action medical research for children

Research Review 2016

Saving and changing children's lives

Action Medical Research at a glance

Celebrating
65
years of saving and changing lives

20
new projects funded in 2016

Funding vital research to help babies and children

£240
can fund a pioneering research project for a day

More than
75
projects across the UK

Supporting more than
260
top researchers

Welcome

Just like our founder who was driven to eradicate polio in the UK, today at Action Medical Research we are determined to help children like Jessica (below) who had open heart surgery within hours of her birth.

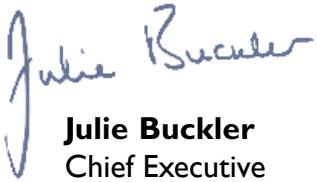
For an incredible 65 years Action has been funding extraordinary medical breakthroughs. In 2016 we funded 20 new research projects, identified through our world-class peer review system, to help babies, children and young people.

We celebrated the impact made by our funding of UK researchers who devised new tests and guidelines that are now used all over the world to identify and support children and teenagers with developmental coordination disorder.

We continued to work in partnership with other charities and launched a new relationship with Borne. Together we plan to spend £1.5m over the next three years to help prevent death and disability resulting from pregnancy complications and premature birth.

Our 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 and to those people who thoughtfully remember Action with gifts in their wills. We greatly appreciate support from many charitable trusts and especially the Garfield Weston Foundation.

Today in the UK the lives of so many children are devastated by disease and disability. Please help us fund more research that can save and change lives.



Julie Buckler
Chief Executive
Action Medical Research

Photo: Ben Rector

Cleft lip and palate

Helping children to speak more clearly

Even after surgery, many children with a cleft lip or palate have ongoing problems with their speech. New research aims to help improve the diagnosis and treatment of these difficulties.

Discovering that their son Robbie had a cleft palate was a shock for Geraldine and husband Stephen. Pregnancy scans had shown no issues but when their baby boy arrived he had breathing problems and struggled to feed.

Around one in 700 babies in the UK is born with a cleft lip and/or palate, which means they have a gap or split in their upper lip and/or the roof of their mouth.

For Robbie, his cleft palate was part of a condition called Pierre Robin Sequence, meaning he also had a small, receding lower jaw. This leads to the tongue being more likely to fall backwards and obstruct the airways.

Robbie's lips were not affected but the gap in his soft palate meant he could only feed using a bottle with a special teat. This was still very tiring for him and he spent three weeks in special care struggling to regain his birth weight.

Back home, the risk of breathing difficulties meant that Robbie could not be laid down on his back – even for nappy changes. "It was a terrifying and really upsetting time," recalls Geraldine.

On average **three** babies are born with a cleft lip and/or palate every day in the UK

Robbie had his first operation on his palate at just three months old, followed by more surgery when he was three. He then joined a long waiting list for specialist speech and language therapy, with much of the work being carried out at home with his parents.

Unfortunately, even with weekly therapy, Robbie's speech did not progress as well as hoped and at 10 years old he needed a major operation on his throat. Thankfully the surgery went well but, even now, his speech can still be difficult to understand.

Like Robbie, many children born with a cleft palate face ongoing problems with their speech.

Researchers at the University of Strathclyde are investigating whether ultrasound scans can help when diagnosing and treating these speech problems. The technique involves placing a small ultrasound scanner under the child's chin and a camera in front of their lips. This enables therapists, and children themselves, to see images on a computer screen that show how the tongue and lips move during speech. The team believes this could allow therapists to more accurately detect speech errors, including some that cannot be easily distinguished by ear. This will help ensure each child gets the best treatment.

Robbie has tried the painless, non-invasive scanner for himself and Geraldine says: "It makes it easier for the child to be assessed and gives a wealth of detail. I just think it's invaluable."

Photo: Adam Cochrane

"Older children ask, 'why do you talk funny?'. That's hard for a teenager to deal with."

Robbie's mum, Geraldine

ADHD

A drug-free treatment for ADHD

Attention deficit hyperactivity disorder, or ADHD, is one of the most common behavioural disorders in the UK and can have a serious impact on everyday life.

Children with ADHD tend to be hyperactive and impulsive. They have short attention spans and struggle with self-control and timing. This can affect how well they do at school and their relationships with other people, and difficulties often continue into adult life. They are also prone to problems such as depression and anxiety.

Medication is a treatment option but it doesn't always work and benefits are often only short-term. Many teenagers dislike taking it, there can be side effects and the longer-term effects of using these treatments are unknown.

With Action funding, researchers at King's College London are investigating the potential of a new treatment which combines playing a specially designed video game with electrical brain stimulation.

