Hope for boys with Hunter syndrome
Read Danny’s story

Plus
Sight tests in special schools help children with learning disabilities

Protecting children, now and always – our COVID-19 appeal
In this issue:

3 Action news
Your ongoing support is vital during these difficult times

4 COVID-19 appeal
Help us fund research into the effects of COVID-19 on children

6 New research
Including work to test a new leukaemia treatment and to protect premature babies from infection

8 Danny’s story
Sadly Danny has the life-limiting disease Hunter syndrome. Read his story and about new research

10 Giving in your will
Why supporter Maureen has planned to leave Action a much-needed gift

11 You made it happen
Exciting progress made in research funded by your support

12 Making an impact
How Action funding has helped pave the way for dedicated eyecare services to be delivered in special schools

14 Fundraising news
Weekly lottery win and our amazing lockdown fundraisers

16 Challenge events
London to Paris 2021, plus our autumn virtual challenge

Your support means so much

With the COVID-19 crisis affecting so many of our events, it’s been wonderful to see people getting involved in our lockdown fundraising. Your support makes such a difference.

While we’ve all had to get used to new ways of living, one thing that hasn’t changed during these difficult times is how amazing Action supporters can be. Our big bike rides, team events and runs, plus busy social events contribute so much to our fundraising, and have all been put on hold. So we know there will be a big gap to fill this year.

To those who have donated to our Emergency Appeal, which has now raised over £90,000. To event participants who took their back gardens or local streets to complete the ride or run distances they had intended to do for us. And to those who joined in with one of our various isolation challenges, or our online quiz. However you have supported us, we are so grateful.

We continue to feel the effects of the pandemic and as a charity that receives no government funding for research, your support is needed now more than ever. Children and families still need our help, and research can bring hope to them. We hope you will continue to help us and together we can fight back and save and change children’s lives – now and always.

For families like Matilda’s, pictured right, who has the rare disease Niemann-Pick type C, this means so much.

Read more about some of our fantastic lockdown fundraisers on page 15. Plus don’t forget to sign up for our new virtual event this October – find out all about Big Steps for Tiny Lives on the back cover.

Thank you so much to all who have supported us during this difficult time. It means so much to all the children and families our research helps.
COVID-19 children’s research appeal

For almost 70 years, Action has risen to the medical research challenges of the day. We’ve helped develop life-saving vaccines, treatments and cures – saving countless lives. In 2020, a new health crisis has emerged.

The current pandemic highlights that medical research has never been more important. But while investment in research to help understand the disease in adults has been swift, there is a worrying lack of research specifically focusing on children and how the virus affects them. We urgently need to fill this gap. That’s why we’ve launched our COVID-19 children’s research appeal – but we cannot fund this research without your support.

COVID-19 can and does affect children, both mentally and physically, sometimes with devastating consequences. As children start to return to school, parents are understandably fearful about the unknown effects of coronavirus and they have questions that need tackling.

- Why are some children more vulnerable than others?
- How can we protect children now, and from viruses like this in the future?
- How will the pandemic affect children’s mental health?

We also need to ensure that children’s care is tailored to their needs – treatments suitable for adults may not always be transferable. So it is important that children and young people are included in any trials of treatments or vaccines. And finding out why most children have been less affected by COVID-19 could also help us to better understand and fight the disease in all age groups.

Expert advice

Building on our impressive track record of funding research that saves and changes children’s lives, we have brought together an expert advisory group of leading children’s health researchers to help us fund research to better understand how COVID-19 affects children.

As Dr Barney Scholefield, paediatric intensive care consultant and researcher, and a member of the Action advisory group, says: “Children have been the quiet, forgotten bystanders in the pandemic. It is essential we bring them to the forefront, as they can help us all understand this disease better and we must improve the way we care for them during these uncertain times.”

Protecting children, now and always

Action began its work back in the 1950s in response to an outbreak of another terrible disease, polio.

Polio was one of the most feared diseases in the world, and at the time schools, churches and cinemas closed. In the 1950s, 8,000 people were paralysed by polio each year in the UK, and up to 10 per cent died after their breathing muscles became immobilised.

We were instrumental in helping to develop the first polio vaccines in the UK which have kept millions of children safe from this deadly virus ever since.

Since then we’ve helped to tackle dangers like meningitis and rubella, saving countless lives.

Today, once again, parents are living in fear of the unknown with many unanswered questions that need tackling. We are now ready to act to protect children from the threat of COVID-19.

Help us raise £1 million to fund vital research for children in the fight against COVID-19

We believe that medical research can help to find answers and protect children against this virus and the mental health effects of this health crisis. But we need your help to make this research happen.

