

How your support
is helping save and
change children's lives

Re:action

Spring/summer 2020

action medical research
for children

In your latest *Re:action*

Fighting for babies born too soon
Powering research into rare diseases
Tackling new strains of meningitis

Welcome

At Action Medical Research, we fight for answers that can lead to cures, treatments and medical breakthroughs for some of the toughest fights our children face.

In this issue, find out about some of our newest research projects – from a total of 14 funded in 2019. There's also exciting news of progress made in research that has now finished, all thanks to supporters like you.

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Find out more!

With your support we're currently funding more than 60 groundbreaking research projects

action.org.uk/research

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Fighting for babies born too soon

With your support, we've raised £520,000 towards our BORN TOO SOON campaign. Together we can reach our £1 million target.

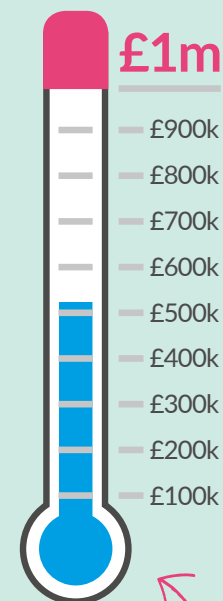
Each year in the UK, around 60,000 babies are born prematurely – like little Wilfred on our cover, born at just 25 weeks.

It's for children like Wilfred that we launched our BORN TOO SOON campaign – to shine a spotlight on the devastating impact of premature birth, and raise £1 million by the end of 2020 to fund vital medical research that could benefit millions.

Parents, families and communities have been fighting back.

In November, supporters helped us mark World Prematurity Day by going purple to raise funds.

Others have trekked across mountains, organised family fun days or taken on our 60,000 Reasons event; challenging themselves to walk, run or even skip 60 miles in 60 days! It has been amazing to see such support.



£520k of our target raised so far!



Help us hit £1 million! There are lots of fun ways to support BORN TOO SOON this summer.
action.org.uk/borntoosoon

Wilfred's fight

**BORN
TOO
SOON**



“This work is really important – to know that research is trying to ensure that people don't have to go through premature birth”

Born extremely prematurely, Wilfred was so very delicate his parents were unable to hold or even touch him for his first three weeks.

Wilfred's mum Imogen's pregnancy had been progressing smoothly, until labour pains started without warning more than three months too soon.

Medics desperately tried to slow her labour, but baby Wilfred was born at just 25 weeks and four days, weighing a tiny 2lb 6oz.

In the following days, tests showed that, sadly, he had suffered a bleed into his brain – and there was no way of knowing how badly he might be affected.

Then doctors feared that a potentially deadly bowel disease, necrotising

enterocolitis (NEC), had taken hold. So, at just six days old, Wilfred endured surgery to have 25cm of his bowel removed and a stoma bag fitted to manage his body's waste.

After four months in hospital, and a further operation to reverse his stoma, little Wilfred finally went home, weighing 7lbs.

Now aged three, he has been diagnosed with cerebral palsy but thankfully is mildly affected, leaving his left side a little weak.

“He's a happy, determined little character and takes everything in his stride,” says his proud mum, Imogen.



With your support...

Action is funding research that aims to develop more effective treatments that could safely stop premature contractions, reducing the risk of early birth.

Although the causes of premature birth are complex, it often occurs when the mother goes into spontaneous early labour – like Wilfred's mum.

Unfortunately, there are no treatments currently available that can reliably

prevent premature contractions while being risk-free for mother and baby. Many drugs that work to reduce contractions may also relax blood vessels and affect blood flow to the womb or placenta, endangering the baby.

With Action funding, a team of researchers at Newcastle University is studying new ways to specifically target just the muscles of the womb, without affecting its blood vessels or those of the placenta.

Action is jointly funding this research with Borne.

**THANK
YOU!**

Thank you for making this vital research happen.
Find out more [action.org.uk/borntoosoon](https://www.action.org.uk/borntoosoon)



“ We are determined to fight NPC ”

Niemann-Pick disease type C (NPC) is a rare neurodegenerative disorder, caused by faults in a gene. It causes unwanted cholesterol and other fatty substances to build up inside the body's cells, resulting in nerve damage.

