost a year later.

"Our study shows, for the first time, that this treatment is feasible, safe and leads to short and longer-term benefits for young people with ADHD," says lead researcher Professor Katya Rubia.

Further funding of £1.3 million has now been awarded from the Medical Research Council and this approach has great potential for children with ADHD.

Around **one in 40** children in the UK has ADHD

Congenital heart disease and brain development

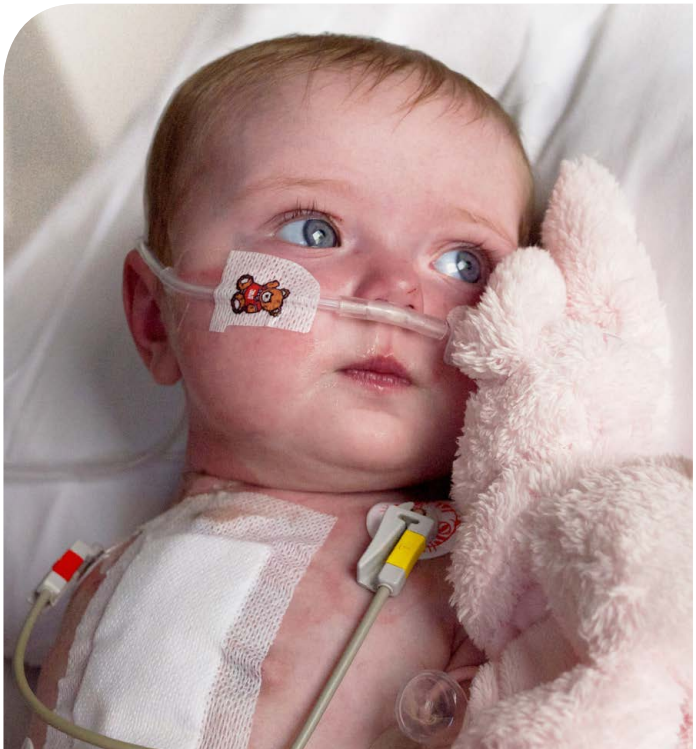
Up to half of all children born with a heart condition also experience neurodevelopmental issues. New research is looking at why.

Thanks to major medical advances over the last 50 years, the majority of children born with a heart condition now survive. But many experience problems with their development, which can affect things like movement, coordination, memory, hyperactivity, attention, and speech and language skills. These children also tend to do worse at school.

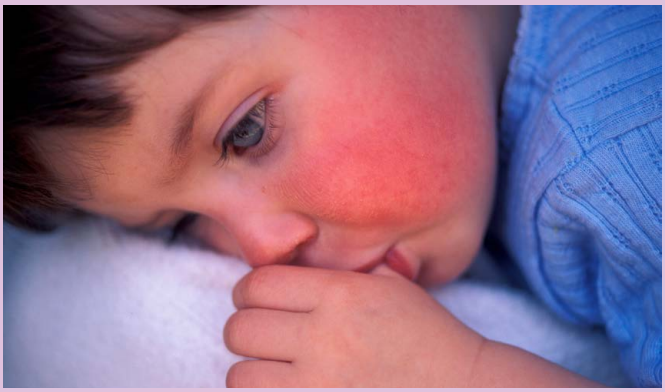
New research aims to find out why and hopes to pave the way for new treatment approaches to help affected children in the future.

Led by Professor Serena Counsell, a team of researchers has already collected detailed MRI brain scans of 80 newborn babies before they underwent heart surgery. With Action funding they will repeat these scans on the children, who are now around two years old. They will compare these images with those taken from a group of healthy children and also carry out tests to assess movement, learning and language skills.

Professor Counsell says: "We need to understand why so many of these children go on to experience difficulties that can have a major impact on their life chances. Our aim is to reduce the long-term effects of congenital heart disease on brain development, helping children to achieve their full potential."



Stopping the spread of scarlet fever



After decades of decline, scarlet fever infections hit a 50-year high in England in 2016, and the bacteria that cause it can trigger more dangerous illnesses.

Thanks to modern antibiotics, scarlet fever is not usually serious and symptoms, including a rash, sore throat and high temperature, generally clear up within a week. However, the strep A bacteria that cause the infection can have a darker side. In rare cases they can trigger life-threatening illnesses such as pneumonia, meningitis, toxic shock or sepsis.

