



A major  
breakthrough  
is imminent

All we need  
is **you**



**“The drooping corners of her mouth and the silent tears on her cheek as the bell and the bustling nurses chivvied ‘visitors’ from the ward will remain with me forever.”**

These were the words of Duncan Guthrie, whose daughter Janet had been struck down by polio in 1949. He made it his mission to raise funds to help eradicate new cases of the disease in the UK, to stop children like Janet suffering and prevent other parents having to bear the distress that he went through. Within ten years, the UK polio vaccine was introduced and it has kept millions of children safe ever since. The work of Action Medical Research, the charity that he founded almost 60 years ago, is still driven by his belief – that the diseases that blight the lives of so many of our children, can be beaten.

Action Medical Research has much to shout about, from the research that tested the early rubella vaccine, to discovering the importance of folic acid during pregnancy in the prevention of spina bifida and advances in the diagnosis and treatment of epilepsy. Further successes have included developments in the use of infra-red scanning technology to help doctors minimise the risk of brain damage in babies, and research that has contributed to the discovery that cooling babies deprived of oxygen at birth can help minimise brain damage and reduce disability.

At Action Medical Research we know that there are children suffering today who could be helped by medical research. Children like Grace, the nine-year-old girl who suffers from a devastating rare disease called Rett syndrome for which there is currently no cure; like Riley who has severe cerebral palsy and struggles to control his arm and leg movements and has whole body spasms; and like little Katie who developed the dangerous condition hydrocephalus – known as ‘water on the brain’ – within weeks of being born prematurely.

The prospects for finding new cures and treatments are excellent thanks to recent advances in science and medicine – a lack of funding for medical research is the biggest obstacle to achieving breakthroughs for children like Grace, Riley and Katie. With your help, we are determined to change this and make a difference to children’s lives. They are depending on us.

Action Medical Research does not focus on one particular disease. This gives us the flexibility to respond to serious health needs as they emerge, to take advantage of new discoveries and technologies and fund important developments in areas as diverse as premature birth, epilepsy and diabetes.



Photo: Jeff Coers

Unlike many charities, we receive no government funding; this not only guarantees our independence but means we have to fundraise for every penny that we spend on medical research. Our work therefore relies entirely on supporters like you. And we have a desperate need for more supporters: we currently can only afford to fund around half of the children’s research that we want to – simply because of a lack of funds.

The lives that have already been saved through the breakthroughs that Action Medical Research has helped deliver demonstrate that your gift to the charity has a real chance of achieving something very special. As a former trustee of the charity, this work means so much to me. So I ask you, this year, join us in something special, please become an Action Partner.

A handwritten signature in purple ink that reads 'Fiona Bruce'. The signature is fluid and cursive, with a long horizontal stroke extending to the right.

**Fiona Bruce**  
Action Partners President

Fiona Bruce is a committed supporter of Action Medical Research and the vital work we fund. She has served as a trustee and is President of the Action Partners programme.



# Important work that needs your support

At Action Medical Research we are determined to stop the suffering of babies and children caused by disease and disability.

We know that medical research can save and change children's lives. We have the flexibility to identify the very best medical research most likely to make this vital difference for sick children of all ages. Only a lack of funds will hold us back.

Today, as the leading UK-wide medical research charity dedicated to helping babies and children, we make a difference by:

- tackling premature birth and treating sick and vulnerable babies
- helping children affected by disability, disabling conditions and infections
- targeting rare diseases that together severely affect many forgotten children.

We do this by:

- finding and funding cutting-edge medical research across the UK most likely to deliver real benefit to babies, children and young people
- assessing the impact of the research we fund and sharing the results
- raising funds to make this vital research possible.

## **Please help**

Action Medical Research needs to continue to contribute to future breakthroughs for the benefit of sick babies, children and their families – it's what we do. But we can't do it alone.

# Tackling premature birth and treating sick and vulnerable babies

One in 10 babies in the UK needs some form of special care at birth as a result of a difficult birth, a life-threatening condition, or because they were born too early. Tragically, each year more than 3,000 babies die before they reach their first birthday and premature birth is the single biggest cause. Yet so little is known about what causes premature birth and how to prevent it.

Babies born too early can face difficulties with breathing, feeding and fighting infection. Many of those born very early develop lifelong conditions such as cerebral palsy or blindness. We also need to invest in more research to prevent pregnancy complications that threaten babies' lives, and help doctors searching for new and better ways to care for babies who are sick and vulnerable following a difficult birth or a life-threatening condition.

Tens of thousands of people have already been moved to give to our Touching Tiny Lives appeal for this vital work and some of their own stories are truly heartbreaking, like Lee Collier and his partner Karen who lost their daughter Phoebe in December 2005 when she was born at just 23 weeks and four days – 'too early and asleep'.



# Katie's story

## The struggle behind the smile

Born almost two months early, Katie needed resuscitating at birth and spent her first weeks of life in neonatal intensive care. At three-weeks-old her head became rapidly bigger. A scan revealed she had hydrocephalus, also known as water on the brain. This had been caused by a bleed in her brain. Often the condition requires an operation to insert a special tube into the brain, called a shunt. Thankfully for Katie, doctors were able to draw off the excess fluid with a needle, avoiding the need for surgery.



Always a fighter, Katie was allowed home just two days later but has since had many difficulties to overcome. At 10-months-old she was diagnosed with epilepsy and she struggled with her balance once she started walking. She seemed unusually clumsy and fell over a lot. Her parents were told she had global developmental delay.