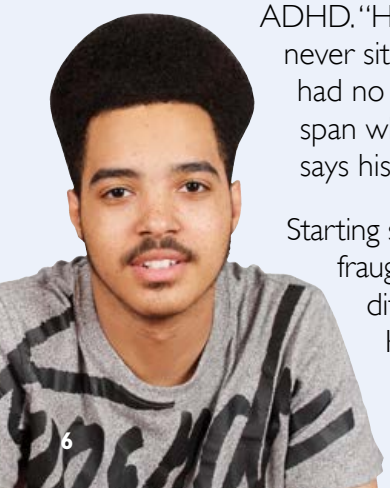
Lead researcher Professor Katya Rubia says: "Brain scans show that children with ADHD typically have poor activity in a region of the brain called the right frontal cortex. Our new approach is designed to stimulate activity in this region without using drugs. The ultimate goal is to give children with ADHD a new, drug-free treatment option."

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

Steps forward

Investigating premature birth and ADHD

Kirshon, now 18, was born three months early, weighing just 2lb 5ozs. He made quick progress in hospital but as a toddler he took a long time to talk, needing speech and language therapy, and started to show signs of ADHD. "He could never sit still and had no attention span whatsoever," says his dad Keith.



Starting school was fraught with difficulty for Kirshon. "It was a nightmare," says Keith. "They used to put him in the library because he disrupted the class."

After three months he was transferred to a school with stronger special needs provision, making the six-mile journey each day by taxi. Here, with the support he needed, he really thrived and was in a mainstream class by the time he left for senior school.

"His school reports used to make us cry," says Keith. "He was so kind to his fellow pupils, many of whom had needs far greater than his."

Premature babies are known to be at increased risk of developing ADHD and researchers funded by Action in 2012 have been studying this. Kirshon was among a group of teenagers taking part in this work.

Professor Jonna Kuntsi and her team found that those who were born early, even when well-functioning, showed impairments in several cognitive and brain processes. Some of these changes were similar to those seen in any person with ADHD but others were specific to being born prematurely. The researchers also found that other risk factors that run in families also have an effect.

By identifying these changes, the aim is for children's difficulties to be identified more quickly, so that families can get help sooner and with better targeted treatments.

Cystic fibrosis

Protecting against a much-feared infection

Action funding is helping researchers fight a common infection that can be devastating for children with cystic fibrosis.

Cystic fibrosis is a serious, inherited lung condition with no cure. It makes children very vulnerable to chest infections and around one third will develop a long-term lung infection with *Pseudomonas aeruginosa* bacteria by their late teens. In people with healthy lungs this is usually a harmless and short-lived infection, but for those with cystic fibrosis it's a major threat which can cause long-term damage and reduce life expectancy.

Alexander, now nine, was diagnosed with cystic fibrosis shortly after birth. There was no family history of the condition and his mum Gill's pregnancy had been problem-free. But doctors quickly became concerned for Alexander. His tummy was distended and every time he tried to take his first feed he was sick.

Within hours baby Alexander was separated from his mum, who was recovering from the difficult delivery, and transferred to Alder Hey Children's Hospital in Liverpool.

"I had a sleepless night waiting to hear news," recalls Gill. "Then a midwife came to tell me they had operated overnight to remove a blockage in his bowel and suspected he had cystic fibrosis. It was a complete shock and the start of a massive learning curve."

As well as affecting the lungs, cystic fibrosis also affects the digestive system and Alexander's bowel was severely affected. He needed a colostomy bag for the first four months of his life and endured four major operations during this time.

Alexander's family also had to learn how to care for their little boy out of hospital and began an ongoing daily routine of medication and physiotherapy – he needs at least 50 tablets a day just to keep well, if he's unwell it will be more.

Alexander first developed a *Pseudomonas* infection when he was just two. Thankfully he cleared the infection but he has remained on preventative antibiotic nebulisers twice a day ever since to try and keep further infections at bay.

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

Unfortunately, the infection has returned several times and when it does he takes stronger antibiotics for anything from a few weeks to a few months. The medicine makes him tired, affects his skin and upsets his stomach. When oral antibiotics don't work he has to spend time in hospital.

"The infection is something we never wanted him to get. It is worrying but we have to take each day as it comes," says Gill.

Dr Jo Fothergill at the University of Liverpool is investigating how *Pseudomonas* infections take hold in children with cystic fibrosis. She believes it may be possible to diagnose and start treating them at their earliest stages by taking regular nasal swabs.

Gill says: "New research means so much to families with cystic fibrosis."

"These infections are a major cause of ill health in children with cystic fibrosis. Families have described them as devastating"

Dr Jo Fothergill

Rare diseases

Fighting a fatal brain disease

Lily was just five years old when she was diagnosed with a devastating neurological condition known as BPAN. Research funded by Action aims to find answers and potential treatments for this cruel, life-shortening disease.

Lily is a little girl who loves to laugh and, to her parents' delight, has recently started to sing. "She sings in the car, it's her own special song and it's amazing," says her dad Simon.

Such innocent, happy moments mean the world to Simon and his wife Samantha. Sadly, they know they face a future of unimaginable heartbreak.

BPAN or, to give it its full name, Beta-propeller protein-associated neurodegeneration, is a rare, progressive and life-shortening condition. Little is known about its underlying cause and there are currently no drugs that can improve or cure it.

