

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

Re:action

Autumn/winter 2021

action medical research
for children

In your latest *Re:action*

Fighting a rare brain disease

Asthma drug breakthrough

Helping vulnerable babies

**STRONG
GIRLS
CLUB**



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 studies to help babies in special care and 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.

In this edition...

- 3 Action update** – Here for children, thanks to your support
- 4 Taking Action** – COVID-19 research update
- 6 Powering research** – Fighting a rare brain disease
- 8 You made it happen** – Asthma treatment breakthrough
- 10 Making an impact** – Research to save lives in twin pregnancies
- 12 New research** – Improving care for premature babies
- 14 Supporters in Action** – Hannah's Big Steps for brother Joey
- 15 Action events** – Get on your bike or Race the Sun
- 16 Weekly Lottery** – Our £5,000 winner!

Find out more!

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

action.org.uk/research

Follow us



@actionmedres



@actionmedres



@actionmedres

Editor Clare Airey **Design** Paige Bowring-Martin

Print Propack Direct Mail **Cover photo** supplied by Beatrice's family

Registered Office Vincent House, Horsham, West Sussex RH12 2DP

T 01403 210406 E info@action.org.uk W action.org.uk

© Action Medical Research 2021 Action Medical Research is a registered charity:

England and Wales no. 208701; Scotland no. SC039284



Here for children

As a new school year begins and we head into autumn, we're delighted to have enjoyed a more normal summer of fundraising. Thank you for all your support.

With so many events put on hold last year, it has felt very special to be able to fundraise together again. The sun shone beautifully for many of our challenge events – from our RIDE series of bike rides to our Race the Sun dawn to dusk triple challenge.

Thanks to all who've supported us in any way. In a world that's ever changing, our mission to save and change children's lives holds strong – but we can't do it without you.

As the pandemic has shown, we cannot know what the future holds but with your help we continue to raise funds to support vital research for children and their families.

Good luck Team Elijah's Star

In December, the crew embark on their epic journey, rowing 3,000 miles as part of The Talisker Whisky Atlantic Challenge. Shining a spotlight on premature birth, they aim to raise £100,000 for Action. Follow their progress on Facebook or Instagram at /elijahsstar



Continuing the fight against COVID-19

While the success of vaccines has enabled the easing of restrictions, the pandemic is far from over. Thanks to your support, research into how the virus affects babies and children continues, and soon we'll be funding further work to help children with long COVID.

There remain many unanswered questions around how the virus affects children. So we're pleased to share news on some of the projects funded by our COVID-19 children's research appeal, including one that addresses one of the biggest unknowns – why do children respond differently to the virus than adults?



Dr Marko Nikolić and Dr Kerstin Meyer hope to explain this and discover why those children who do become infected are more susceptible. This could lead to new approaches to prevent or reduce serious illness – and help inform public health measures.

Babies and COVID-19

We're also funding research to assess the risk the virus might pose to babies – and whether it could affect the developing brain. Dr Ela Chakkarapani and his team are assessing children exposed to the virus in the womb or shortly after birth, against those who were not. This could help shape advice for pregnant women and new parents, and identify children who may need additional checks and support.

Treating cancer in a pandemic

Further research is looking at the wider impact COVID-19 has had on children's cancer treatment. Focusing on children diagnosed with a brain tumour during the pandemic, Dr Ibrahim Jalloh and Professor Rachel Isba hope their work will inform guidance for managing cancer care and other specialist services during any future periods of disruption.

At the forefront of vaccine development

We're incredibly proud that the leader of the Oxford/AstraZeneca vaccine trial was a former Action Research Training Fellow, Professor Sir Andrew Pollard.

Vaccines are playing a crucial role in the fight against COVID-19 and Professor Pollard has received a knighthood in recognition of his services to public health. But while his recent focus has been on a vaccine to primarily protect adults, most of his career has been spent preventing and treating diseases that threaten children, particularly meningitis.

It's a career that Action is delighted to have supported, funding a Research Training Fellowship back in 1995. "This was critical because it was my research training. It gave me a

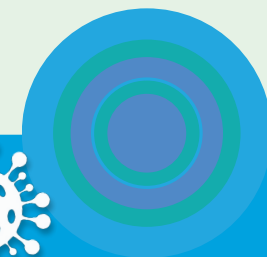


portfolio of understanding and skills which I continue to use today," says Professor Pollard.

With your support, our Research Training Fellowship scheme helps fund future leaders in medical research – people like Professor Sir Andrew Pollard, whose work has helped save millions of lives.

The last year has given a stark reminder of the huge difference medical research can make. The COVID-19 projects we're funding could lead to important answers that could help now and help fight future pandemics. This research would not be possible without your support. **Thank you.**

action.org.uk/COVID-19



Fighting a rare brain disease



“Research is so important. Being able to predict outcomes for a disease where there are still many unknowns would have helped us so much”

Aged just eight and previously healthy, Beatrice suffered the terrifying effects of autoimmune encephalitis, which caused her to lose the ability to walk or speak.