Over time, children with NPC develop major problems coordinating movements such as walking. They also face losing their speech, a gradual decline in their intellectual abilities and symptoms of dementia. Tragically, many children do not survive into adulthood.

Matilda's story

Sadly, for a little girl full of smiles, it was an unusual reaction to laughter that first gave Matilda's parents cause for concern. Then aged five, when she found something funny Matilda would lose all muscle tone and collapse to the floor, sometimes badly hurting herself.

Doctors ruled out many causes before testing for NPC. The diagnosis, when it

came, left the family overwhelmed by anger and fear.

Matilda is now 10 and at the moment has issues affecting her sight, and some memory and cognitive impairments. She is a few years behind in her school education. The future is very uncertain and research means absolutely everything to Matilda and her family.

“ This disease will try and take away abilities that Matilda has, so we need to fight against it ”

Testing a new treatment

Right now, treatments for NPC are extremely limited. But, with your support, Action is helping to fund a two-year study to test a potential new drug treatment.

Led by Professor Frances Platt at the University of Oxford, this research is examining the exciting discovery that an existing multiple sclerosis drug could also help fight the symptoms of NPC. This is an exciting collaboration, with Action, the Niemann-Pick Research Foundation and Niemann-Pick UK funding this study, together with NPSuisse and Niemann-Pick de Fuenlabrada. If the results are promising, it is hoped that this could quickly move into clinical trials involving patients.



Thank you for making this vital research happen.
Find out more [action.org.uk/NPC](https://www.action.org.uk/NPC)



Changing Lives

Your support has enabled researchers to further develop a new technique to help children with hard-to-treat epilepsy. It uses new ways of electrically stimulating different parts of the brain using electrodes placed under the skull.

Children have already benefited, with some amazing results.

“Sophie is doing very, very well.” says her mum, Anne. “She got A grades in her A Levels, then took a gap year and visited 30 countries. She is now at university, living independently of course.”

“None of this would have been possible without the surgery she underwent”

Find out more: action.org.uk/successes

A phenomenal transformation

Your support has helped to refine a surgical treatment that can be life-changing for children suffering from severe dystonia.

Edward was just 16 months old when he began to suffer from dystonia – a serious and unpredictable movement disorder that sees children experience uncontrollable spasms.

Triggered by abnormal signals from the brain, dystonia causes repetitive movements and parts of the body may be twisted into unusual positions. Severely affected children can require heavy sedation and long hospital stays. It can become life-threatening.

Within a few months Edward, who had previously started walking and saying his first words, could no longer lift up his arm to take a toy. He could no longer sit up, unless his body, head and limbs were strapped into place, and he was being fed through a tube.

“I’d written off the idea that Edward would ever walk again”

It was one of the worst cases doctors had ever seen and Edward needed ‘huge volumes’ of medication to keep him comfortable. “If he wasn’t medicated or asleep, he was in pain and crying,” says his dad, Martin.

How research helped

In 2012 Action awarded funding to a team led by Dr Jean-Pierre Lin at London’s Evelina Children’s Hospital. He sought to improve a surgical treatment called deep brain stimulation (DBS). This involves the insertion of electrical wires into specific areas of the brain, through which electrical pulses are delivered via a battery to control the spasms.

The team took highly specialised scans to make maps of children’s brains. These new images helped to more clearly identify different areas which could be treated. They also helped doctors decide when

surgery was a suitable option, and informed neurosurgeons of the best area to insert wires.

This all improved the chances of success and children are already benefiting. Some, like Edward, with astonishing results.

Edward’s treatment

Dr Lin’s team saw Edward when he was two and, after many tests, decided that he was suitable for DBS surgery.

His operation, just after his third birthday, was more successful than his family dared believe possible. Within a day, his medication was halved, and the improvements continued over time. Now nine, Edward is in mainstream school and runs around happily with his walking frame.

“The original goals were blown right out of the water,” says Martin. “It’s a phenomenal transformation.”

“I’ll never be able to put into words how grateful we are”

THANK YOU!

It’s estimated that several hundred UK children could benefit each year from this treatment.

Thank you for making this vital research happen.



Tackling new and emerging causes of meningitis

Most cases of pneumococcal meningitis are now caused by strains of bacteria not covered by existing vaccines. Action is supporting a nationwide study to improve understanding and treatment of these non-vaccine strains.