"When BPAN was first mentioned, we were totally devastated," Simon recalls. "We asked about our daughter's future and the doctors said they would be concerned for Lily in her second decade."

Lily, now eight, has severe global developmental delay. She uses a wheelchair and has very limited communication skills. BPAN also causes epilepsy and Lily has to take medications to control both this and dribbling. She also suffers from sleep problems.

"Lily can point to things she wants, use a little sign language and say a few words," says Simon. "Her life revolves around food. We have to lock the cupboards and the fridge. And she likes to carry a spoon around in her left hand at all times – the shinier the better!"

Tragically, teenagers and young people with this condition eventually develop abnormal muscle tone, and symptoms of Parkinson's disease and dementia. Lily's parents know her health will deteriorate: "We're giving her the best time we can while she's here," says Simon.

75%
of rare diseases affect children

Lily is taking part in an Action-funded study. Awarded one of the charity's prestigious Research Training Fellowships, Dr Apostolos Papandreou is investigating how genetic changes associated with BPAN cause brain cells to malfunction. He hopes to identify potential drug treatments that could reduce or reverse this.

Since the disease shares similarities with other neurodegenerative disorders, this work could also bring new insights into the treatment of conditions like Parkinson's and Alzheimer's.

Simon and Samantha know that a cure is unlikely in Lily's lifetime, but they passionately support this work. "Medical research like this for children is absolutely vital – you have to start somewhere," says Simon. "We know we won't get a cure for Lily but as parents we need to be bigger than that and look to the long term. Other children might benefit through Lily. We are so proud of her."

Photos: Adrian Sherratt

"It was clear that Lily had some sort of condition. But nothing could have prepared us for what was eventually diagnosed."

Lily's dad, Simon

Rare diseases

A condition that causes hearing loss and infertility

With Action funding, doctors are investigating the causes of Perrault syndrome, a rare, inherited condition. It is hoped this could lead to new treatments.

Some babies with Perrault syndrome are born deaf, while others have hearing loss that begins in early childhood and deteriorates over time. Such hearing loss can affect many areas of a child's life, including speech and language, education and social development.

For girls born with this condition there is also likely to be a serious impact on their future fertility. They may have missing ovaries or ovaries that stop working properly. Some never have periods, while others have an early menopause. As adults, they may be unable to have their own biological children or have difficulty getting pregnant.

Changes in several different genes have already been linked to Perrault syndrome but they don't explain all cases. Researchers at the University of Manchester aim to find more genetic changes that cause this condition and are investigating how these lead to symptoms. They hope this will lead them to a suitable target for future drug treatments.

As well as helping children with Perrault syndrome, the team believes that such treatments could also benefit people who have hearing loss or fertility problems for other reasons.

Steps forward

Finding answers for children like Eva

Five-year-old Eva is a happy, sociable and determined little girl. But behind her smile life is challenging. She has a rare neurological condition called ataxia.

The first sign that something was wrong came when Eva was nine months old and her parents noticed her hands shaking. She was then slow to crawl, stand and walk.

Eva was eventually diagnosed with ataxia when she was three. It affects her strength, balance and coordination, and she tires easily. Ataxia can be progressive, leading to severe disability. It can also shorten lives. Eva is currently making good progress but her future remains uncertain.

Action funding awarded in 2012 has led to improved genetic testing for ataxia and better understanding of the condition. This research, led by Professor Andrea Nemeth at the University of Oxford, is already benefiting families who've received a clear genetic diagnosis. In some cases it has also allowed doctors to give more information on what the future might hold and it could, one day, lead to new treatments.

"The work that's been done gives us hope that more funding will become available to help answer other questions, not just for Eva's sake but for other children too" Eva's mum, Carla

Epilepsy

A better night's sleep

Children with epilepsy may be especially susceptible to breathing problems at night. New research aims to improve diagnosis and prevent unnecessary suffering.

Children with a disorder called obstructive sleep apnoea stop breathing for a few seconds at a time during sleep. This causes oxygen levels in the blood to drop and disturbs their sleep – although children may wake only very briefly and be unaware of what's happened. This can happen many times a night.

Overall, it's thought that up to six per cent of all children have this problem, but preliminary evidence suggests that it could be affecting more than half of those with epilepsy. With Action funding Dr Don Urquhart and his team at Edinburgh's Royal Hospital for Sick Children aim to find out if this is true.

Around **60,000** UK children and teenagers under 18 have epilepsy

This sleep disorder is almost always treatable but if undiagnosed it can cause symptoms such as problems with learning and behaviour, and poor growth. Children with epilepsy often have learning problems and the researchers believe that if they also have undiagnosed sleep apnoea then this could be unnecessarily adding to their difficulties.

Steps forward

Better brain scans to help stop seizures

Medication doesn't work for up to one third of children and teenagers with epilepsy. The seizures these children experience can make day-to-day life very difficult, sometimes even dangerous.

