action medical research for children

Research Review 2015

Saving and changing children's lives

Action Medical Research in numbers

Founded in **1952** to tackle polio and help introduce the first polio vaccines

More than **£12 million** invested in current research projects

23 New projects funded in 2015

£290 funds a pioneering project for a day

Saving and changing children's lives for over **60** years

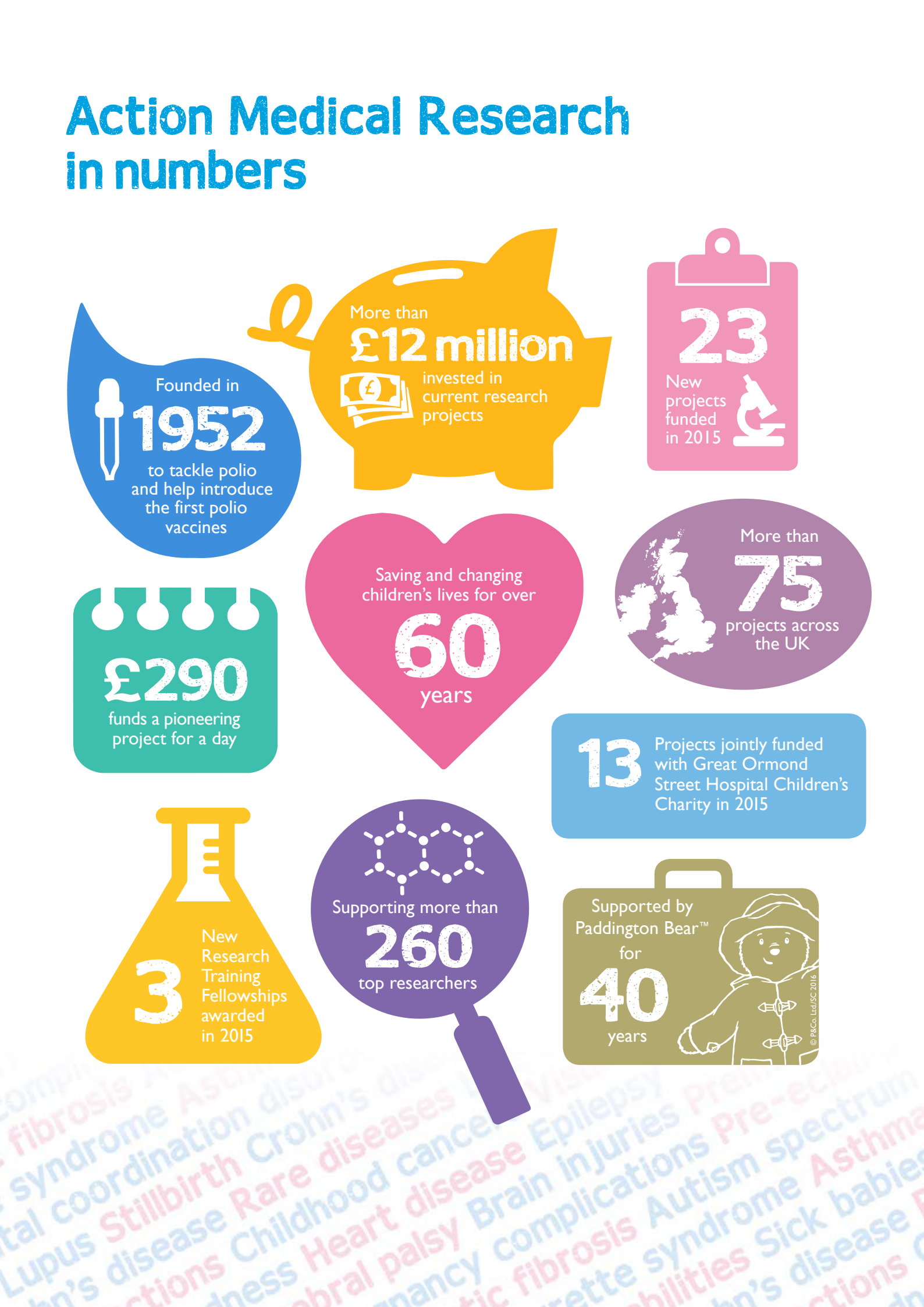
More than **75** projects across the UK

13 Projects jointly funded with Great Ormond Street Hospital Children's Charity in 2015

3 New Research Training Fellowships awarded in 2015

Supporting more than **260** top researchers

Supported by Paddington Bear™ for **40** years



Welcome

2015 was a truly great year at Action Medical Research. We were proud to invest over £3.5m to fund 23 new research projects to help babies, children and young people.

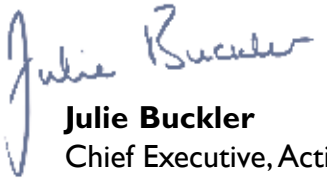
Since the charity began over 60 years ago we have been funding extraordinary medical breakthroughs, last year celebrating a new heart rate monitor, developed with our support, that shaves off critical seconds when newborn babies need resuscitating. Read more on page 18.

We were also delighted to partner with Great Ormond Street Hospital Children's Charity last year, together funding 13 new research projects across the UK identified through Action's world-class peer review system.