Professor Shiranee Sriskandan, of Imperial College London, is leading an important programme of research that will guide future public health strategy on scarlet fever. This includes finding out which antibiotics are most effective and whether current hygiene and treatment guidelines for schools and nurseries are enough to limit the spread.

19,000+ children diagnosed with scarlet fever in England and Wales in 2016

"Given the current magnitude of scarlet fever outbreaks, it's really important that we find out how we can control it better," says Professor Sriskandan. "We aim to build our understanding of how scarlet fever infects children and spreads so we can identify the best ways to slow down transmission. We hope that this will, in turn, save children's lives from more dangerous conditions caused by the same bacteria."

Making an impact

The secret switch that's helping Finn pursue his football passion

Research funded by Action in the 1990s has played a key role in developing a technology used to treat dropped foot, a condition that makes walking difficult.

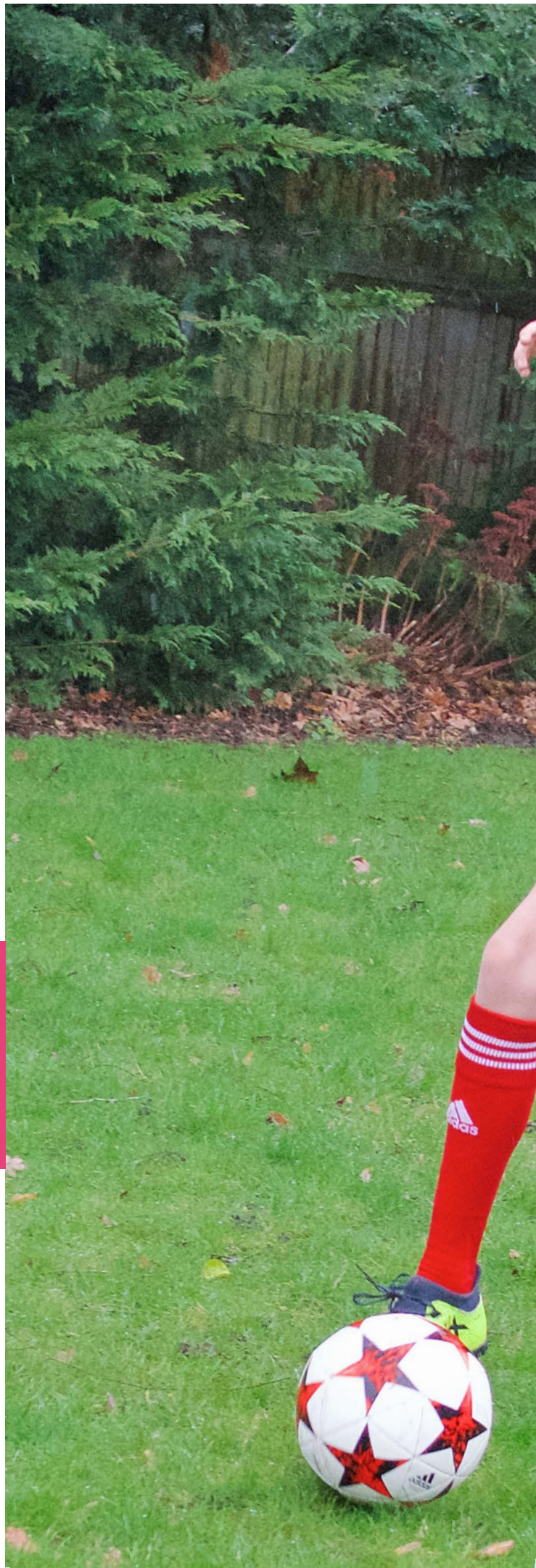
Children with dropped foot are unable to properly lift one or both feet. They struggle to move their ankle and toes upwards, causing the front part of the foot to drop down, or drag, making them prone to tripping and falling. Walking can be very difficult and tiring, affecting their confidence, independence and quality of life. It can be caused by cerebral palsy, multiple sclerosis, stroke and some inherited neurological diseases. It can also be caused by brain or spinal cord injury.