Brain surgery can be a life-changing treatment option but is a major undertaking – it works by removing the part of the brain that is triggering seizures, so doctors need to be able to pinpoint where this is and the impact surgery might have.

Research funded by Action has allowed doctors successfully to test new brain scanning techniques to more easily identify children with drug-resistant epilepsy who could have surgery.

Dr David Carmichael and his team have combined existing scanning methods together to create more accurate images. They have been able to identify brain abnormalities where other scans had previously failed to find them. They also successfully tested a child-friendly approach, allowing children to wear headphones and watch cartoons inside the scanner. This helped to reduce movement, even in children as young as six.

The team are hoping their results will lead to a new clinical service within London's Great Ormond Street Hospital and to make this accessible to other hospitals treating children with epilepsy.

Helping sick babies

Protecting newborn babies from brain damage

Aiden endured a traumatic start to life and his survival was uncertain. Researchers funded by Action aim to help more babies who've suffered life-threatening brain damage at birth.

Complications at birth meant Aiden was deprived of oxygen for more than 20 minutes and needed prolonged resuscitation. With his life at serious risk, he was whisked away to intensive care and wrapped in an ice jacket to reduce his body temperature, a process known as cooling therapy.

"Aiden was cooled for 72 hours and without this vital treatment our little boy may not be here now," says his dad, Keith.

This breakthrough treatment is now routine in the UK and is the product of a 20-year programme of research to which Action Medical Research contributed more than £1m. By cooling the body to reduce brain temperature, doctors can alter the chemical processes that can cause brain damage and death.

But while cooling therapy can give babies like Aiden a much better chance, sadly it doesn't save them all. Neonatal encephalopathy, brain damage that is usually caused by a shortage of oxygen at birth, still kills around one in five affected babies in the UK and many others develop serious disabilities.

Each year around **700,000** babies worldwide die or face lifelong disability due to neonatal encephalopathy

Dr Ahad Rahim, of University College London, is investigating whether a commonly used diabetes medicine could help more babies like Aiden. The drug is thought to have protective effects on the brain and clinical trials are already underway to find out if it helps people with Alzheimer's and Parkinson's diseases.

If successful, it should be quicker to develop as a treatment for babies with neonatal encephalopathy rather than a totally new medicine. It could then be used either alone or with cooling therapy to help more babies who are at risk.

Aiden is now almost three years old and is a happy little boy who loves to play and be around people. He has cerebral palsy and cannot sit or walk unaided. But a combination of specialist therapies is helping him to get stronger.

"Aiden has the biggest, brightest smile and laugh. He can easily melt your heart," says his mum, Fleur. "By undergoing cooling we were given hope – hope that Aiden could be helped, hope that he would have a life to live. Research that could further advance treatment is so important to us."

"Without previous research Aiden's outcome could have been even more severe. More babies and their families deserve to have the hope that we were given."

Aiden's mum, Fleur

Helping sick babies

Protecting babies from a deadly bowel condition

Necrotising enterocolitis usually strikes the smallest and most vulnerable newborn babies. Researchers are investigating how different milk feeds affect the chances of developing this dangerous condition.

Necrotising enterocolitis happens when the lining of the intestines becomes inflamed and starts to die. It usually happens without warning, causing babies to become seriously unwell very quickly.

Premature babies, those with a very low birth weight and babies who are already poorly are the most susceptible to this life-threatening condition. It is the most common reason for emergency surgery in newborn babies and is, sadly, a significant cause of death and suffering in premature babies. Those who do survive can suffer long-term complications, such as persistent bowel problems, poor growth and learning difficulties.

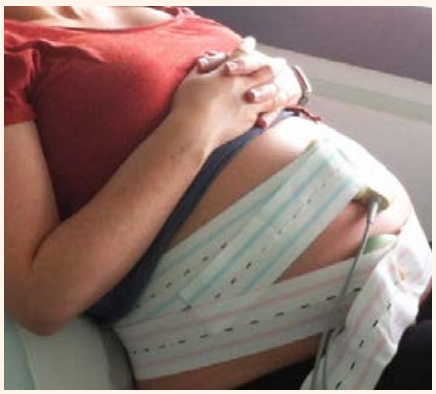
Action funding is supporting researchers based at the University of Oxford and University of Southampton. They are analysing data from a previous study, known as the ADEPT trial, which was also funded by Action and captured detailed feeding logs of more than 400 premature babies in their first few days of life. Their ultimate goal is to find out if changes in feeding practices could reduce the risk and save babies' lives.

Up to **1 in 20** babies in neonatal units develop necrotising enterocolitis

Steps forward

A new monitoring system for use in labour

Midwives and doctors often monitor babies' heartbeats during labour to check how well they are coping and identify those who may not be getting enough oxygen and need urgent help. The equipment used, introduced almost 50 years ago, produces a chart on a long paper strip which has to be assessed by eye. This can be unreliable – the pattern showing the baby's heartbeat can be complex and even experts may disagree as to what a chart suggests.