Today we remain determined to help children like Noah, pictured below. Noah has a rare brain condition that robbed him of his speech that our Research Training Fellow Dr Adeline Ngoh is tackling. You can find out more on page 14.

None of this great work would be possible without the support of many people, organisations and charitable trusts. Special thanks go to our network of local committees and volunteers who work so hard, as well as to the individuals who thoughtfully remember Action with gifts in their wills. We also greatly appreciate support from The Henry Smith Charity and the Garfield Weston Foundation.

Today all around the UK, there are hundreds of thousands of children whose lives are devastated by disease and disability. Please help us fund more research to help them.



Julie Buckler
Chief Executive, Action Medical Research

Photo: Ben Rector

Pre-eclampsia

Investigating the causes of pre-eclampsia

This condition can be so dangerous that early delivery, despite the risks, is sometimes essential for babies like Emily. New research funded by Action hopes to reveal why some women develop this life-threatening pregnancy complication.

At 14 months old, Emily is happy, lively and rules the roost at home. She is small for her age and people are amazed to see her already walking. For proud parents Tracy and Martin, every step is a little miracle. Emily was born six weeks early, weighing just 3lb 8oz, after Tracy developed pre-eclampsia.

"Everything had been going fine," recalls Tracy. "It seemed there was nothing to worry about." But a routine antenatal check suggested otherwise. Tracy had high blood pressure and protein was found in her urine. To her shock, she was admitted to hospital straight away.

Doctors could not stabilise Tracy's blood pressure and scans suggested that baby Emily was at serious risk. So, with an entire paediatric team in the operating theatre, Emily was born by caesarean section at 34 weeks.

Unable to hold her baby for the first 24 hours, the first cuddle is one Tracy will never forget. "I couldn't believe how small she was. You see premature babies on the TV but you don't realise how tiny they are until you hold your own," she says.

An estimated **1,000** babies die each year because of pre-eclampsia

Thankfully, after a month in hospital Tracy and Martin were allowed to take their little girl home. But, sadly, pre-eclampsia is a leading cause of death and illness in both babies and mothers worldwide.

With Action funding, Dr Christoph Lees and his team, based in London, are investigating why some women develop the condition. Recent evidence suggests that the way in which a mother's heart changes during the very early stages of pregnancy could be significant.

The research team will track hundreds of women before, during and after pregnancy to see how their heart function and circulation changes. They aim to establish whether or not any differences early on in pregnancy are associated with developing pre-eclampsia later.

This research could lead to earlier diagnosis and closer monitoring for women who may be at increased risk. It could also lead to the development of new treatments.

"We've been so lucky," concludes Tracy. "For me, pre-eclampsia came on suddenly and developed very quickly. It's only now that I realise how much danger Emily and I were in."

"I know research like this could potentially save lives. I was at real risk, and so was Emily."

Tracy, Emily's mum

Photos: Ben Rector

Premature birth

Helping children who are born too soon

Being born very prematurely puts children at greater risk of experiencing learning difficulties in school, especially in maths. New research will help teachers provide more tailored support in this key subject.

With one in every 50 UK babies being born before 32 weeks of pregnancy, almost all teachers will be responsible for supporting children who were born very early – but many lack awareness of how best to help.

Supported by Action funding, Dr Samantha Johnson is leading a research team which is developing a web-based training programme, showing teachers how best to support premature children in maths.

"We've found that teachers often have poor knowledge about the needs of premature children, with many feeling ill-equipped to support their learning, especially in maths," explains Dr Johnson, who is a specialist in how premature birth affects children's development.

Maths skills are vital for future life chances and are linked even more strongly than reading to employment prospects and earning potential. "Difficulties, even in primary school, can affect a child's prospects throughout their whole life," says Dr Johnson.

Around **16,000** babies are born before 32 weeks of pregnancy each year in the UK

The team is studying the skills of teenagers who were born very early. With previous Action funding, they studied the same children when they were aged eight to 10 years old. Now they are exploring how the children develop as they tackle increasingly complex work during their all-important secondary school years.

"We want to find out which areas of maths they are struggling with and why. Most importantly we hope to find out what types of support these young people need," says Dr Johnson.

"We hope to help all premature children achieve their full potential."

Dr Samantha Johnson

Delaying early labour, saving babies' lives

Premature babies are incredibly vulnerable. Sadly, some don't survive. A new treatment is being tested that could help prolong pregnancy in women at risk of giving birth too soon.

Premature birth is the biggest killer of babies in the UK. Tragically, around 1,200 babies die here each year after being born prematurely. Many who survive develop lifelong problems such as cerebral palsy, learning difficulties, blindness or hearing loss.

Treatment with a hormone called progesterone can reduce a woman's risk of giving birth early but it doesn't work for everyone. With Action funding, a London-based research team, led by Professor Mark Johnson at Imperial College London, is now investigating whether combining progesterone with another medicine works better.

Around **60,000** babies are born prematurely every year in the UK

This medicine, called aminophylline, has already been used widely in pregnant women to treat asthma and to help breathing in very premature babies.

The new treatment is being tested on a small group of pregnant women who are known to be at high risk of going into labour too early. If it seems successful, the team will go on to set up a much larger clinical trial in many more women.

