

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

Re:action

Spring/summer 2021

action **medical research**
for children



In your latest *Re:action*

Fighting cystic fibrosis

Preventing premature birth

Funding new research into COVID-19

Welcome

At Action Medical Research we're on a mission to save and change children's lives – seeking answers that can lead to cures, treatments and medical breakthroughs.

In this issue, find out about some of our latest research projects, including new studies to better understand the effects of COVID-19 on children. 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 around 60 groundbreaking research projects

action.org.uk/research

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Bringing hope to families

Despite the challenges of the past year, we are really pleased to have achieved so much for sick babies and children thanks to your continued support.

We were able to fund eight new research projects last year and have just announced another 10 that we're funding this year. These include five new studies to better understand the impact of COVID-19 on babies and children, which you can read more about on pages 4-5.

Our continued partnership with British medical research charity LifeArc means we're funding over £1m of research to help children with rare diseases.

We also continue, with your help, to fight for babies born too soon and to shine a spotlight on the devastation caused by premature birth.

We're looking forward to a vibrant summer and autumn of cycling, challenge and social events, raising funds for vital medical research to save and change children's lives.

We hope you'll join us!

My Cream Tea

We've taken the decision to postpone our Action Cream Teas in a box for 2021.

But if you still want to enjoy a scone or two, we'd love you to get baking and host your very own cream tea!

Find out more action.org.uk/mycreamtea



Protecting babies and children from COVID-19

Thanks to your support, we're funding five new research projects across the UK to help better understand the impact of COVID-19 on babies and children. This work is vital to ensure they are not forgotten in the fight against the virus.

COVID-19 can affect children, sometimes with devastating consequences, and there is much still unknown about the risks in pregnancy and to babies. That's why last summer we launched our COVID-19 children's research appeal – to fund vital research that is now getting underway.

Identifying risk factors

While children are generally less affected, some sadly develop severe COVID-19 and become critically ill with a rare condition known as Paediatric Multisystem Inflammatory Syndrome (PIMS). These children can face lasting problems that impact their health and wellbeing.

Based at the University of Cambridge, Dr Nazima Pathan aims to identify biological factors that affect a child's risk of becoming very ill as a result of COVID-19. This could lead to earlier and more personalised treatments to help give these children the best possible outcome.

This research also expects to uncover new knowledge about why children are generally better protected from the virus than adults – information that could help now and in fighting future pandemics.

Infection in pregnancy

We're also funding new research that could inform how to protect pregnant women and their babies from any potential risks from the virus, both now and in the future.

“ We hope to identify risk factors that can affect disease severity and outcomes in children “

Dr Nazima Pathan

It's thought that thousands of pregnant women in the UK are likely to have been infected with the virus that causes COVID-19, many with no symptoms.



istock.com/KatarzynaBialasiewicz

Professor Kirsty Le Doare, at St George's University of London, is leading a study that aims to screen women from hospitals across England to identify how many have been infected – and whether the virus or protective antibodies are passed from mother to baby during pregnancy, delivery or breastfeeding.

“We hope that our findings will help to reassure pregnant and breastfeeding women about how to safely care for their babies. It will also help inform future

decision-making about vaccinating pregnant women to help protect them and their babies from infection,” says Professor Le Doare.

[Read more about this and other new COVID-19 research at action.org.uk/tacklingCOVID](https://action.org.uk/tacklingCOVID)

Your support makes research happen

Thanks to supporters like you, we've been supporting research to develop breakthrough treatments, vaccinations and cures for children for nearly 70 years – and our response to this pandemic is no different.

action.org.uk/COVID-19



Living with cystic fibrosis



“For us, research is a real lifeline. It gives us hope”

Sophia's daily life revolves around physiotherapy, treatments and medications. The constant risk of infection damaging her lungs has also made the COVID-19 crisis an especially scary time.

“When the midwife phoned to say newborn screening had raised some concerns, she asked me, is your partner in?” says Sophia's mum, Sarah. “I knew then that something was seriously wrong.”

Sophia's diagnosis with cystic fibrosis at just two weeks old was, says Sarah, a horrendous shock: “It was a strange kind of grieving. No one had died, but you are mourning a life you thought was going to be, while coming to terms with the reality that you may outlive your child – and you're feeling all these things whilst holding your tiny baby.”