In May 2019, Beatrice's mum Amy received a call to say that her daughter had become unwell at school. Beatrice had suffered a seizure and was rushed to hospital. Doctors ruled out a brain tumour and arranged further tests for epilepsy. But over the following weeks, Beatrice was not her usual bubbly self. She barely slept, became withdrawn and didn't want to talk or eat.

Things then took a greater turn for the worst, and Beatrice was rushed to the Evelina London Children's Hospital. She had started making nonsensical speech sounds, unable to say actual words. "We knew something was seriously wrong," says her dad, David. "By the time we arrived, she'd become non-verbal and lost all control of her body," adds Amy.

Beatrice was diagnosed with NMDAR-antibody encephalitis. This is caused by the immune system mistakenly attacking the brain. While most children survive, recovery is usually slow and many are left with lasting difficulties.

Beatrice was unable to speak, walk or control her body, and needed a feeding tube. She spent several months in hospital before beginning to regain her skills. "We were so very proud of her recovery, but so little was known about what would happen next," says David. "We were very worried and uneasy."

Thankfully, with lots of support, Beatrice recovered well. But sadly this isn't always the case, and that is why research is so important.



With your support...

Dr Michael Eyre at King's College London is investigating if advanced early brain scans can identify vital clues to improve treatment for NMDAR-antibody encephalitis. It is hoped this will help doctors predict likely outcomes, select the

best possible treatment for each child and reduce the long-term impact on children's lives.

This Research Training Fellowship is co-funded with the British Paediatric Neurology Association.



Breakthrough in asthma treatment

With your support researchers have shown that prescribing medication based on a young person's genetics could treat severe asthma more effectively.

In a landmark trial, Professor Somnath Mukhopadhyay and his team used a simple saliva test to determine differences in the genetic make-up of patients aged 12-18. They then prescribed one of two drugs accordingly.

This approach is known as personalised treatment and those who received it showed greater improvements to their quality of life.

Asthma is the most common chronic medical condition in children in the UK, so this work could go on to benefit many thousands here, and many, many more around the world.

“ I hope that the results of this trial will have globally significant implications ”

Professor Somnath Mukhopadhyay

This project was also supported by The Henry Smith Charity.

Saving tiny twins

Thanks to your support, researchers are now trialling a new procedure that could be safer than the current treatment for twin-twin transfusion syndrome, helping to save more tiny lives.

Twin-twin transfusion syndrome affects around one in 10 identical twins who share a placenta. Abnormal connections develop in the shared placenta, connecting the babies' blood supplies. This causes unequal sharing of blood between them and can be life-threatening.

Severe cases are usually treated by laser ablation surgery. This is keyhole surgery, with a small laser inserted through a cut in the womb and used to block the blood vessels causing the problem. But it carries risks, many of them due to its invasive nature. Sometimes it can still only save one twin, and sometimes neither baby survives. There is also a risk of infection, miscarriage or premature birth.

How research helped

In 2012, Action awarded funding to Professor Christoph Lees and his

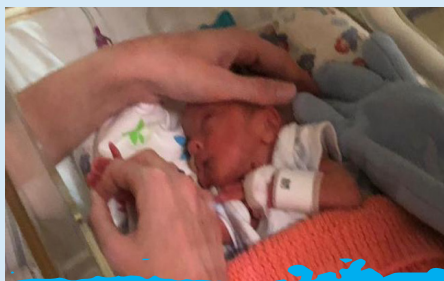
team to investigate the safety and effectiveness of a new technology called high frequency ultrasound. "As it does not involve invasive surgery, we believed it could be safer," he says. "It could also mean babies could be treated earlier in pregnancy, hopefully improving their chances of surviving and escaping disability."

The technique uses a focused ultrasound beam, generated outside the body and aimed precisely at a treatment area inside the womb. It is already used in the treatment of uterine fibroids and to kill tumours – and now could be used to block blood vessels in the placenta.

"The results from the Action-funded work showed that our new technique appears to be effective – and, importantly, has few side effects," says Professor Lees.

A clinical trial in pregnant women opened this year, now taking this even closer to becoming a treatment option.

Sadly around **300** UK babies die each year as a result of this syndrome



Katie's story

In 2018, Katie and husband Fred were overjoyed to discover they were expecting identical twins. But by 19 weeks the babies had developed severe twin-twin transfusion syndrome.

"With immediate effect, we needed to make a life-or-death decision which could compromise both of our babies," recalls Katie.

Katie chose to have laser surgery: "You do it because there's a chance you might save your babies," she says. "But having something pushed into your pregnant stomach is terrifying."