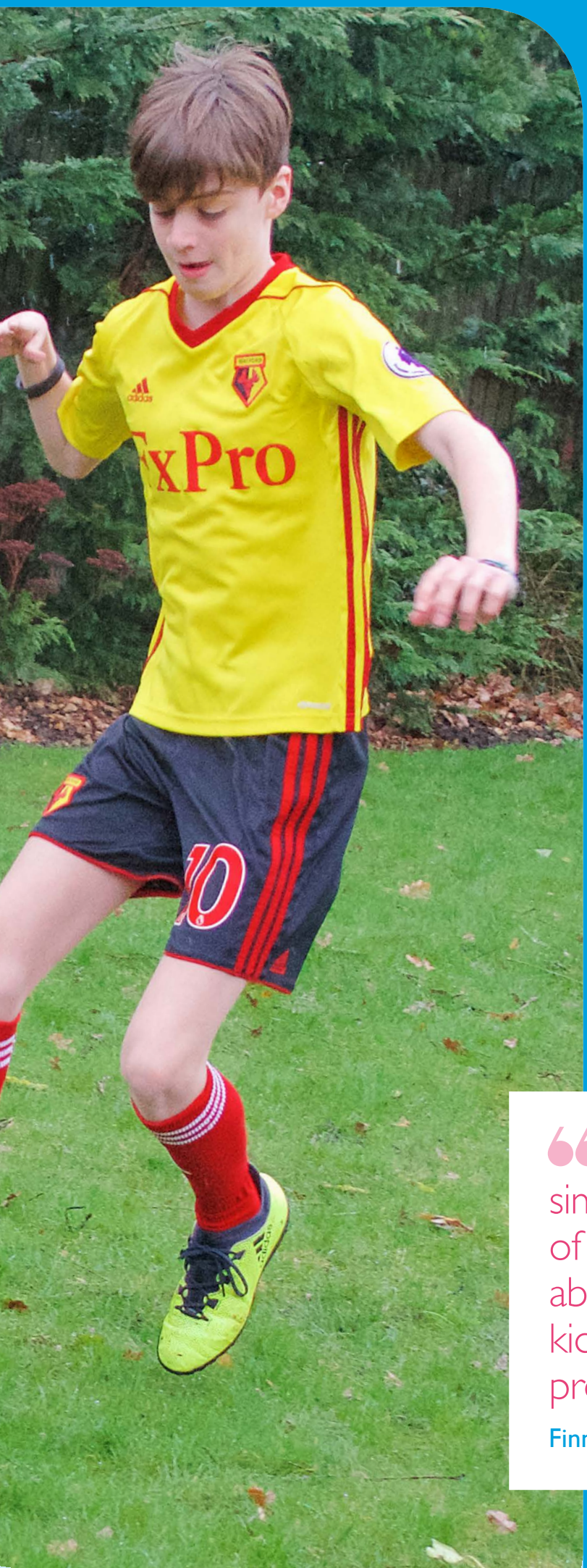
In the late 1990s Action funded three studies, investing £182,000 into work where researchers adapted, refined and tested a technology called functional electrical stimulation (FES). Led by Professor Ian Swain and his team, based at Salisbury District Hospital in Odstock and the Universities of Surrey and Southampton, their work created a small, simple device, called the Odstock Dropped Foot Stimulator. It can be worn and used in everyday life, as opposed to the bulky, complex equipment previously used in a medical setting, and improvements in walking have been found to be sustained even when the device is not in use.

The dropped foot stimulator has already helped around 15,000 people in the UK alone

FES works by using low-energy electrical pulses to mimic the natural signals that should be sent along nerves to the muscles, telling them when to contract. When these natural signals are disrupted, movement becomes difficult or impossible. FES uses specially timed electrical stimulation to do the same job and help lift the foot when walking.

Today, the team runs a dedicated children's FES clinic at the National Clinical FES Centre in Salisbury, and has treated children affected by cerebral palsy, stroke and head injury.





Finn's story

11-year-old Finn has cerebral palsy affecting the right side of his body – a condition known as right-side hemiplegia. When his condition was diagnosed, at six months old, his mum, Helen, threw herself into researching every possible source of help, which led the family to discover FES.