This is an appeal for funds specifically for research into COVID-19 and children, so the number of projects we are able to fund will depend entirely on how much we raise from this appeal. Quite simply, we cannot fund this research without your support.

Please help with a donation today – 100% of your gift will go towards making this research happen.

Donate now at action.org.uk/COVID-19
Our latest Research Training Fellowships

Despite the challenges of recent months, we’re delighted to have been able to fund two new projects – to help children with a rare brain disease and to improve treatment for children with peanut allergy.

Our Research Training Fellowship scheme supports some of the UK’s most gifted doctors and scientists early on in their research careers, helping to develop future leaders in children’s medical research.

Starting in spring 2021, Dr Michael Eyre of King’s College London will investigate if advanced scans, taken early on in treatment for a rare brain disease, can identify clues that could help doctors to personalise treatment.

The disease, a type of autoimmune encephalitis, is caused by the body’s immune system mistakenly attacking the brain. This causes alarming and rapidly progressive symptoms – including seizures, confusion and loss of speech. With prompt treatment, most children survive but they face a slow recovery, and many are left with long-lasting difficulties. Dr Eyre hopes his research will lead to earlier symptom control, shorter hospital stays and reduced long-term effects. This Fellowship is co-funded with the British Paediatric Neurology Association.

Meanwhile at Imperial College London, Dr Sharanya Nagendran hopes to help children with peanut allergy – a condition that has been increasing in recent decades.

A new type of treatment, called oral immunotherapy, can be beneficial but not all children can tolerate its current form. Dr Nagendran will test an alternative approach that hopes to boost the safety and long-term effectiveness of treatment, helping more families live without fear of a life-threatening reaction.

Testing a life-saving new leukaemia treatment

With Action funding, researchers are investigating a new drug combination for a hard-to-treat type of leukaemia that typically strikes babies under a year old.

Leukaemia is the most common childhood cancer and affects white blood cells, causing them to grow too fast and take over the bone marrow.

While the majority of children can now be cured, some types of leukaemia remain difficult to treat. One is a subtype of acute leukaemia that is caused by a particular DNA change in the cancer cells. It usually affects babies, and sadly they often do not respond to treatment.

Dr Owen Williams and his team, based at the UCL Great Ormond Street Institute of Child Health, have already shown that a new combination of two existing drugs can fight this disease. They will now carry out further tests to explore the safety and effectiveness of this treatment, and the best dose.

“This could save the lives of babies and children for whom no effective treatments currently exist,” says Dr Williams.

This research is funded jointly with LifeArc.

Continuing our fight for babies born too soon

Our BORN TOO SOON campaign continues into next year, plus new research to protect these most vulnerable babies from life-threatening infections.

Our BORN TOO SOON campaign was launched two years ago, specifically to shine a spotlight on the devastation caused by premature birth – and before COVID-19 hit, our supporters had raised more than £520,000 towards our target of raising £1 million by the end of 2020. But with so many fundraising activities affected by the pandemic, our ability to reach this target has been delayed.

There is also now the urgent need to fundraise for research that could give vital insights as to how this new global disease affects children. So, whilst we continue to fund research into premature birth, we will extend our BORN TOO SOON fundraising efforts into next year.

New research underway

Bacterial infections are especially dangerous for premature babies. So, we’re delighted to already be funding new research which could lead to life-saving new tests and better treatment.

“Sadly, many preterm babies lose their lives due to a severe bacterial infection”

Around 60,000 babies are born prematurely in the UK each year and, tragically, more than 1,000 die as a result of being born too soon – many due to infections.

Those who do survive can be left with life-changing disabilities, like cerebral palsy.

With Action funding, Dr Deena Gibbons and her team at King’s College London aim to improve understanding of how the immune systems of these tiny babies react to infections, and find new ways to prevent and treat them more effectively.

The research team will study blood samples taken from premature babies, hoping to identify specific features that could be used to aid diagnosis and treatment.

“Ultimately, we hope this will lead to new tests that can help identify babies who may be at higher risk of developing a severe infection so that steps can be taken to help protect them in the critical first few weeks of life,” says Dr Gibbons.

We know that research like this could make a big difference for babies in the future.

We hope you will join us to mark World Prematurity Day in November and we look forward to continuing our fight for babies born too soon.

With your support, we can achieve our £1 million target. action.org.uk/borntoosoon
Hope for boys with Hunter syndrome

Danny’s family are painfully aware that time with their youngest son will be cruelly cut short by Hunter syndrome, a rare disease with no cure and very limited treatment options. Action is funding vital research that could help families like theirs in the future.