Action funding awarded in 2012 has supported work to develop a new computerised system for interpreting these charts. In 2016 the researchers reported back on their progress, which has seen them develop and test the first prototypes.

Based at the University of Oxford, the team has studied how babies' heartbeats change during labour, especially under stress, using high-level computer analyses of thousands of pre-existing charts and accompanying medical history. Their aim has been to identify the patterns that matter most for the baby and develop a system to recognise these and improve the decision-making process for doctors and midwives.

The prototype system is now being further refined and the team envisage it will be ready for testing in a clinical trial within three to five years.

Premature birth

Predicting the risk of early labour

A new test aims to identify women who face a higher risk of delivering their babies too soon.

Premature birth and its complications are a leading cause of death and disability in babies and children across the world. Here in the UK around 61,000 babies are born prematurely every year. Some, sadly, don't survive and very many more have an increased risk of developing lifelong disabilities.

The reasons why some women go into labour early are still not fully understood but medical research is making progress. With previous funding from Action a team of researchers discovered that women who lack white blood cells at the cervix (the opening to the womb) are more likely to give birth prematurely. White blood cells play an important role in protecting against infection.

Worldwide **15 million** babies are born prematurely every year

With further Action funding the team, led by Professor Nigel Klein, is now developing a new diagnostic test, suitable for widespread use early in pregnancy. The test would detect the presence of white blood cells so that those women who are found to be at higher risk could be monitored more closely and offered extra treatment to try and prolong their pregnancy.

"Preventing premature birth remains a major challenge to modern medicine"

Professor Nigel Klein

Early learning to help tiny tots

Babies who are born very prematurely are more likely to develop learning difficulties. Researchers are testing special play activities that could help them.

Each year in the UK around 9,000 babies are born before 32 weeks of pregnancy. While better care has greatly improved their chances of surviving, some of these babies, born before the brain is fully developed, go on to experience learning difficulties and special educational needs. Researchers at Anglia Ruskin University, Cambridge, are investigating whether taking part in special play activities, with their parents, around three months after their original due date can help.

The simple, quick and inexpensive activities have already been shown to have long-lasting benefits for babies born at full term. With Action funding, the team aims to see if they also work for the smallest, most vulnerable babies.

Cerebral palsy

Helping children with cerebral palsy

Children like Tom face lifelong difficulties with their movement and coordination and they are especially prone to hip damage. New research funded by Action aims to improve screening so that problems can be detected and treated sooner.

Tom is a bright and determined little boy but everyday life is very challenging for him. "In his imaginary play he's not disabled – he's a fireman or an astronaut," says his proud mum Maria.

But sadly Tom has quadriplegic cerebral palsy, caused by damage to his brain either before or during birth. He finds it extremely hard to control his arms and legs, has a lack of balance and suffers from muscle stiffness and weakness.

"We've been told it is unlikely that he will ever be able to walk unaided," says Maria. "He will need immense levels of care throughout his life."

Tom's condition was diagnosed before his first birthday and discovering that their baby son faced a lifetime of disability was heartbreaking for Maria and husband Terry.

Around **2,000** UK babies are diagnosed with cerebral palsy every year

Thankfully Tom can see, hear and talk but everyday activities such as eating and playing with toys are difficult. And at six years old, he has already endured muscle-release surgery on his legs and hips, because his muscles were pulling his joints out of place.

Problems affecting the hip joint are a key cause of disability, pain and reduced mobility for children with cerebral palsy. In the worst cases, some children's hips become fully dislocated.

With Action funding Dr Adam Shortland and his team, based at the One Small Step Gait Laboratory at Guy's Hospital, London, are developing a portable 3D ultrasound system for scanning the hip joints of children with cerebral palsy. It is hoped this could eventually replace the use of repeated x-rays for screening and monitoring these children, providing a safer and more accurate way of assessing their hip development. It may also make it easier to predict earlier which children are at greatest risk from hip damage, meaning they can be treated sooner.

Life can be difficult for Tom and his family but Maria says: "We adore him just exactly as he is and are determined that he should lead as normal a life as possible. Research that helps improve outcomes for children with cerebral palsy has got to be a really positive thing."

Photos: Ben Rector

"Tom's still a little boy and we want him to have a normal childhood and just have fun."

Tom's mum, Maria

Making an impact through research

The global tests now helping children with DCD

Developmental coordination disorder, also called dyspraxia, can make everyday activities and school work much more difficult. Action funding helped UK researchers to devise new tests and guidelines that are now used all over the world to identify and support affected children and teenagers.

Developmental coordination disorder (DCD) affects children's movement and physical coordination. Things like using cutlery, getting dressed, playing sports or riding a bike can all be a struggle and, for some, handwriting can be an especially difficult skill to master.

The condition is often not spotted until children start school at around age five – and it's estimated that up to one in 20 school-aged children in the UK is affected. The sooner the condition is identified, the earlier support can be given.