"Our ultimate goal is to stop babies from being born too soon, save their lives and protect them from disability," says Professor Johnson.

Steps forward

Transforming blood tests for babies like Sophie

When she was born, two months early and suffering from a rare congenital condition, Sophie needed many blood tests to monitor her response to vital medicines. This meant pricking her heel and squeezing blood onto a special card – known as a heel-prick test.

"It wasn't nice but we had to get used to it," recalls her dad, Mark. "At first she was so sick she wasn't really aware of the pain but later she started to react more."

Action funding, awarded in 2012, has helped to successfully test a new, minimally invasive way to monitor drug levels in sick babies. This could be available for use in hospitals worldwide within five years.

Developed by Professor Ryan Donnelly and his team at Queen's University, Belfast, the new technique uses a patch, worn on the skin like a plaster. Its surface is covered in tiny 'microneedles', which puncture the outer layer of skin without causing bleeding or pain. These swell to allow skin fluid to be collected and analysed for more frequent and accurate monitoring.

This project was supported by a generous grant from The Henry Smith Charity.

Photo: Cai Graham Photography

Brain injury

Advanced scans to help children with head injuries

Serious head injuries can cause lasting damage and life-changing disabilities. New brain scans could give early, more accurate diagnosis to ensure the best possible care.

Head injuries are common in children, usually happening unexpectedly in falls or other accidents. They can be very dangerous if they damage the brain and seriously affected children can experience ongoing difficulties which have a major impact on their lives. These may not be obvious straight away and can include problems with memory, concentration and learning, or behavioural issues such as aggression.

Professor David Sharp, of Imperial College London, hopes to develop advanced brain scans that provide valuable information on the sort of damage a head injury has caused and how it might affect a child in the future. He hopes to reveal links between different patterns of injury and different problems children can experience.

Each year **35,000+** UK children are admitted to hospital with a traumatic brain injury

This research could help doctors to predict how an injury is likely to affect a child and identify what sort of treatment and educational support they might need.

Since most modern MRI scanners can perform these scans, this work could benefit children across the UK and beyond.

How brain injury has affected Kira

Kira was a bright, happy two-year-old when she was involved in a tragic accident, causing devastating injuries to her brain. These have affected all aspects of her life, leaving her with learning and physical disabilities.

"Kira is now 16, but in many ways she's still a little girl," says her mum, Erika. "Her speech is limited and she reads at the level of a six or seven-year-old."

Kira uses a wheelchair and needs help with everyday basics like eating and washing, so carers come to the house twice a day.

Erika is thankful that Kira has the same cheerful personality she had before her head injury. She is always positive and very popular. But she has no sense of danger, which makes her very vulnerable.

"The brain is so complex. There definitely needs to be more research into how different parts are used," says Erika. "More detailed brain scans have the potential to really help other children who have suffered a serious head injury."

"I would love to know more about what Kira is able to understand and whether she thinks ahead."

Erika, Kira's mum

Sight

Helping children to see more clearly

Children in special schools are more likely than others to struggle with their eyesight. This is often unrecognised, compounding their other disabilities. A new visual assessment programme aims to help.

More than 100,000 children and young people attend special schools in the UK. These children have a range of physical and learning disabilities, and evidence suggests that many are struggling with an untreated visual impairment. Failing to identify such children can add to the challenges they already face and unnecessarily limit them in reaching their full potential.

Supported by Action funding, a team based at Ulster University is testing a new visual assessment programme, delivered in special schools.

Estimates suggest up to **17%** of children in special schools may be visually impaired

Children with special needs can find unfamiliar environments stressful so it is hoped that being in a familiar setting will make it easier for them to cooperate with eye examinations.

The research team will also observe children in the classroom and use questionnaires and interviews with parents and teachers.

"If vision problems go unrecognised, people may wrongly attribute things like a lack of interest in educational materials to children's other difficulties, rather than poor eyesight," explains lead researcher Professor Kathryn Saunders.

"We hope to enhance everyone's understanding of the children's visual strengths and limitations, and improve both children's ability to see the world and their behaviour. Better vision and engagement in education could improve children's quality of life and independence, meaning they can reap the rewards for years to come."

Photo: Ben Rector

Steps forward

Better diagnosis to save children's sight

Children with brittle cornea syndrome, a rare genetic condition, have perilously fragile eyes. Common childhood accidents, such as being poked in the eye or hit by a ball, can be devastating – rupturing the eyeball and leaving them blind. Unfortunately, the condition is often only discovered once a child has already lost their sight in one eye.

A research grant awarded in 2011 to Professor Graeme Black and his team at St Mary's Hospital, Manchester, has led to new guidelines and genetic tests for diagnosing this condition.

Earlier diagnosis can save children's sight by highlighting the need to wear special glasses and protect their eyes.

Childhood cancers

Fighting high-risk neuroblastoma

Around 100 children, most of them under five, are diagnosed with a cancer called neuroblastoma each year in the UK. Two new projects aim to help those with the most life-threatening form of the disease.

Every year around **250** children die from cancer in the UK

While many children with neuroblastoma make a good recovery, some face a bleak outlook. Their cancer can be very hard to cure and despite enduring intensive treatment, usually lasting more than a year, many die.