“We are going to lose our beautiful boy”

Hunter syndrome, which is also known as mucopolysaccharidosis type II, almost exclusively affects boys. It’s caused by a faulty gene that leads to the lack of an enzyme that’s vital for breaking down sugars in the body. Without this enzyme, waste sugar molecules build up in all the major organs, tissues and joints, causing a range of problems that get worse over time.

Children with the most severe form of the disease, like Danny, have progressive learning difficulties due to a build-up of sugars in their brain. In his younger years, this meant that Danny’s behaviour could be extremely unpredictable and challenging.

Life has got a lot calmer lately but for the saddest of reasons, as Danny is now losing skills he previously had. He no longer speaks, having previously said over 50 words and short phrases, and his parents are now having to think about adapting the house for when he becomes more reliant on his wheelchair.

“I’d love to have my little trouble back in full force, because I know what this calm after the storm is leading to,” says Sally.

Danny was diagnosed with Hunter syndrome when he was three years old, having had a history of developmental delay. When he was finally referred to a paediatrician, the doctor homed in on his looks, which his parents discovered were typical of his condition.

“As soon as I read the symptoms and saw pictures online, I knew that he had it,” says Sally.

And what she read was heartbreaking for any parent.

Danny’s parents are now having to think about adapting the house for when he becomes more reliant on his wheelchair.

Sally and husband Craig waited several agonising weeks before tests confirmed their fears and life changed forever. It would later be found that Danny has a complete gene deletion in his DNA, the worst possible outlook.

Danny has regular enzyme replacement therapy, a treatment that can help relieve some symptoms. However, this cannot currently reduce the damage the disease is causing to his brain.

For several years he was part of a clinical trial, but sadly this treatment didn’t work for him.

Sally knows that research now will come too late for them.

“Bit by bit, we will lose our beautiful boy,” she says. “But each new development brings real hope. And any new hope is worth fighting for. So that families in the future don’t have to feel that the bottom is dropping out of their world.”

New research

Being able to reduce the neurological damage caused by this disease could transform the outlook for boys like Danny in the future. Thanks to your support, Professor Brian Bigger and his team at the University of Manchester are testing a new way of overcoming the problem of getting much-needed treatment into the brain.

This research has been jointly funded as part of our partnership with LifeArc to develop treatments for children with rare diseases.

“At Action Medical Research we fight for medical breakthroughs. We fight to help children like Danny. Thank you for joining us and making this vital research happen.”

Danny’s mum, Sally
Maureen’s special gift

Maureen Chapman has been a loyal supporter for more than 40 years. Here she explains what supporting Action has meant to her, and why she has left a gift in her will to fund future medical research for babies and children.

Maureen first became an Action supporter in 1968, joining a group of friends in setting up the Hull and Beverley Committee. She fondly remembers organising regular events, including a dinner and dance, quizzes and antique evenings. A dedicated fundraiser, Maureen was secretary of the committee for 37 years. Her husband, who was Professor of Chemistry and Pro-Vice Chancellor at the University of Hull, was also involved, although Maureen comments: “He didn’t go to dances as he wasn’t very good at dancing!”

Sadly, Maureen had personal experience of one of Action’s research areas. In 1950 she lost a baby due to severe pre-eclampsia. Thankfully, five years later; she gave birth to her son Nigel, although she suffered from pre-eclampsia again and he was delivered a month early.

“Doctors back then thought that you couldn’t have pre-eclampsia twice,” says Maureen. “Which shows how far we’ve come with medical research.” Maureen also has a daughter, Rosamond, born safely in 1960 with no signs of the pre-eclampsia that had affected her earlier pregnancies.

Maureen has remained a close supporter of the charity over the years. “Action has played a big part in my life,” she says. “So I decided to leave the charity a gift in my will. I can’t get as involved as I’d like to anymore, so this is my way of continuing my support.”

“The charity has given me great pleasure, and we’ve seen some fantastic results. If you have anything to leave, leave it to Action. They work on very important research and have a great amount of success.”

A life-changing epilepsy treatment

Your support has helped researchers to develop and refine a new technique to treat children and young people suffering from severe seizures, with some amazing results.

More than 60,000 children and teenagers aged 18 and under have epilepsy in the UK. Sadly, medication doesn’t work for up to a third of these young people and other treatments are not always effective.

With Action funding, Dr Antonio Valentin, of King’s College London, has developed a new way to treat children with focal epilepsy, which originates in localised areas of the brain. It involves suppressing the area that triggers the seizures by stimulating very specific parts of the brain, using electrodes placed under the skull.