How we helped

Between 1999 and 2006 Action Medical Research funded DCD research worth a total of more than £217,000. At the University of Leeds, Professor David Sugden and Dr Mary Chambers focused on young children. They showed that simple interventions made at nursery, school and in the home could have a big impact, helping children develop basic skills and improve their coordination.

They developed a set of practical guidelines for parents and teachers, detailing simple steps to guide children through tailored activities. These are now used in the UK and across the world to identify young children with DCD and help them to learn new skills before their lives are adversely affected.

We also supported Professor Sheila Henderson and Professor Anna Barnett, at University College London and Oxford Brookes University, resulting in two further tests to help older children and teenagers.

This research saw a test already used for younger children successfully adapted to create the first suitable test for older children. Called the Movement Assessment Battery for Children-2 or MABC-2, it is now one of the most popular and respected tests worldwide for the assessment of motor skills.

The team also developed the Detailed Assessment of Speed of Handwriting (DASH). Children with DCD, and some other conditions, may struggle to write quickly or legibly enough to cope with the demands of school, putting them at a huge disadvantage, especially during exams. As Professor Henderson says: "The frustration of having the answers in your head but not being able to get them down on paper must be enormous."

This test enables teachers and therapists to better understand these problems and provide tailored support or special arrangements to help children perform as well as they can at school.

> "Without help from Action we would not have achieved a quarter of what we have done"
>
> Professor David Sugden

Liam's story

Liam is a determined and confident 14-year-old who dreams of running his own business. He's in his GCSE year at college and enjoying his studies but this hasn't always been the case.

Liam had been a bright, happy baby and toddler. "He spoke very well, had a vocabulary beyond his years and was very perceptive," says his mum, Lisa.

But problems arose when he started primary school. He suffered from exhaustion beyond the tiredness you would expect, struggled to learn to read and found writing almost physically impossible.

"It felt like he was bottom of the class, which didn't seem to tally with his actual intellect," says Lisa. "He would often end up cross or in tears. Nobody really understood what the problem was – or even whether or not there definitely was one."

It was only after an occupational therapist visited his school that Liam, then aged seven, was tested for DCD, using the MABC-2 – one of the tests developed with Action funding.

Having a diagnosis meant Liam could get the support he needed. He was set activities to improve his fine and gross motor skills and began to use a laptop to make up for his problems with writing.

He is now much happier, doing well and is able to manage his DCD. Lisa says they have accepted he will never be able to write properly but he has learnt to touch-type at speed.

There have been many occasions when people have failed to understand the impact of DCD. "One of the hardest things is that it isn't immediately obvious but it can make life really hard," says Lisa.

"Research is so important as it helps to increase understanding and awareness of the condition – helping children to live better lives."

Liam's mum, Lisa

Research grants awarded in 2016

Action Medical Research is funding over 75 projects, 20 of them awarded in 2016. **The next medical breakthrough could be on your doorstep.**

ADHD – treatment with brain stimulation *
Lead researcher:
Professor K Rubia
King's College London and University of Oxford

Birth asphyxia – finding medicines to protect the newborn brain
Lead researcher:
Dr A Rahim
University College London and St Thomas' Hospital, King's College London and UCL Institute for Women's Health

Cerebral palsy – monitoring hip development
Lead researcher:
Dr A Shortland
Guy's and St Thomas' NHS Foundation Trust, Evelina London Children's Hospital, St Thomas' Hospital and King's College London

Charcot-Marie-Tooth disease – an inherited neurological disorder
Lead researcher:
Professor D Bennett
University of Oxford

Cleft lip and palate – improving speech therapy **
Lead researcher:
Dr J Cleland
University of Strathclyde and Glasgow Dental Hospital

Craniosynostosis – analysing non-coding DNA and improving genetic diagnosis
Lead researcher:
Dr S Twigg
University of Oxford

Cystic fibrosis – understanding how a common bacterial infection persists in the lungs ***
Lead researcher:
Dr J Fothergill
University of Liverpool and Alder Hey Children's Hospital, Liverpool

Epilepsy and sleep-disordered breathing ****
Lead researcher:
Dr R Urquhart
Royal Hospital for Sick Children, Edinburgh and University of Edinburgh

Food allergy – does early introduction of food allergens reduce the risk?
Lead researcher:
Professor G Lack
King's College London, Guy's and St Thomas' NHS Foundation Trust, St Thomas' Hospital and Evelina London Children's Hospital

Inflammatory bowel disease in children – predicting disease severity
Lead researcher:
Dr M Zilbauer
University of Cambridge, Addenbrooke's Hospital, Cambridge and Cambridge University Hospitals

Necrotising enterocolitis – the importance of different feed types for premature babies
Lead researcher:
Associate Professor E Juszczak
University of Oxford, Southampton General Hospital and University of Southampton