Finn finds it hard to lift his right foot when walking and his parents were so convinced that FES could help, they fought their local authority for funding. Finn now attends the children's clinic in Salisbury twice a year and he and his family have been delighted with the results. His dad, Anthony, says: "It has been fantastic. It makes a huge difference to his walking."

Explaining how the system works, Anthony says: "Finn wears a little box, about the size of two match boxes, attached to his belt, and wires down his legs. These attach to sticky pads on his shins. This system is wirelessly connected to a special switch inside his right shoe."

The pads deliver small electrical pulses to Finn's muscles. One pad lifts his toes and the other turns them outwards. The timing and strength of the pulses can be adapted according to how he's feeling, using the switch, and Finn doesn't wear the device all day long as this helps to allow muscle memory to develop. This means improvements in his physical skills may be sustained even when the device is not being used.

FES is helping Finn pursue his greatest passion in life: football. He plays for Chelsea Football Club under-12s team through the Chelsea Foundation's Disability Inclusion Programme. He also plays for the Football Association's regional talent centre in the south east, in recognition of his skill and potential as a football player with cerebral palsy.

> "Without this research, Finn simply wouldn't have the benefit of this technology. It's absolutely about giving opportunities to kids, to make a difference – a profound difference"
>
> **Finn's dad, Anthony**

Research grants awarded in 2017

Action Medical Research is funding over 65 projects, 17 of them awarded in 2017.

The next medical breakthrough could be on your doorstep.

Amblyopia (lazy eye) – safety and acceptability of a new treatment

Lead researcher:
Dr A Dahlmann-Noor
Moorfields Eye Hospital and UCL Institute of Ophthalmology, London

Cerebral palsy – measuring the benefits of physical activity [1]

Lead researcher:
Professor H Dawes
Oxford Brookes University, Oxford University Hospitals NHSFT

Congenital heart disease – effects on brain development

Lead researcher:
Professor S Counsell
King's College London and Guy's and St Thomas' NHS Foundation Trust, London

Congenital heart disease – improving surgical treatment

Lead researcher: **Dr E Sauvage**
UCL Institute of Cardiovascular Science and Great Ormond Street Hospital for Children, London, and Bristol Heart Institute, Bristol Royal Infirmary

Craniosynostosis – investigating drug treatment

Lead researcher: **Dr D Jenkins**
UCL Great Ormond Street Institute of Child Health, London, John Radcliffe Hospital, Oxford, University of Oxford and University College London

Crohn's disease – studying interactions between genes, the immune system and gut bacteria

Lead researcher: **Dr J Ashton**
Southampton General Hospital, University of Southampton and Southampton Children's Hospital

Cystic fibrosis – new treatment approaches for a common bacterial lung infection [2]

Lead researcher: **Dr T Bharat**
University of Oxford

Cystic kidney disease – finding new drug treatments

Lead researcher:
Professor C Johnson
St James' University Hospital, Leeds, University of Leeds and University of Newcastle

Developmental delay in young children – improving early identification

Lead researcher: **Dr S Johnson**
University of Leicester, University of Oxford, University of Warwick, University College London and University of Birmingham

[1] Jointly funded with the Chartered Society of Physiotherapy Charitable Trust
[2] Jointly funded with the Cystic Fibrosis Trust
[3] Jointly funded with The Chief Scientist Office, Scotland
[4] Jointly funded with Borne
[5] Funded by a generous donation from the Exilarch's Foundation
[6] Funded together with The Addenbrooke's Charitable Trust

Developmental language disorder – understanding genetic mechanisms [3]

Lead researcher: **Dr S Paracchini**
University of St Andrews, Scotland

Neural tube defects – prevention with inositol

Lead researcher:
Professor N Greene
UCL Great Ormond Street Institute of Child Health, London

Pelizaeus-Merzbacher disease – finding new treatments

Lead researcher:
Professor D Rowitch
University of Cambridge

Preterm birth – a new biomarker to identify women at risk [4]

Lead researcher: **Dr R Tribe**
St Thomas' Hospital and Institute of Pharmaceutical Science, King's College London

Preterm birth – preventing brain injury associated with being born too soon [4]

Lead researcher:
Professor D Peebles
University College London

Preterm children – identifying those at risk of anxiety problems [5]

Lead researcher: **Dr C Nosarti**
King's College London

Scarlet fever and streptococcal infections – stopping the spread of infection

Lead researcher:
Professor S Sriskandan
Imperial College London and Public Health England

Traumatic brain injury – monitoring to reduce complications [6]

Lead researcher: **Dr S Agrawal**
Addenbrooke's Hospital, Cambridge University Hospitals NHS Foundation Trust and University of Cambridge.