Children with cystic fibrosis experience a range of symptoms including a persistent cough, shortness of breath and frequent chest infections, caused by sticky mucus clogging their lungs and airways.

Medications and physiotherapy can help but sadly there is currently no cure.

Sophia is now a spirited and curious toddler but her condition requires constant care.

Knowing the risks posed by any infection, the start of the COVID-19



pandemic last year was a terrifying time. Nobody knew how the virus might affect children with cystic fibrosis. Sophia and all the family, including older brother Tom, had to shield for many months. “We only felt safe in our house,” says Sarah.

Thankfully, Sophia is well most of the time but the impact of cystic fibrosis on family life is enormous. “You have to discover a new normal,” Sarah says. “It's all about managing risk. We have, without realising it, made so many changes to our everyday life, to keep Sophia as safe as possible.”

With your support...

Cystic fibrosis is the UK's most common life-threatening inherited disease. Action funding is helping researchers develop new ways to prevent lung damage and

fight infections that are dangerous for children with cystic fibrosis.



You're helping make this research happen. Find out more action.org.uk/cysticfibrosis



Making children like Ian smile

Nine-year-old Ian loves being active. But with cerebral palsy affecting one side of his body, he faces difficulties with muscle strength, stiffness, coordination and balance. RaceRunning, a sport for people with disabilities, has had a big impact. Ian enjoys regular coaching sessions through a pilot study made possible with Action funding and his potential as an athlete is shining through.

This research, funded with the Chartered Society of Physiotherapy Charitable Trust, is investigating the health benefits of RaceRunning. These could last long into the future, benefiting children's everyday mobility and quality of life, plus reducing the risk of poorer health when they grow up. Ian's mum Sheena says:

“ RaceRunning gives children physical independence and a sense of achievement. Their faces light up ”

Find out more: action.org.uk/lanstory

Developing a cure for a rare disease

Your support has helped drive forward research to develop gene therapy for XLP, a life-threatening rare immune system disorder.

In 2003, David and Allison Hartley were told that all four of their sons had XLP. Joshua was then 12, Nathan 10, Daniel eight and Luke just four.

X-linked lymphoproliferative disease, or XLP, is an inherited condition that usually only affects boys. While very rare, it can have devastating consequences as the immune system is unable to function properly. Affected boys suffer recurrent infections, a third develop a type of cancer called lymphoma and many develop a life-threatening immune system over-reaction, which can be fatal.

Bone marrow transplants were, and currently still are, the only cure. Without them, David and Allison's boys were unlikely to survive their teenage years. "It was numbing," recalls David. "Nothing could have prepared us for the devastating news."

The diagnosis saw the start of a high-profile, worldwide, race-against-time hunt for four suitable bone marrow donors. This was followed by years of tests, operations and hospital stays.

More options to treat this disease are urgently needed.

“This work would not have happened without Action funding”

How research helped

Between 2013 and 2020 Action awarded two research grants to Professor Bobby Gaspar and Dr Claire Booth at the UCL Great Ormond Street Institute of Child Health. The second of these was co-funded with Great Ormond Street Hospital Children's Charity. The research team wanted to develop a new gene therapy to restore immune system function in boys with XLP.

Gene therapy uses the child's own cells, so there's no need to find a donor. There's also less need to suppress the immune system to avoid post-transplant rejection and other complications. The ultimate



hope is that this will allow affected boys to live normal lives, without needing a bone marrow transplant.

“We're not just trying to make these children a little bit better. We are trying to cure them,” says Professor Gaspar.

The new treatment uses T cells, a type of white blood cell, and the Action-funded research has shown it can work. Now the next step is to transplant corrected cells into patients in a clinical trial.

Without treatment
70% of boys
with XLP lose their
lives by age 10

Thankfully, all four of David and Allison's boys eventually had successful bone marrow transplants but their treatment was long and gruelling. While Nathan and Daniel made full recoveries, Joshua and Luke have suffered severe post-transplant complications.

With Action funding, the team at UCL have taken a big step forward since the Hartley's faced their heartbreaking diagnoses, and it's very exciting that clinical trials for this new gene therapy are now so near.

THANK YOU!