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** Jointly funded with The Chief Scientist Office, Scotland
*** Funded with the Cystic Fibrosis Trust
**** Funded by a generous donation from The R S Macdonald Charitable Trust
***** Research Training Fellowship co-funded with the British Paediatric Neurology Association
****** Funded by a generous donation from the Exilarch's Foundation

Neurodegenerative disorders with brain iron accumulation – finding new treatments *****
Lead researcher:
Dr A Papandreou
University College London and UCL Great Ormond Street Institute of Child Health

Neurodevelopmental disorders – investigating common pathways
Lead researcher:
Dr H Jungbluth
King's College London

Neurofibromatosis type 2 – finding new treatments for nerve tumours
Lead researcher:
Dr S Ammoun
Plymouth University

Perrault syndrome – genetic basis of inherited hearing loss and infertility
Lead researcher:
Professor B Newman
University of Manchester

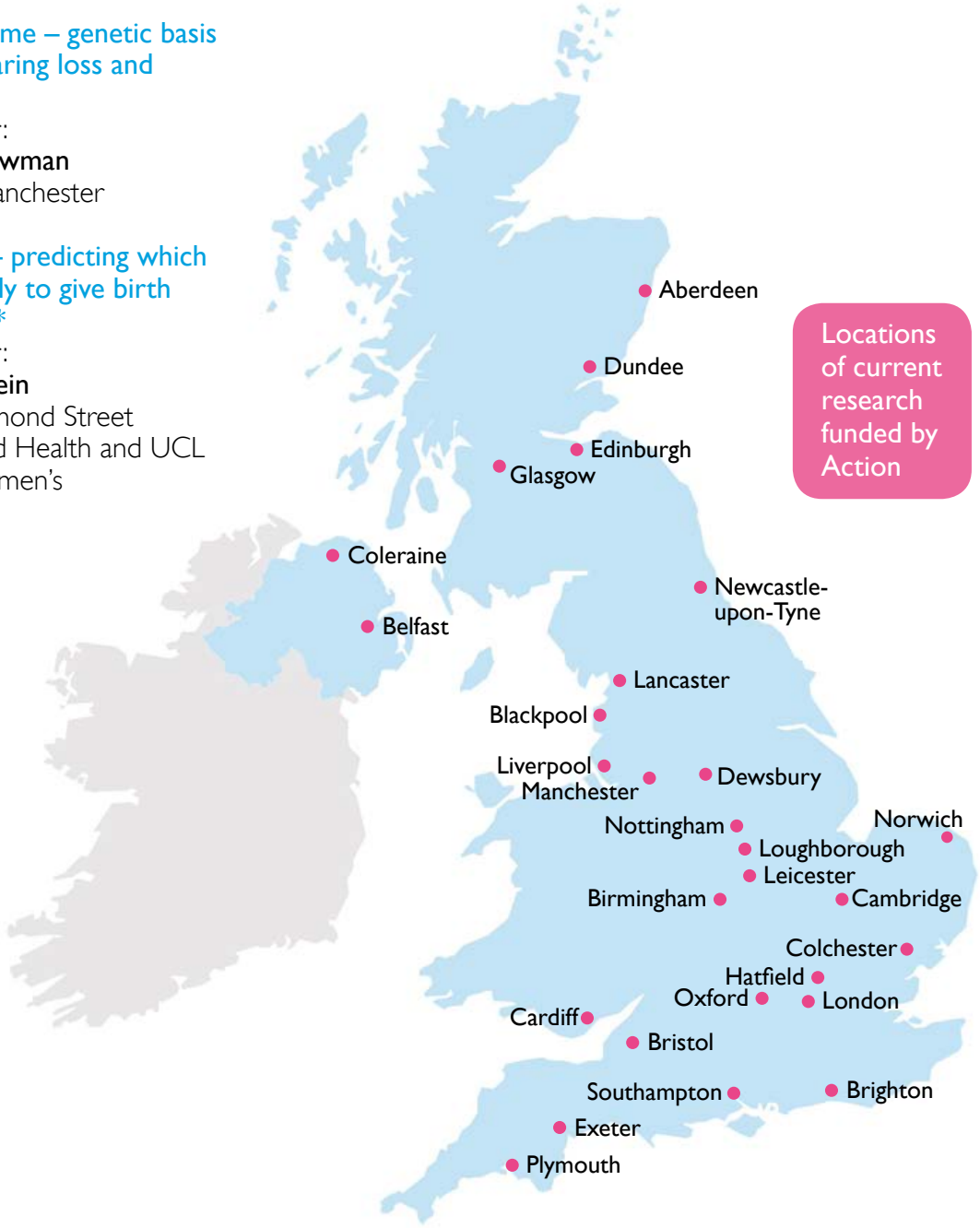
Preterm birth – predicting which women are likely to give birth too soon ******
Lead researcher:
Professor N Klein
UCL Great Ormond Street Institute of Child Health and UCL Institute for Women's Health

Preterm birth – special play activities to aid learning and development
Lead researcher:
Dr R Ford
Anglia Ruskin University, Cambridge, University of Leicester and Rosie Maternity Hospital, Cambridge