In 2015 Action funded two research teams striving to develop more effective treatments for those children at greatest risk.

In London, Professor John Anderson and his team are developing a new therapy which involves modifying a child's own immune cells to combat their disease. The approach, known as immunotherapy, has already been successfully used to fight other types of cancer and could spare children from the unpleasant side effects of existing treatments.

Professor Anderson says: "Our ultimate aim is to design a safe new treatment that not only destroys a child's cancer but also prevents it returning, providing a cure for life."

Action funding is also supporting Professor Deborah Tweddle and her team, based at the Northern Institute for Cancer Research in Newcastle. They aim to find better ways to predict early on which children with neuroblastoma are at greatest risk of suffering a relapse after their cancer has initially been treated. This would allow doctors to adjust treatment recommendations sooner – they may even suggest trying an experimental treatment if one is available.

This team will study the medical records of hundreds of children with neuroblastoma who have suffered a relapse. They will also study genetic and clinical features of tumours and how these relate to the chances of relapse and how long a child survives if their cancer returns.

By looking for genetic changes, the team hopes in future to be able to target existing treatments more effectively and, if necessary, develop new drugs.

"We hope our approach will one day save the lives of more children with neuroblastoma."

Professor Deborah Tweddle

Better treatments for children with leukaemia

With Action funding, doctors aim to find new, more targeted drugs to treat the most common type of cancer to affect children.

Acute lymphoblastic leukaemia is an aggressive cancer that develops rapidly. Thanks to medical advances already made, most children have a high chance of being cured. Their treatment, however, will be hard and intensive, often lasting several years.

Children with this type of cancer will usually be given chemotherapy and steroids, but sometimes radiotherapy and a bone marrow transplant are also needed. They can suffer many unpleasant side effects and some face further problems later in life, such as restricted growth or infertility.

Doctors at University College London's Institute of Child Health are searching for new drug treatments that kill cancer cells while leaving normal cells unharmed, meaning the intensity of chemotherapy could be reduced.

Around **300** children develop acute lymphoblastic leukaemia each year in the UK

The team, led by Dr Owen Williams, are focusing on a particular type of acute lymphoblastic leukaemia, which affects a quarter of all children with the disease.

"Our ultimate goal is to spare children from some of the side effects of chemotherapy and protect more children from relapses," says Dr Williams.

Steps forward

Brain tumour research to help children like Jack

Jack was diagnosed with a brain tumour at 14 months old. Following a gruelling year of treatment, involving brain surgery, chemotherapy and radiotherapy, he thankfully recovered.

Pictured here aged five, Jack is now 11 and has been clear of cancer for almost nine years. However, having radiotherapy at such a very young age affected his working memory and he's needed extra support at school. His mum Lisa says: "He is doing amazingly well and only has an annual check-up now. But we met so many lovely families who weren't as lucky as us."

Action funding awarded in 2011 supported Research Training Fellow Dr Rebecca Hill in her bid to improve understanding and treatment of the type of brain tumour Jack had, called medulloblastoma. This is the most common cause of brain cancer in children and survival rates vary. For those children who later suffer a relapse in their cancer, it is nearly always fatal.

Dr Hill has made important discoveries around what happens biologically at the time of relapse. This has led to early tests of a new, targeted drug treatment, which is showing encouraging results.

Photo: Dirk Van Der Werff

Heart disease

Improving life-saving surgery for tiny hearts

At their 20-week scan, parents Louise and Michael discovered their unborn daughter had a rare, potentially fatal heart condition. Within hours of her birth, baby Jessica underwent open heart surgery.

The news that Jessica had hypoplastic left heart syndrome, a condition which affects one baby in 5,000, had come as a complete shock. "We were told that she was unlikely to survive," says Louise.

Amazingly, doctors carried out pioneering surgery on Jessica's heart while she was still in the womb. This was followed by her first open heart surgery shortly after she was born. "I had one quick cuddle and then they took her down to theatre," says Louise. "We were filled with euphoria that she'd arrived and was well, but also terrified."

Jessica's condition means the left side of her heart is much smaller than usual and cannot pump enough blood to the body. She had further surgery when she was one week old and again at 14 weeks and six months. Each time, her recovery was fraught with worry. She suffered wound infections and other complications, and needed many different medicines.

Today Jessica is a happy four-year-old, who loves drawing, dressing up and playing in the home corner at nursery. She takes medicine every day and tires more easily than other children, as well as turning blue if she gets cold. And in the next year or so, she will need further surgery to make the right side of her heart stronger.

With funding from Action, Dr Pablo Lamata at King's College London hopes to help babies with hypoplastic left heart syndrome. He is creating virtual 3D computer models of babies' hearts, using information from their own scans. The models will show in detail how each tiny heart is working. The aim is to help doctors decide on the best type of surgery for each baby.

"Research like this will give a lot of hope to a lot of families," says Louise.

"I have handed my child to a surgeon, knowing that they will stop my child's heart and prayed that I will see my child alive again."

Jessica's mum, Louise

Rare diseases

Tackling a brain condition that robs children of speech

Landau Kleffner syndrome left Noah unable to talk. New research funded by Action aims to give answers and hope to families like his.