Pneumococcal meningitis most often affects babies and toddlers, and can have devastating consequences. Sadly, up to one in seven lose their lives and a quarter are left with severe after-effects – including sight or hearing loss, seizures and learning disabilities.

Thankfully, in the UK and Ireland, vaccines now protect against the most common strains of pneumococcus bacteria. These have led to a rapid decrease in severe pneumococcal infections, including those which cause meningitis. Researchers now need to find out more about cases caused by non-vaccine strains.

Led by Dr Godwin Oligbu, researchers are analysing data from children diagnosed with pneumococcal meningitis across the UK and Ireland, and from laboratory analyses of biological samples to identify the bacteria involved. They are also studying symptoms and treatment received.

The team hopes to identify opportunities for improving diagnosis and treatment, and to develop new guidelines that reflect the changing nature of the disease.



“Our results will enable us to make recommendations for future studies, and interventions that can save lives and reduce complications,” says Dr Godwin Oligbu.

Over the
last 40 years
Action has invested
£1.4m
into meningitis
research

Pneumococci bacteria can cause meningitis



Remembering Elijah

When their premature baby son Elijah tragically died, Jenny and James Halse set out to raise £37,000 – £1,000 for every day of his short life.

Baby Elijah was born extremely prematurely, at just 25 weeks and three days, in March 2013. Sadly, although his initial prognosis had been promising, he became desperately ill with necrotising enterocolitis (NEC), a devastating bowel infection.

His heartbroken parents decided to channel some of their emotion and grief into raising money in his memory. Jenny and James signed up for their local Percy Pud 10K – an event that starts and finishes close to where Elijah is buried. More than 50 people joined them, many returning every year since, running as Team Lightning – the nickname given to Elijah by one of his two older brothers because he'd been born so quickly.

Reaching their target understandably brings mixed emotions for the family. "But what it really represents is love," says Jenny. "Seven years of active, loving support from countless people who stood beside us. People who believe in the opportunity to try and change outcomes for babies like Elijah."

Elijah's Tribute Fund has raised more than £38,000 for Action. Thank you to Jenny, James and Team Lightning for all their support.



Keith's big birthday challenge

Keith is turning the big 5-0 and taking on five half-marathons to celebrate!

Keith Mitchell's five-year-old son, Aiden, suffered a shortage of oxygen to the brain when he was born. He received cooling therapy, reducing his body temperature to try and protect him from brain damage. This therapy is a breakthrough treatment that Action helped to develop, and Aiden's parents credit it with saving his life.

"Aiden has cerebral palsy, but he's a fighter," says Keith. "He's getting a chance at life and we want to ensure others get that same chance."

Keith's efforts are supporting several charities who have helped the family, and he recently took on the Big Half, raising an amazing £625 for Action.



“Action has a very special place in our hearts”

10 years of cycling with Davina

Davina McCall, our charity ambassador, has been fundraising for Action since she was a little girl but for the last decade her support has seen her getting on her bike every summer. As always, she would love as many people as possible to join her for Davina's Big Sussex Bike Ride on 14 June – let's make this milestone event one of the biggest yet!



action.org.uk/davinaride

Challenge events 2020

Running

Virgin Money London Marathon 26 April

Edinburgh Half Marathon, 5K & 10K
23 to 24 May

Virgin Money London 10,000 25 May

Cycling

NEW **Wyking Ride** York, 19 April

Castle Ride 100 Kent, 3 May

Suffolk Sunrise 100 17 May

Land's End to John O'Groats 9 to 21 June

**Davina's 10th anniversary Big Sussex
Bike Ride** 14 June

Maratona dles Dolomites 5 July

London to Paris with Sean Yates 15 to 19 July

Prudential RideLondon-Surrey 100
16 August

Essex 100 6 September

Treks

Snowdon by Night 13 to 14 June

Yorkshire 3 Peaks Weekend 28 to 30 August

Jurassic Coast Weekend 4 to 6 September

Team Challenges

Race the Sun Brecon Beacons 12 to 13 June

Race the Sun Lake District 4 to 5 September



PLUS more... Sign up today action.org.uk/events
Give us a call on 01403 327444, or email events@action.org.uk