You've helped support the development of a vital new treatment, with a trial in patients due to start soon. [action.org.uk/successes](https://www.action.org.uk/successes)



iStock.com/cdwheatley

Improving pregnancy care for cancer survivors

Doctors suspect that women who had a bone marrow transplant as a child face a higher risk of pregnancy complications and premature birth. New Action funded research is investigating this further, with the aim of improving care in the future and reducing the risk of babies being born too soon.

Thanks to advances in treatment and fertility technologies like IVF, more childhood cancer survivors than ever before are reaching adulthood and starting families of their own. But sadly it's believed that women who've had a bone marrow transplant are more likely to experience problems during pregnancy – especially if they also had radiotherapy to their womb area. This includes a high risk of having their babies very early.

Dr Melanie Griffin, at University Hospitals Bristol NHS Foundation Trust, is using data from population studies to study pregnancy complications experienced by women who had a bone marrow transplant when they were younger.

She and her team will also look at what care is currently given to affected women and develop recommendations for the future.

“If we can prove that these women have an increased risk of pregnancy problems, they could be offered specialist care before and during pregnancy to reduce the risk of their babies being born too soon,” says Dr Griffin.



This research is jointly funded in partnership with Borne and with generous support from The James Tudor Foundation.



Cycling for Little Lives

Lockdown saw Adrian Orban rediscover cycling and he's signed up for our London to Paris event. He's riding for a reason – his daughter Sonia was delivered prematurely and sadly her twin sister, Sophia, didn't survive.

"I will never forget April 28 of 2016," recalls Adrian. "My wife, Amelia, called to tell me that doctors needed to start emergency birth procedures. It was a twin pregnancy, but they could only hear one heartbeat."

Amelia was already in the operating theatre when Adrian arrived at the hospital. Sonia was delivered safely, four weeks early, but sadly Sophia hadn't survived. Sonia needed specialist care but thankfully recovered well from her traumatic start. She is about to turn five and, says her proud dad, is full of energy and extremely sociable.

It was the first lockdown last year that saw Adrian get back into cycling. Finding himself furloughed, he bought a bike trailer and took Sonia and her younger brother Luca out every day.

"Seeing all those babies in incubators was something I never thought I'd see in front of my own eyes," says Adrian. "Now I want to raise as much as possible to help others."

Feeling inspired? See back cover for more events news – or go to action.org/events



Meet Team Elijah's Star

Embarking on an epic challenge to row across the Atlantic Ocean in 37 days for babies born too soon.

Elijah's Star is a four-man crew aiming to raise £100,000 for Action as they set their sights on completing the Talisker Whisky Atlantic Challenge this December. The 3,000-mile row will see them battling waves of up to 20 feet in a 28 x 5-foot boat, pushing themselves to the limit by rowing and sleeping in continuous two-hour blocks.

The team is rowing in memory of baby Elijah Halse, who was born at 25 weeks and three days, weighing just 823g, and lived for only 37 days.

The crew 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.

Dean says: "We are taking on this epic journey to shine a light on the impact of premature birth and to encourage others to support Action. Financial support is great and donations can be made through our website or through



Action's. There's also the opportunity to spread the word, share your own premature birth story, attend events and cheer us along on social media."

[Find out more at elijahsstar.com](https://elijahsstar.com)



Challenge Events



Cycling

RIDE Castle, Kent 9 May

RIDE Suffolk 30 May

RIDE Davina's Big Sussex Bike Ride 13 June

RIDE Essex 5 Sept

RIDE Vyking, York 19 Sept **NEW**

Maratona dles Dolomites - Enel 4 July **SOLD OUT**

London to Paris 14-18 July



Running

Vitality London 10,000 31 May

Virgin Money London Marathon

3 Oct or 24 April 2022

Team challenges

Race the Sun, Dawn to

Dusk Triple Challenge:

Brecon Beacons 12 June **SOLD OUT**

Lake District 11 Sept **SOLD OUT**

Lake District 18 Sept **EXTRA DATE**

Trekking

Snowdon at Night 18 Sept and 16 Oct

National Three Peaks Challenge various dates

Virtual events

BIG Steps for Tiny Lives, plus more see action.org.uk/virtual



With so much uncertainty at the moment, let us reassure you with our **Action Guaranteed, money-back-promise**. Should an event be cancelled due to COVID-19 your registration fee will be fully refundable.



For event details and to register visit action.org.uk/events