Noah had been a happy baby and toddler, whose swift development delighted his parents, but at three years old he suddenly started having seizures. He was initially diagnosed with a childhood epilepsy disorder. But then he started to stutter and slur his words.

Noah needs to take **seven** different medicines every day

"Noah had an extensive vocabulary and spoke clearly. So the change was very noticeable," says his mum, Madeka. "It was so sudden and so sad." To his family's shock, within just two weeks Noah's ability to talk disappeared altogether.

Noah's nursery organised music therapy to help him express himself and the family learnt British Sign Language, which Noah picked up quickly.

But it was still not clear what was causing Noah's distressing symptoms.

Desperately worried, Madeka and husband Ryan opted for a private consultation. By now Noah was experiencing up to 40 seizures a day and was finally diagnosed with Landau Kleffner syndrome or LKS. His parents were told 'don't look it up because it will really scare you'.

The condition is very rare and robs children of the ability to speak and understand language. Unsurprisingly, children often lose their confidence and become frustrated or withdrawn. Many develop behavioural problems and autistic spectrum symptoms, and it can severely affect their social development and education.

Thankfully Noah, now five, is making good progress. His seizures have stopped and, following intensive speech and language therapy, he is able to talk again. But he has to take seven different medicines every day – including steroids, which can cause him to suffer extreme mood swings.

Dr Adeline Ngoh, of University College London's Institute of Child Health, has been awarded an Action Research Training Fellowship to investigate the genetic causes of this disabling condition.

She hopes her work will improve understanding of Landau Kleffner syndrome, give families answers to some of their many questions and, ultimately, lead to the development of better treatments.

For Noah and his family, more research is crucial. "It's known that steroids work but not why," says Madeka. "Research could eventually lead to treatments with fewer side effects. Because this is such a rare disease, and symptoms can be so sudden and extreme, it often gets diagnosed as something else. More awareness is vital, so doctors can spot it and treatment can begin sooner."

"When we were told Noah had Landau Kleffner syndrome, the doctor said 'don't look it up because it will really scare you' – so we didn't."

Noah's mum, Madeka

Photos: Ben Rector

Rare diseases

A step closer to developing a new cure

Building on earlier work funded by Action, researchers are moving ever closer to developing a much-needed new treatment for a rare, inherited condition that affects only boys.

X-linked lymphoproliferative disease, or XLP, impairs the body's immune system and boys typically become seriously ill during their childhood or early teens.

"The first sign that boys have the disease often comes when they suffer a severe bout of glandular fever, which can be so serious it becomes life-threatening," explains lead researcher Professor Bobby Gaspar. "Some of the boys develop a cancer called lymphoma and they can have difficulties fighting off other infections as well."

Without a successful bone marrow transplant, the disease is usually fatal. Families face a race-against-time hunt for a donor and, sadly, a compatible match cannot always be found. It can be a cruel lottery.

Professor Gaspar is a pioneer in gene therapy and a former recipient of an Action Research Training Fellowship early on in his career. He and his team at University College London's Institute of Child Health are developing a treatment that replaces the faulty gene that causes XLP with a healthy copy. This involves modifying cells from a child's own immune system. This latest work aims to perfect the techniques involved and test the safety and effectiveness of the treatment.

Without treatment around **70%** of boys with XLP die by age 10

"Results of earlier studies, also funded by Action Medical Research, have been promising and the work we're doing now will take us closer to the stage when clinical trials can begin in children, hopefully within the next two to three years," he says. "If we are successful, boys with XLP won't get infections anymore, they won't get lymphoma, they will be able to live normal lives."

"We are not trying to make these children a little bit better. We are trying to cure them."

Professor Bobby Gaspar

Autism and ADHD in babies with a rare disorder

Many children born with neurofibromatosis type 1 develop learning and behavioural difficulties as they get older. New tests could predict which babies will be affected, so they can get support sooner.

Neurofibromatosis type 1 affects one in 2,500 children and is caused by a faulty gene. It means that the growth of nerves is not properly controlled and this can lead to a number of problems typically affecting the skin.

Around half of babies born with this condition also develop attention deficit hyperactivity disorder, or ADHD, and at least a quarter will develop an autism spectrum condition. But too often, these symptoms are underdiagnosed or dismissed as just part of having neurofibromatosis. This means parents and children can struggle to get the support they need soon enough.

75% of rare diseases affect children

Funded by Action, a team based at Birkbeck, University of London is working with a group of affected families to develop new tests which they hope will predict which babies will go on to develop ADHD or an autism spectrum condition. This would give doctors and parents earlier warning, allowing for quicker diagnosis and, most importantly, treatment.

This could significantly improve the lives of affected children and their families. It would make it easier to plan future support as they grow up, both at home and in school. Ultimately, however, this work could also lead to new treatments to reduce symptoms, or prevent them from developing in the first place.

Steps forward

Genetic discoveries helping children like Joshua

Joshua was born with Jeune syndrome, a rare and incurable hereditary condition.

Babies born with the condition have short arms and legs and an unusually small ribcage, which can cause life-threatening breathing problems.

Joshua struggled from birth and was at one point given just three weeks to live. He has endured major surgery on his chest and still needs a ventilator to help him breathe. Despite this, he is a happy little boy who continues to amaze his family.