Summarised financial statements

for year ended 31 December 2017

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 25 April 2018. The full financial statements, on which the auditor, Buzzacott LLP, gave an unqualified audit report on 4 May 2018, was submitted to the relevant statutory bodies, including the registrar of companies, on 15 May 2018.

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 2017.

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 Martin Richardson, Vincent House, Horsham, West Sussex RH12 2DP.

Signed on behalf of the trustees

Phil Hodkinson
Chair

Income and expenditure	2017	2016
	£000s	£000s
Net incoming resources		
Donations and legacies	2,666	3,303
Investments	95	14
Trading	0	1
Total net incoming resources	2,761	3,318
Outgoing resources		
Medical research projects	3,013	3,124
Medical dissemination	758	801
Net outgoing resources	(1,010)	(607)
Net (losses) gain on investments	321	1,578
Net movement in funds	**(689)**	**971**

Balance sheet	31 Dec 17	31 Dec 16
	£000s	£000s
Fixed assets		
Tangible	351	364
Investments	13,549	14,712
Total fixed assets	13,900	15,076
Current assets	1,575	2,005
Current liabilities within one year	(5,474)	(6,591)
Liabilities falling due after one year	(2,740)	(2,541)
Total net assets	7,261	7,949
Representing:		
Unrestricted funds	7,248	6,979
Restricted funds	13	0
Total funds	**7,261**	**6,979**

Thank you

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

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

Thank you too to those who have made provision for Action in their will and to those, named here, who left us a legacy gift in 2017.

Supporters

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Gingerman Group
Highfield Foundation
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M&S Horsham
Mace Foundation
Maserati
Milton Damerel Trust
Next Retail
One Stop
Oso Foundation

Who's who 2017

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

President
Field Marshal The Lord Guthrie GCB LVO OBE DL

Vice Presidents
Patrick Brenan OBE FCA – deceased March 2017
Stephen May
Richard Price
The Duchess of Northumberland
The Earl of Snowdon GCVO RDI FSIAD
– deceased January 2017

Trustees
Phil Hodkinson, Chair
Charles Jackson, Honorary Treasurer
– retired July 2017
Luke Bordewich, Honorary Treasurer
– from July 2017
Esther Alderson
Professor Sarah Bray BA MPhil PhD FMedSci
Professor David Edwards MA MBBS DSc MRCR FRCP FRCPCH FMed Sci
Professor Nigel Klein BSc MBBS PhD MRCP FRCPCH
Rachel Molho – retired February 2017
Nick Peters
Val Remington-Hobbs
Richard Wild – appointed November 2017

Scientific Advisory Panel
Professor Nigel Klein BSc MBBS PhD MRCP FRCPCH, Chair
Professor Graeme Black OBE DPhil FRCOphth
Professor Clare Bryant BSc PhD BVetMed
Professor Inderjeet Dokal MBChB MD FRCP FRCPCH FRCPath FMedSci
Professor Catherine Hawrylowicz PhD
Professor Mark Johnson PhD MRCP MRCOG
Dr Samantha Johnson PhD CPsychol AFBPsS
– appointed July 2017
Professor Fenella Kirkham MB BChir MD FRCPCH
Professor Colin Michie MA FRCPCH FLS FRSPH FRSS
– retired June 2017
Professor Scott Nelson PhD MRCOG
Professor David Rowitch MD PhD ScD
Dr Adam Shortland BSc PhD MIPEM CSci – appointed December 2017
Dr Julie Stebbins DPhil CSci SRCS
– retired October 2017
Professor Kate Storey FRSE
– retired January 2017
Professor Michael Taggart BSc PhD – appointed July 2017
Professor Dieter Wolke PhD DipIPsych CPsychol AFBPsS
– retired June 2017

Paddington at Paddington Station
PureGym
Quai Administration Services
Rouleur
Rusk & Rusk
Skerritts Consultants
St Edmundsbury Financial Services
Team Sky
Willmott Dixon