Sickle cell disease – treating abnormal breathing during sleep
Lead researcher:
Professor F Kirkham
UCL Great Ormond Street Institute of Child Health, North Middlesex Hospital NHS Trust, King's College Hospital NHS Trust, Imperial College, London and University Hospital Southampton

Vision in children born to mothers taking methadone in pregnancy **
Lead researcher:
Dr R Hamilton
Royal Hospital for Children and Princess Royal Maternity, NHS Greater Glasgow and Clyde, University of Glasgow and Princess Alexandra Eye Pavilion, NHS Lothian, Edinburgh

X-linked ichthyosis – an inherited skin disease
Lead researcher:
Professor E O'Toole
Queen Mary University of London



Summarised financial statements

for year ended 31 December 2016

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 2017. The full financial statements, on which the auditor, Buzzacott LLP, gave an unqualified audit report on 5 May 2017, was submitted to the relevant statutory bodies, including the registrar of companies, on 22 May 2017.

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 2016.

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	2016	2015
	£000s	£000s
Net income from:		
Donations and legacies	3,303	3,580
Investments	14	0
Trading	1	2
Total income	3,318	3,582
Expenditure on:		
Medical research projects	3,124	3,899
Medical dissemination	801	851
Net expenditure	(607)	(1,168)
Net gain/(losses) on investments	1,578	(179)
Net movement in funds	**971**	**(1,347)**

Balance sheet	31 Dec 16	31 Dec 15
	£000s	£000s
Fixed assets		
Tangible	364	385
Investments	14,712	13,620
Total fixed assets	15,076	14,005
Current assets	2,005	2,500
Current liabilities within one year	(6,591)	(6,508)
Liabilities falling due after one year	(2,541)	(3,018)
Total net assets	7,949	6,979
Representing:		
Unrestricted funds	7,839	6,979
Restricted funds	110	0
Total funds	**7,949**	**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 2016.

Supporters

AON Benfield
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BDO
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Exilarch's Foundation
Garmin
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Halliday Fraser Munro
Keepmoat
Liberty Specialty Markets
Mace Foundation
Maserati
Milton Damerel Trust
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One Stop

Who's who 2016

Patron
HRH The Prince Philip Duke of Edinburgh
KG KT OM GBE

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
The Earl of Strathmore and Kinghorne
– deceased February 2016

Trustees
Phil Hodkinson, Chair
Charles Jackson, Honorary Treasurer
Esther Alderson
Luke Bordewich
Professor Sarah Bray BA MPhil PhD FMedSci
Professor David Edwards MA MBBS DSc MRCR FRCP FRCPCH FMed Sci – appointed January 2016
Professor Mark Gardiner – retired July 2016
Caroline Hume-Kendall – retired November 2016
Professor Nigel Klein BSc MBBS PhD MRCP FRCPCH
– appointed January 2016
Rachel Molho – retired February 2017
Nick Peters
Val Remington-Hobbs

Scientific Advisory Panel
Professor Nigel Klein BSc MBBS PhD MRCP FRCPCH, Chair
– appointed Chair January 2016
Professor Graeme Black DPhil FRCOphth
Professor Clare Bryant BSc PhD BVetMed
– appointed December 2016
Professor Inderjeet Dokal MBChB, MD, FRCP, FRCPCH, FRCPath, FMedSci – appointed April 2016
Professor David Edwards MA MBBS DSc MRCR FRCP FRCPCH FMedSci – retired October 2016
Professor Catherine Hawrylowicz PhD
– appointed October 2016
Professor Mark Johnson PhD MRCP MRCOG
Professor Fenella Kirkham MB BChir MD FRCPCH
Professor Thomas MacDonald PhD FRCPath FMedSci
– retired June 2016
Professor Colin Michie MA FRCPCH FLS FRSPH FRSS
Professor Scott Nelson PhD MRCOG
– appointed January 2016
Professor David Rowitch MD PhD ScD
– appointed October 2016
Professor Kate Storey FRSE
– appointed March 2016
Dr Julie Stebbins DPhil CSci SRCS
Professor Dieter Wolke
PhD DipIPsych CPsychol AFBPsS

Photo: Ben Rector

Quai Administration Services

Rouleur

Skerritts Consultants

Smeg (UK) Ltd

Stewart Milne Group

Team Sky

Wilmott Dixon

Gifts in wills

Malcolm Arthurton
Eleanor (Jeanne) Beck
Kathleen Beer
Annie Burnett
Nancy Cooper
Mary Davies
Gwenneth Dishman
Elsie Edminson
Jean Fardoe
Nancy Findlay
Andrew Middlemiss
Denys Rothery
Jesse Runnells
Gordon Tiley
Christopher Wade

If you would like to find out more about leaving a gift in your will, please contact Jane Tarrant on 01403 327414, by email at **jtarrant@action.org.uk** or visit **action.org.uk/giw**

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Cover photo: Ben Rector