Katie has been slower to develop both physically and in terms of her speech, and her eyesight is also poor. But with extra help, the little girl has thrived.

Proud mum Siobhan says: "She has done amazingly well. All in all she is your average four-year-old now. You would never realise she has been through so much."





# Helping children affected by disability, disabling conditions and infections

Every six hours in the UK a child is diagnosed with cerebral palsy. We need to find ways to help make life more comfortable for disabled children facing a lifetime of challenges caused by conditions like cerebral palsy, as well as helping children dealing with disabling conditions such as epilepsy and diabetes. And we have to find out more about conditions like multiple sclerosis and stroke, that we usually associate with adults, but that devastate the lives of thousands of children in the UK today. We need right now to help children like Alfie, the six-year-old boy who has had more strokes than birthdays; we cannot stand by and let this suffering continue.

While polio and rubella are thankfully all but consigned to history in the UK, other infections still pose a very real threat to babies and children today. Meningitis and septicaemia for example, kills more children in the UK than any other infection and can devastate the lives of those who survive. The charity needs to fund doctors searching for new vaccines and antibiotics to treat and prevent such potentially deadly infections.

# Supporting disabled children

## Riley's story

Being able to sit comfortably is something most children and their parents take for granted. But for some very severely disabled children, like six-year-old Riley Runciman, sitting in a standard seat or wheelchair can actually be a frightening and painful experience. Riley suffers from a severe form of cerebral palsy meaning he struggles to control his arm and leg movements and has whole body spasms. Riley is only really comfortable sitting on someone's lap, so his parents Mandy and Lee are almost constantly taking it in turns to hold Riley.

Now an Action Medical Research funded team is developing a chair that moves with the child during a spasm, emulating the behaviour of a parent or carer. "This chair could radically change Riley's life and improve our family life too," says

Lee. "Riley would be able to participate more fully at school, and for us to be able to carry out normal tasks, such as putting out the rubbish or cooking dinner, would make an enormous difference."





# Targeting rare diseases that severely affect many forgotten children

Thousands of families across the UK are right now caring for a child with a rare and devastating disease whose very rarity often makes it less of a priority for research institutions and pharmaceutical companies. We need to bring hope to those forgotten families by supporting research to tackle rare, pernicious diseases such as the metabolic condition Sandhoff disease.

A baby with Sandhoff seems normal at birth, but within six months, parents may start to notice that something is wrong – their baby may not learn how to sit up properly, or play with

toys. Sadly, most children with Sandhoff disease die at around three years of age after suffering distressing symptoms, such as blindness, seizures, and difficulties swallowing and breathing.

To hear that your child has been diagnosed with an incurable disease like Sandhoff, about which so little is known other than it will cause such suffering to your child, would be most parents' worst nightmare.

But there is hope – our researchers believe that there is a real possibility of making breakthroughs in preventing and treating rare diseases. But more funding is needed, and urgently.

# Hope for girls like Grace

Grace Watson had often been the first baby from her mum's antenatal class to reach those all-important early milestones, but between nine and 12 months things began to change. Other babies started crawling, walking or talking but Grace didn't. Nobody knew it at the time, but she had Rett syndrome, a rare developmental disorder that causes profound and multiple physical and learning disabilities.

Babies with this debilitating condition, predominantly affecting girls, initially seem to develop normally but then a devastating regression occurs and they start to lose previously learned skills and control of movement.

Grace's mum Gwenda says: "The diagnosis was a huge shock. The worst case scenarios so awful. I was told it was very unlikely she'd ever speak or walk unaided and that she would undoubtedly need to go into residential care when she's older."

Now nine-years-old, Grace did learn to walk on her own just two years ago, but life is difficult for her and her family. "There are still times that I grieve for the child I thought I had. I had looked forward to my little girl doing ballet, cooking with me and lots of other things," says Gwenda. "We get stared at a lot, especially by people who don't understand her difficulties because she doesn't really look disabled. And we don't know what the future holds. It is daunting but we try to live for today and appreciate the things Grace can do."

Action Medical Research is supporting pioneering work that could lead to a therapy to reverse the symptoms of Rett syndrome.



# What does becoming an Action Partner really mean?

Action Medical Research needs to raise £15m for medical research by the end of 2014. This means we can double the amount that we have been able to spend on research for children, and fund around 100 new medical research projects. This ambitious challenge is one that we must meet, because we know that medical research can save and change children's lives, but we need your help to make this a reality.

## **Your commitment**

Action Partners give a minimum of £5,000\* a year for three years. This helps us to focus funds as and where they are needed. Of course, the more you can donate, the more we can do; the average Action Partner gift is closer to £8,000 a year.

Each of the three areas of work described has been specifically chosen because important advances have already been made in working towards effective treatments and with your help we can drive this vital research through to the next stage and help make major medical breakthroughs a reality.

Your support over three years could change the lives of countless newborn babies and children. And as an Action Partner, you'll be kept up to date with the latest developments in the medical work you are funding – so you can be there at the breakthrough.

## **Our commitment**

Action Partners are free to choose their own level of involvement. We can offer you newsletters providing feedback on the programmes, plus exclusive reports and updates on each specific project area and its progress.

We can arrange visits to universities or hospitals to meet with leading researchers, as well as giving you opportunities to attend specific research presentations. In addition, Action Partners are invited to our annual Action Partners reception and other Action Medical Research events.

\*Due to gift aid and tax benefits this actually equates to approx £3,000 for higher rate tax payers. You can help us achieve so much more when you become an Action Partner.

Be there at the  
breakthrough

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for children, for life