Gifts in wills

Margaret Adderley
Sydney Atkinson
Jennifer Baker
Alexander Barlow
Barbara Bevis
Dora Bloomer
Heather Botwood
Dorothy Bradbury
Imeon Bradshaw
Mollie Corbett
Lena Crockett
Nancy Dean
Herbert Gill
Eileen Goulstone
Gordon Hutton
Margaret Hutton
Kathleen Ling
Ruth Pearson
Vera Reynolds
Enid Rice
Waveney West

If you would like to find out more about leaving a gift in your will, please contact Sharon Gearing on 01403 327413, by email at **legacy@action.org.uk** or visit **action.org.uk/giw**

65 years of success

Action Medical Research has supported some of the most significant advances in modern medicine – saving thousands of children's lives and changing many more. Some of the breakthroughs we've helped fund include...

1950s

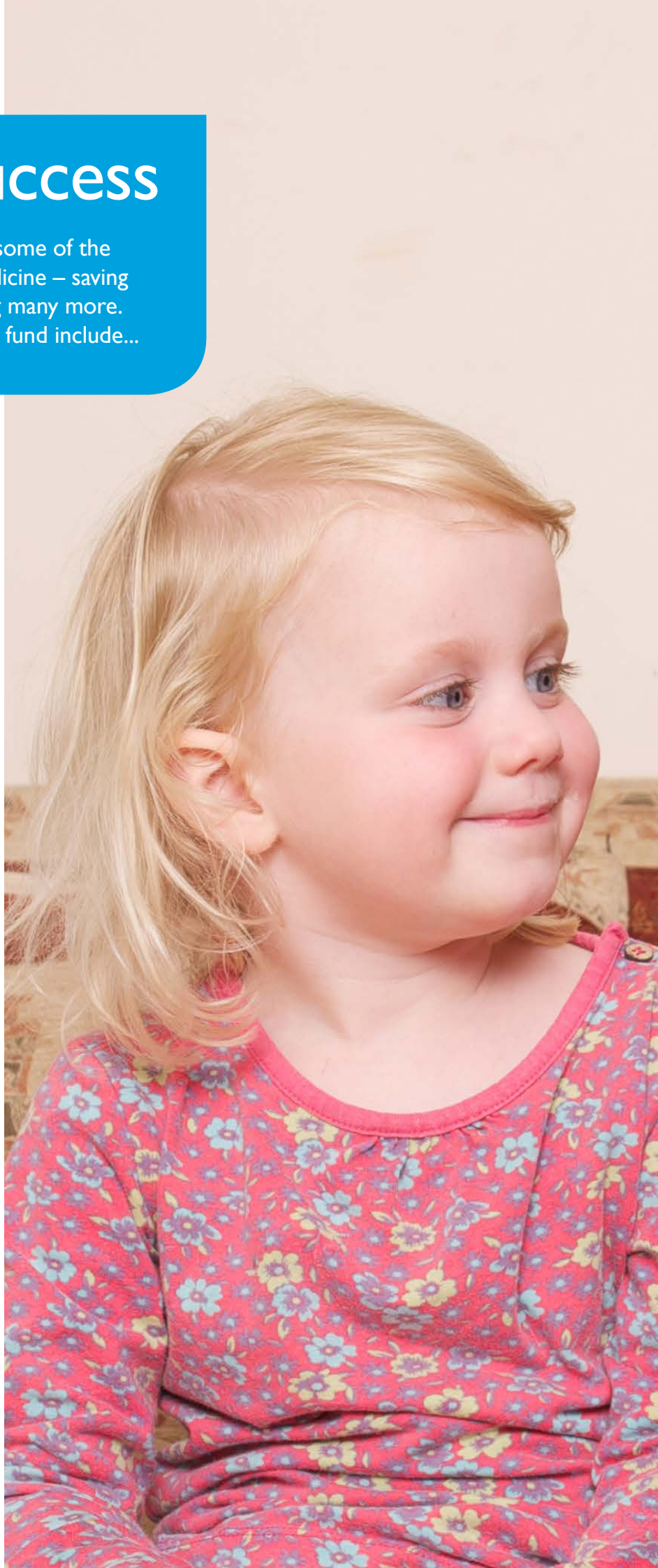
Helping to develop the first UK polio vaccines, keeping millions of children safe from this deadly virus