Dr. Valentin has reported that 12 children have already benefited, and it’s estimated up to 30 a year could be treated in the future.

Sophie, pictured, suffered multiple seizures a day – even with very high doses of medicine. These happened without warning, leaving her constantly at risk of serious injury.

“Sophie’s doing very, very well. None of this would be possible without the surgery she underwent.”

Research helps children with severe asthma

Action funding has helped reveal important new information about how allergies to fungi can make a child’s asthma much worse.

The prevalence of asthma is increasing, especially among children, and for those with severe disease, frequent flare-ups, hospital stays and the side effects of treatment can all harm their quality of life.

With funding from Action and the Henry Smith Charity, Dr Erol Gaillard and his team at the University of Leicester wanted to find out why some children are more severely affected and respond less well to medication. It was known that adults with allergies to fungi were more likely to suffer serious illness, but for young patients there was very limited data.

Testing almost 200 children, the team found that there was indeed a link. Those affected by fungi appeared to have asthma that was more resistant to treatment and be more likely to experience acute asthma attacks.

“We’re now screening all children attending our asthma clinic for fungal allergies – and closely following those who are affected. Using this knowledge to better tailor treatment, which we hope will lead to improvements in symptoms and quality of life.”

New free will-writing service for Action supporters

Writing a will is something that we all need to do, but we often put it off when we’re leading busy lives. The COVID-19 pandemic has sadly made many of us even more aware of this. Action’s new free will-writing service enables you to write or update a will through a local solicitor or online at home.

We know that writing a will takes careful consideration. You will want to take care of your family and friends. Many people who have taken part in this offer have chosen to leave a gift in their will to Action, and we are very grateful, but it is not compulsory to do so. For more information visit action.org.uk/freewilloffer

“Asthma affects around 1.1 million children in the UK.

You made it happen
Bringing eyecare to special schools

With your support, researchers have successfully shown the benefits of offering comprehensive eyecare services in the familiar setting of a child’s school. This could improve health, wellbeing and educational outcomes for many thousands of children with learning disabilities.

Children with learning disabilities are 28 times more likely to have a serious sight problem than other children, but nearly 40 per cent have never had an eye test or any eyecare.

Part of the problem is that access to eyecare can be extremely challenging for children with developmental disabilities and their families. These children may also be less able to express, or even recognise, that they have sight problems. Plus, health professionals and other adults can make assumptions that their behaviour is a part of their disability without exploring other factors.

As a result, sight problems often remain undiagnosed, and therefore untreated, preventing children from reaching their full potential.

How we helped

In 2016, Action awarded almost £190,000 to a team led by Professor Kathryn Saunders at Ulster University. They wanted to determine if providing full eyecare, in the familiar school setting, could help improve the vision of children with special educational needs, and improve how they engage in the classroom.

Called the Special Education Eyecare project, or SEE for short, two hundred children and young people from Northern Ireland’s biggest special school took part.

Researchers found that nearly two thirds of the children had at least one significant eye or vision problem. Nearly half also had at least one unmet visual need, like no glasses or needed large print learning materials.

The research proved that providing in-school eyecare – with glasses dispensed on-site and written information and advice shared with teachers and parents – had a positive impact.

“Benefits were apparent in both children’s vision and behaviour. The support from Action was pivotal”

Professor Kathryn Saunders

“Our study demonstrates, for the first time, measurable benefits to children and young people. These were apparent in both children’s vision and behaviour,” says Professor Saunders.

“The modern NHS requires evidence of benefit when developing and funding services. So the support from Action was pivotal in providing this evidence,” she says.

The findings have already begun to influence provision of in-school vision services for children in England, with plans to inform provision more widely across the UK in the near future – meaning this research is set to benefit many thousands of children and young people.

Matthew’s story

Matthew, picture above, has autism and was one of the children who took part in the SEE project. His mum Julie says: “The project was invaluable to us. Although we knew Matthew had vision problems from an early age, it wasn’t until the SEE project that we got a really accurate prescription for him and this was really the first time that he started to wear his glasses regularly.”

Being seen at school, in an environment he’s comfortable in was, says Julie, crucial for Matthew. “It was fantastic for an autistic child, as Matthew can be very fearful. We had both previously found eye tests very stressful. This really helped take that pressure off,” she says.

Matthew had also previously been very reluctant to wear his glasses. He would cry when his teachers asked him to. But following the detailed advice provided by the SEE project, school staff worked as a team to encourage him. They developed a simple daily routine, where they would hold Matthew’s hand, so he couldn’t take his glasses off, and take him for a walk up and down the corridor. He now looks for his glasses as soon as he wakes up.