In 2011, Action awarded a Research Training Fellowship to talented young researcher Dr Miriam Schmidts to investigate the causes of Jeune syndrome. Her work identified six new genes which cause this devastating condition.

Families are already benefiting since certain genes are linked to certain symptoms, helping doctors to predict what problems a child may face and plan treatment accordingly. Improved genetic testing and counselling is also vital, since most people are unaware that they are carrying a faulty gene which causes the condition.

Photo: Ben Rector

Making an impact through research

The time-saving sensor that could save tiny lives

Every new parent listens out for that first cry when their baby is born, but some babies need extra help to take those first breaths. Action funding has helped to develop a new heart rate monitor to make resuscitation easier in these crucial early minutes.

The HeartLight sensor on baby Daisy

About one in 10 babies need help to stimulate breathing in the minutes after their birth. Many have been born too early, but even babies born at full term can need support. Without immediate help, there is a serious risk that these babies will suffer brain damage or die.

Supported by Action funding, a new heart rate monitor has been developed that shaves critical seconds off current methods used when a baby is being resuscitated. Called HeartLight, this new device is now in the final stages of evaluation and it's hoped it will be available across Europe within the next year, to help the most vulnerable new arrivals.

How we helped

In 2008, Action Medical Research awarded almost £116,000 to a team of doctors and engineers based at the University of Nottingham. This money was used to help develop, test and refine the hands-free HeartLight sensor, which is attached to a baby's forehead and used to provide a continuous heart rate reading.

Usually during resuscitation, doctors or midwives will monitor a baby's heart by listening in with a stethoscope then calculating the heart rate themselves. This means stopping every 30 seconds or so to repeat the checks and see how the baby is responding. The research team hoped their new device would save vital time, as well as reducing the risk of human error.

The initial trial, which our funding supported, was crucial to find out whether or not the proposed technology could work for newborn babies – the sensor idea was based on protective equipment originally developed for miners to use inside their hard hats.

These first tests, involving more than 100 babies, showed that HeartLight was indeed reliable and accurate when used in a neonatal setting, with the new device taking only a few seconds to set up and able to give a first reading within just five seconds.

Research team member Dr Don Sharkey says: "Effective newborn resuscitation can prevent long-term problems such as brain damage, respiratory illness and even blindness. This new device could make the process quicker and safer."

The use of HeartLight in reducing babies' chances of developing long-term disabilities could provide significant long-term cost benefits to the NHS. Of course, the benefits to babies and their parents would be priceless.

This work was supported by a donation from the Jessie Spencer Trust.

Daisy's story

Now five, Daisy was born 13 weeks early, weighing just 1lb 8.5oz. Her arrival was sudden and she was small for her gestational age. While she did make an attempt to breathe by herself, she was so tiny that life-saving ventilation was the only option.

"When we first saw her in intensive care it was very frightening," recalls her mum, Deborah. "She was connected to all the wires and machinery, looking so vulnerable. She could easily fit in the palm of her dad's hand."

Daisy's early weeks were a roller coaster of ups and downs and her parents became acutely aware of the importance of research to help sick and premature babies.

When she was four weeks old, Daisy took part in the Action-funded HeartLight trial. "We knew Daisy had been given treatments that had been previously researched and we wanted to be able to do something to help babies in the future," says Deborah.

Daisy eventually came home two weeks after the date she was originally due and recently became a big sister to baby Poppy, who also arrived early.