Sadly, despite the treatment initially seeming to have gone well, Katie and Fred later lost their smaller twin, Faith, due to the damage twin-twin transfusion had caused. Katie's waters then broke at 28 weeks, and Constance was delivered weighing just 2lb.



Thankfully Constance made good progress, but having endured such a traumatic pregnancy, Katie found the intensity of caring for such a tiny, vulnerable baby almost overwhelming.

For Katie, the non-invasive nature of the new treatment is key. "It would be so beneficial for the patient," she says. "And while laser treatment maybe did save Constance, she was delivered at 28 weeks, most likely due to the previous procedures – so preventing this would make a huge difference in the future."

THANK YOU!

You've helped support the development of a vital new treatment that could save babies' lives. Find out more about other research successes action.org.uk/successes



Studying sleep cycles in premature babies

Premature babies are cared for in an environment quite unlike the womb. Researchers believe this may interrupt natural sleep cycles, in turn affecting brain development. They are testing this theory, with the aim of improving care.

Advances in treatment have led to improved survival, but babies born prematurely still face an increased risk of long-term neurodevelopmental complications. There are many reasons for this, but there is evidence to suggest an important relationship between babies' sleep cycles and healthy brain development.

In the second half of pregnancy, a baby's brain undergoes rapid development, forming new connections and networks. At the same time, distinct sleep states emerge, with babies cycling between so-called 'active' and 'quiet' sleep. For babies born too soon, these sleep cycles may be interrupted by medical procedures, bright lights and loud noises.

Thanks to your support, Professor Topun Austin and his team, based at Cambridge University Hospitals NHS Foundation Trust, are investigating this further. Using a non-invasive imaging system – a lightweight, flexible cap, fitted with sensors – they are studying changes in blood flow in different parts of the brain. They are looking for differences between active and quiet periods of sleep, and between pre- and full-term babies.

“Ultimately, we hope to develop a new system for use in neonatal units that can help to promote sleep cycling and support healthy brain development in these vulnerable babies,” says Professor Austin.



Taking Big Steps for Tiny Lives

We're so proud of Hannah, who's raised more than £700 by taking part in our Big Steps for Tiny Lives virtual event, inspired by her big brother Joey who survived brain cancer.

Joey was just four years old when he was diagnosed with a brain tumour. It was a terrifying time for the family but thankfully his treatment was a success.

Joey was treated under the care of Professor Andrew Peet, whose work to improve treatment for children with brain tumours has been helped by Action. So when Hannah decided she'd like to raise money for charity, we were a natural choice.

Joined by family and friends, including Joey, now 15, and Bessie the Springer Spaniel, Hannah clocked up 35 miles over three weeks!

"It was lovely to see how much support Hannah received, which far surpassed her original target," says her mum, Kathryn.

"Action's research can lead to treatments and medical breakthroughs for some of the toughest fights children face. As a family we know too well how hard that fight is. We're blessed that we are a complete family and truly hope that our contribution will in the future help more families like ours to stay complete."



Big Steps for Tiny Lives continues this Autumn. You can take part in your own time and at your own pace. Sign up now at bigsteps.action.org.uk

Take on an Action challenge

Race the Sun 2022

After a phenomenal year in 2021, which saw two new events due to unprecedented demand, Race the Sun will return in 2022. Teams of four compete against each other, racing the setting sun, to be crowned champions in a dawn to dusk triple challenge – cycling, hiking and canoeing or kayaking around courses in the beautiful Brecon Beacons and Lake District National Parks. [Find out more action.org.uk/race-sun](https://action.org.uk/race-sun)



Come along for the ride!

Our RIDE Series of one-day cycling sportives returned this summer and we've exciting plans for next year. Some of our long-established and ever-popular events will be expanding, with new routes, including off-road gravel options. Plus there'll be some brand new rides added to the series. [For latest details see action.org.uk/RIDE](https://action.org.uk/RIDE)

With cycling, running, trekking and team challenges, we've events to suit all. We'd love you to join us!

See website for dates and details, action.org.uk/events or scan the QR code with your phone camera.





£5,000
Rollover Jackpot
WINNER!
David, from Lincoln

Next time, it could be you!

In June our **FIGHT BACK** Friday Lottery rollover prize reached **£5,000** which meant it had to be won.

The lucky winner was David from Lincoln, who now plans to treat his wife to an extra special holiday. David says: "I was very surprised. I support Action because I can see that their work really does change people's lives, more importantly those of children."

If you're not already playing our **FIGHT BACK** Friday Lottery, then sign up today! It costs just **£1** a week and you could be in with a chance of winning **£500** every Friday or up to **£5,000** in our Rollover Jackpot.

Find out more at **fightbackfridaylottery.org.uk** or scan the QR code with your phone camera.



Ts & Cs apply