“Wearing his glasses means he can concentrate better and see what he needs to without squinting his eyes,” says Julie.

“Matthew can now concentrate better and see what he needs to without squinting his eyes”

Matthew’s mum, Julie

Your support has helped pave the way for dedicated eyecare services to be delivered in special schools across the UK, making a real difference for children with learning disabilities. Find out more about other research successes action.org.uk/successes
Lockdown Lottery win

Congratulations to Victory Osuagwu who won an amazing £4,000 in our Rollover Jackpot back in May!

The mum of three and one our nation’s keyworkers, working in her local supermarket, was happily surprised: “I wasn't expecting it at all. It made my day! I joined the lottery because the research Action funds helps to give vulnerable and sick children a fighting chance, changing their lives for the better,” she says.

It’s easy to enter our FIGHT BACK Friday Weekly lottery. From just £1 a week, you can help us save lives and be in with a chance of winning cash prizes every Friday. And if you're already playing, don’t forget to check online each week to see if you’re a winner! Terms and conditions apply. Find out more at fightbackfridaylottery.org.uk

Introducing Team Elijah’s Star

We’re very excited to have a team taking on The Talisker Whisky Atlantic Challenge in December 2021. This is the premier event in ocean rowing, covering more than 3,000 miles, east to west from the Canary Islands to Antigua.

Team Elijah’s Star will attempt to make the epic crossing in 37 days or less to honour the memory of little Elijah Halse, who was born very prematurely and lived for just 37 days. The team is made up of four friends, existing Action supporters Philip Bigland and Dean Frost, plus Kevin Watkins and Mac McCarthy – all have a love of the sea and time spent in the forces and hope to raise as much money as possible for Action.

Brilliant business supporters

We are incredibly grateful to those companies who’ve continued to support us during this difficult time. Among them are Liberty Specialty Markets, who have generously given £10,000 to our COVID-19 appeal. This is in addition to their usual annual support, and also the significant fundraising undertaken by their team of marathon runners each year. Plus Brit Insurance have also supported both our emergency and COVID-19 appeals.

Perfect partners

Our two-year partnership with accountancy firm Price Bailey has raised more than £107,500 for our BORNTOO SOON campaign to fight premature birth – breaking the firm’s previous record for charity fundraising.

The support we’ve received from across the company has been astonishing – from cycling to Paris and trekking the Three Peaks, to two hugely successful charity days and a charity ball, to name just a few events! Action could not be more grateful to all the Price Bailey staff for getting behind our cause over the last two years and helping us to invest in future research.

Lockdown legends rise to the challenge

With so many of our usual events postponed or cancelled, we are hugely grateful to those supporters who refused to let lockdown get in their way – you are all amazing!

Eve’s excellent 2.6 challenge

One of our youngest lockdown fundraisers was six-year-old Eve Urquhart. She did the 2.6 Challenge, completing a ‘to do list’ of 26 things – including both walking and cycling 2.6 miles, chatting to 26 friends on Facetime and building a tower 26 cubes high. She smashed her fundraising target raising more than £600. Eve said: “Last year my mummy ran the London Marathon but this year because of the horrible virus no one could do it so all the children’s charities won’t get as much money to help them. So I wanted to help.”
Join the peloton to Paris!

If you thought you’d missed the chance to ride alongside a former Tour de France yellow jersey holder, your luck is in! Former pro rider Sean Yates has confirmed that he will join cyclists on our London to Paris bike ride next year – just as he’d planned to this year. Sean will ride with us for the final stretch into the French capital. The ride will take place 14 to 18 July 2021.

We’re offering Touching Lives readers 50% discount on the registration fee (usually £145) until 31 October, using the code TL50.

Register today with confidence — we don’t want anything to hold you back from signing up to your next Action event! So we want to reassure you that if we cannot go ahead with the ride in 2021 due to COVID-19, we will offer you a money back guarantee. Sign up now at action.org.uk/L2PTL.

Take Big Steps for Tiny Lives this October

Get active for Action this autumn in our new virtual event for all.

This October we challenge you to join our fight for tiny lives by running or walking 20, 40 or 60 miles to raise funds for medical research for babies and children.

It’s free and easy to take part. Just choose your distance and fundraise at least £20, £40 or £60 depending on your distance chosen. If you’d like to push yourself further that’s fine by us!

Cover your distance wherever you like and at your own pace between 1-31 October and log your progress on your own fundraising webpage.

Every step counts, so why not get all the family involved? Sign up now at bigsteps.action.org.uk