Every day
200+
UK babies need help to breathe at birth

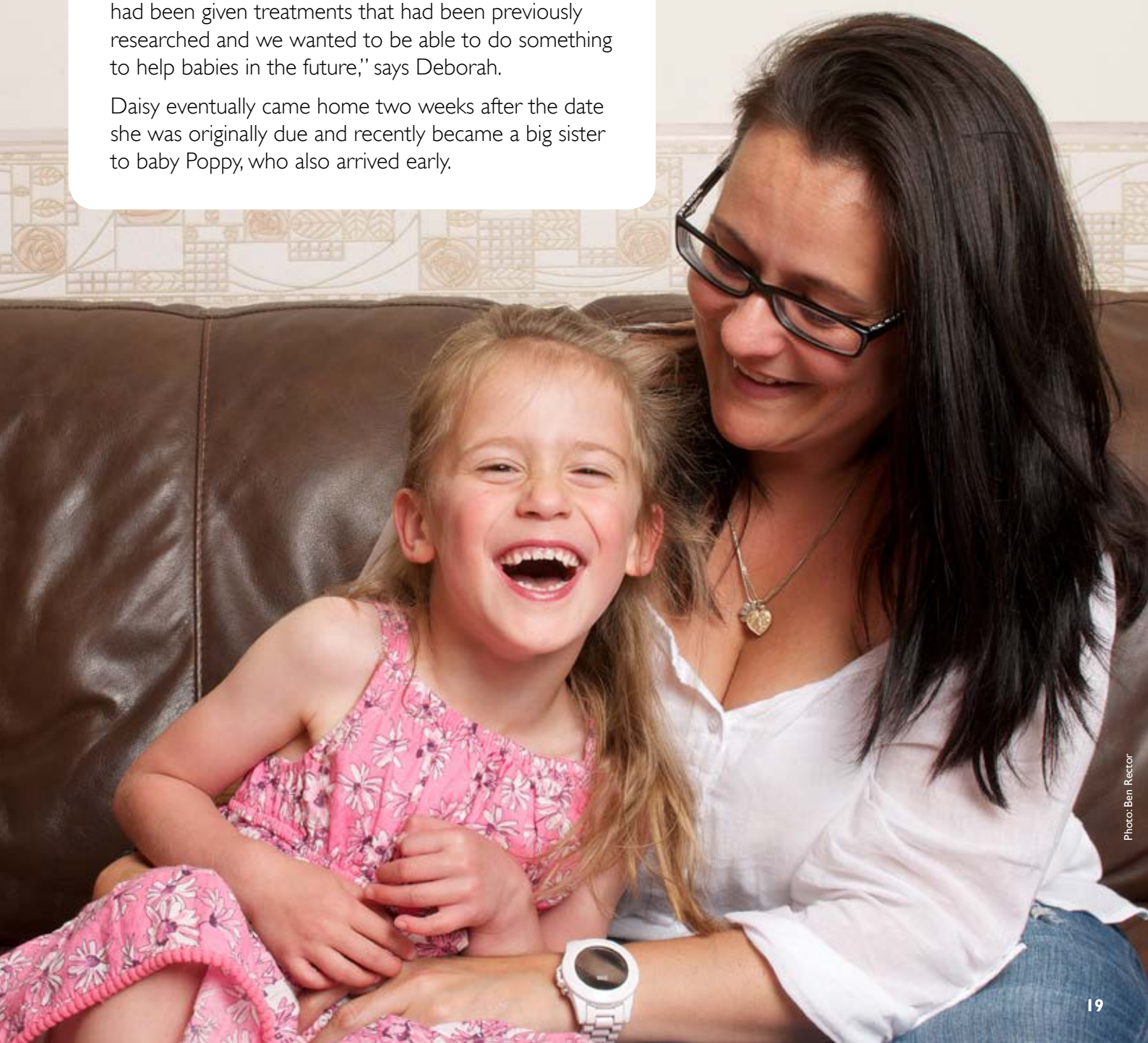


Photo: Ben Rector

Research grants awarded in 2015

Action Medical Research is funding over 75 projects, 23 of them awarded in 2015. **The next medical breakthrough could be on your doorstep.**

Cerebral palsy – communication by eye pointing*
Lead researcher:
Dr Michael T Clarke
University College London and Great Ormond Street Hospital

Crohn's disease in children – dietary treatment
Lead researcher:
Dr Harween Dogra
Barts and the London School of Medicine and Dentistry

Cystic fibrosis and spinal muscular atrophy – prenatal diagnosis**
Lead researcher:
Professor Lyn S Chitty
University College London and Great Ormond Street Hospital

Developmental coordination disorder – understanding how young people learn new movements***
Lead researcher:
Professor Helen Dawes
Oxford Brookes University and University of Oxford

Epilepsy – treatment with electrical brain stimulation*
Lead researcher:
Dr Antonio Valentin
King's College London and King's College Hospital, London

Infections in children with weakened immune systems*
Lead researcher:
Professor Judith Breuer
University College London and Great Ormond Street Hospital

Landau Kleffner syndrome – a childhood epilepsy disorder
Lead researcher:
Dr SF Adeline Ngoh
University College London

Leukaemia – targeting treatment*
Lead researcher:
Dr Owen Williams
University College London

Leukodystrophies – gene therapy (Pelizaeus-Merzbacher disease)
Lead researcher:
Professor David H Rowitch
University of Cambridge

Metabolic disease – understanding the causes and developing new treatments*
Lead researcher:
Professor Nicholas DE Greene
University College London

Neuroblastoma – developing new targeted immunotherapy****
Lead researcher:
Professor W John Anderson
University College London and Great Ormond Street Hospital

Neuroblastoma – predicting relapse and survival*
Lead researcher:
Professor Deborah A Tweddle
Newcastle University

Neurofibromatosis type 1 – identifying early signs of autism and ADHD*
Lead researcher:
Professor Mark H Johnson
Birkbeck, University of London and University of Manchester

* Jointly funded with Great Ormond Street Hospital Children's Charity
** Funded with Great Ormond Street Hospital Children's Charity and Cystic Fibrosis Trust
*** Jointly funded with The Chartered Society of Physiotherapy Charitable Trust
**** Funded with Great Ormond Street Hospital Children's Charity and Neuroblastoma UK

Obesity in children – is there a link with diet and physical activity of mothers during pregnancy?
Lead researcher:
Professor Lucilla Poston
St Thomas' Hospital, King's College London

Personalising surgery for babies with a serious heart disease*
Lead researcher:
Dr Pablo Lamata
King's College London, St Thomas' Hospital and Evelina Children's Hospital, London

Pre-eclampsia – do changes to a mother's cardiovascular system during early pregnancy predict outcome?
Lead researcher:
Mr Christoph C Lees
Queen Charlotte's and Chelsea Hospital, Imperial College London, Addenbrooke's Hospital, Cambridge and the University of Cambridge