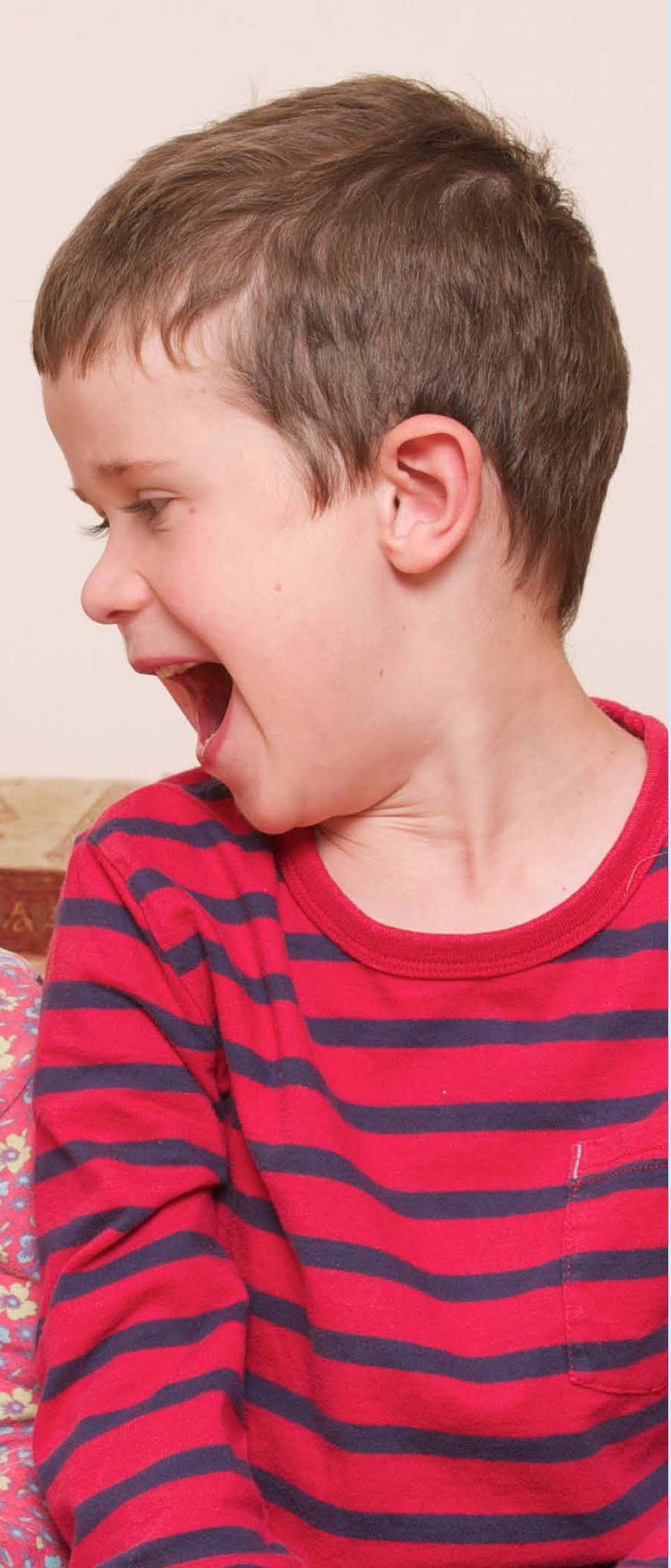
1960s

Discovering the importance of taking folic acid before and during pregnancy to prevent spina bifida

1970s

Helping to develop ultrasound scanning in pregnancy





1980s

Developing the award-winning Matrix seating system for children with severe disabilities

1990s

Supporting the researcher who led development of a vaccine for Hib meningitis, now a routine immunisation

2000s

Helping develop cooling therapy to prevent brain damage in babies deprived of oxygen at or around birth

2010s

Identifying genes which cause the life-threatening rare condition Jeune syndrome

Thank you

With your support we continue to fund life-changing research for some of the UK's sickest babies and children

Action Medical Research
Vincent House
Horsham
West Sussex
RH12 2DP

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