Preterm children – reducing learning disabilities in maths
Lead researcher:
Dr Samantha J Johnson
University of Leicester, University of Nottingham, Loughborough University, University College London and University of Ulster

Preterm labour prevention
Lead researcher:
Professor Mark R Johnson
Chelsea and Westminster Hospital, Imperial College London and St Thomas' Hospital, King's College London

Systemic lupus erythematosus in children
Lead researcher:
Dr Kate R Webb
University College London

Tourette syndrome – understanding tics and habits*
Lead researcher:
Professor Georgina M Jackson
University of Nottingham

Traumatic brain injury – improving the diagnosis of potential future problems*
Lead researcher:
Professor David J Sharp
Imperial College London, Hammersmith Hospital, University College London and Great Ormond Street Hospital

Visual health in children with disabilities
Lead researcher:
Professor Kathryn J Saunders
Ulster University, Queen's University, Belfast, Royal Victoria Hospital, Belfast

X-linked lymphoproliferative disease – further development of T cell gene therapy*
Lead researcher:
Professor H Bobby Gaspar
University College London

Summarised financial statements

for year ended 31 December 2015

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 20 April 2016. The full financial statements, on which the auditor, Buzzacott LLP, gave an unqualified audit report on 20 April 2016, will be submitted to the relevant statutory bodies, including the registrar of companies, on 11 May 2016.

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 2015.

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
20 April 2016

Income and expenditure	2015	2014
	£000s	£000s
Net incoming resources		
Donations and legacies	3,580	3,708
Investments	0	18
Trading	2	2
Total net incoming resources	3,582	3,728
Outgoing resources		
Medical research projects	3,899	3,345
Medical dissemination	851	816
Net outgoing resources	(1,168)	(433)
Net (losses) gain on investments	(179)	451
Net movement in funds	**(1,347)**	**18**

Balance sheet	31 Dec 15	31 Dec 14
	£000s	£000s
Fixed assets		
Tangible	385	411
Investments	13,620	15,055
Total fixed assets	14,005	15,466
Current assets	2,500	1,686
Current liabilities within one year	(6,508)	(6,085)
Liabilities falling due after one year	(3,018)	(2,741)
Total net assets	6,979	8,326
Representing:		
Unrestricted funds	6,979	8,326
Restricted funds	0	0
Total funds	**6,979**	**8,326**

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 2015.

Supporters

Abbeygate Wealth Management
Allen Ford
Arun Estates
BDO
British Telecom PLC
Garmin
High 5
Keepmoat
Maserati
Milton Damerel Trust
Mortimers Bakery
Next Retail Ltd
One Stop
Quai Administration Services
Rouleur
Sherwood Press

Who's who 2015

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
Stephen May
Richard Price
The Duchess of Northumberland
The Earl of Snowdon GCVO RDI FSIAD
The Earl of Strathmore and Kinghorne
– deceased February 2016

Trustees
Phil Hodkinson, Chair
Charles Jackson, Honorary Treasurer
Esther Alderson
Luke Bordewich – appointed January 2015
Professor Sarah Bray BA MPhil PhD FMedSci
Professor Mark Gardiner
Professor Andrew J T George MA PhD DSc
– retired April 2015
Caroline Hume-Kendall
Rachel Molho – appointed January 2015
Nick Peters
Val Remington-Hobbs

Scientific Advisory Panel
Professor Sarah Bray BA MPhil PhD FMedSci, Chair
– retired from chair December 2015
Professor Graeme Black DPhil FRCOphth
– appointed September 2015
Professor David Edwards
MA MBBS DSc MRCR FRCP FRCPCH FMedSci
Professor Judith Goodship MD FRCP – retired June 2015
Professor Mark Johnson PhD MRCP MRCOG
Professor Fenella Kirkham MB BChir MD FRCPCH
Professor Nigel Klein BSc MBBS PhD MRCP FRCPCH
Professor Sailesh Kotecha PhD FRCPCH – retired January 2015
Professor Thomas MacDonald PhD FRCPath FMedSci
Professor Colin Michie MA FRCPCH FLS FRSPH FRSS
– appointed September 2015
Mr James Robb MD FRCS – retired January 2015
Professor Rod Scott MBChB PhD – retired January 2015
Professor Gordon Smith MD PhD FMedSci
– retired December 2015
Dr Julie Stebbins DPhil CSci SRCS
Professor Dieter Wolke PhD DipIPsych CPsychol AFBPsS

Photo: Ben Rector

Skerritts
Smeg (UK) Ltd
Stobart Group
Team Sky
The Athenaeum
Thomas Miller Investment
Unilever

Gifts in wills

Alexander Barlow
Beatrice Bridson
Freda Carter
Billy Coburn
Rachel Cowan
Lenore Davies
Cynthia Dean
Jean Denton
Marjorie Dutton
Isabel Elmslie
June Exworth
Pauline Green
Edward Harris
John Hawkins
Doreen Kirby
William Myers
Mary Peacock
Angela Rendall
Frank Shipperbottom
John Sparks
Diana Stevens
Marie Verrall
Sylvia Walter

Gifts in wills are a simple and flexible way of giving.
For more information please contact Jane Tarrant, Legacy Officer, on **01403 327414** or email **jtarrant@action.org.uk**

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