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Phage therapy offers new hope for children with cystic fibrosis

In a major advancement for personalised medicine, The Children's Hospital at Westmead has launched a world-first clinical trial investigating the use of phage therapy to treat a chronic bacterial infection in children with cystic fibrosis (CF).

Funded by the Cure4 Cystic Fibrosis Foundation (Cure4CF) in collaboration with Sydney Children's Hospitals Foundation, the trial will use bacteria-eliminating viral treatments called bacteriophages (phages) to treat children with CF who have chronic *Pseudomonas aeruginosa* infection.

The phages will be manufactured locally at the Westmead Institute for Medical Research, part of the Westmead Health Precinct.

Minister for Medical Research David Harris has welcomed the trial which is being supported by a \$3.5 million NSW Government funding boost to manufacturing capacity.

"It's pleasing to see the recent investment making a meaningful difference by supporting this very important clinical work," Mr Harris said.

Pseudomonas aeruginosa is a common bacteria found in places such as showers and swimming pools. For healthy individuals, the bacteria ordinarily have no lasting impact; but those living with CF are at greater risk due to impaired mucus clearance in their lungs, with almost one in four children living with CF infected by this bacteria.

Currently, the only available treatment is prolonged and repeated courses of strong antibiotics. However, the bacteria often develops a protective film that enables it to evade antibiotics and the body's natural defences, making it extremely difficult to treat.

The ongoing use of such antibiotics can also lead to hearing impairment and kidney disease and is not always effective in treating the infection.

Dr Jagdev Singh, Paediatric Respiratory and Sleep Consultant at The Children's Hospital at Westmead, is leading the trial, with his PhD supported by Team Simon and said phage therapy offers a new way to directly target the infection.

"Phages are specific and specialised viruses that can be exactly matched to eliminate bacteria without harming human cells. If successful, this could offer a potentially lifesaving treatment for children with CF who have a *Pseudomonas aeruginosa* infection, helping to alleviate dependence on antibiotic use and as a result, reduce hospital visits due to infection," Dr Singh said.

The clinical trial is the first major step, both locally and internationally, in demonstrating the suitability of phages as a routine treatment for chronic *Pseudomonas aeruginosa* bacterial infection in children with CF.

"*Pseudomonas aeruginosa* infections increase the risk of death for children with CF by almost nine times, with the infection often meaning these children aren't eligible for lifesaving lung transplants due to the risk associated with post transplant infection."

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“Current treatment involves strong antibiotics but the bacteria can become resistant to medication over time. The unique advantage of phages is that they can adapt as the bacteria evolves, meaning we have more chance of effectively treating and stopping the infection altogether.”

Unique to this trial, phage therapy will be delivered through the lungs via bronchoscopy and nebulisation, giving children the ability to receive future treatment at home and reduce time spent in the hospital.

Suzy Dimaline, Cure4CF CEO, said it is fantastic to be contributing to potentially groundbreaking research.

“This clinical trial could offer a potentially lifesaving treatment for young people with cystic fibrosis and we are so proud to be working alongside Dr Singh and his team at The Children’s Hospital at Westmead to support this vital research,” Mrs Dimaline said.

“Through this trial, we are truly seeing the impact fundraising has not only in driving transformational research but also in changing the care that can be provided to young people living with CF for the better.”

Dr Singh added, “We couldn’t do what we do without the support of communities and organisations like Cure4CF and Team Simon. Their funding has been integral to this trial and we are incredibly grateful for their continued and generous support.”

Phase one of the Cure4CF personalised phage treatment of *Pseudomonas aeruginosa* for children with cystic fibrosis clinical trial will involve approximately ten children. If successful, the trial will then expand nationally and internationally, before furthering the treatment to target other bacteria that causes lung damage in patients with CF.

Case study story attached. Images can be downloaded here: <https://we.tl/t-acwSdel9uu>

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About Cystic Fibrosis

Cystic fibrosis (CF) is the most common inherited disease in the developed world, and it’s very expensive to treat. This is because it affects multiple organs including the lungs, gut, liver, pancreas and reproductive tissues – and each relies on a different doctor or specialist.

CF creates a build-up of fluid in the lungs and is often described as feeling like never having enough breath. There is currently no cure.

Due to ongoing infections, tissue damage and lung disease, people with CF have a severely shortened life span. 1 in 25 people with Caucasian ancestry carry the defective CF gene without

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care, advocacy, research, education

knowing it. And if both parents are carriers, there's a 25% chance of their child being born with the disease.

About Sydney Children's Hospitals Network

Sydney Children's Hospitals Network (SCHN) is the largest paediatric healthcare entity in Australia and cares for thousands of children each year both in hospital and in their homes – with one purpose in mind – to help children and young people live their healthiest lives. SCHN brings together the advanced facilities, expertise and services of The Children's Hospital at Westmead, Sydney Children's Hospital, Randwick, the Newborn and paediatric Emergency Transport Service (NETS), Bear Cottage and Kids Research to provide the very best care for sick children and their families. With more than 8,000 staff, the Network is committed to providing world-class paediatric health care, furthering ground-breaking research into the causes, treatments and cures of childhood illnesses and investing in education and training of the health workforce to advocate for a healthy future for children in NSW and beyond.

About Dr Jagdev Singh

Dr Jagdev Singh is a paediatric respiratory and sleep consultant at The Children's Hospital at Westmead. He has spent 15 years treating and caring for children in various fields in paediatrics which include; infants (neonatology), critical medicine, endocrine, retrieval medicine, general, rural, and developmental paediatrics in different parts of Australia and Malaysia before finding his true calling in paediatric respiratory and sleep medicine.

Dr Singh is a researcher and a clinical lecturer with the University of Sydney. He has delivered various talks and teaching sessions for GPs, paediatricians and trainees in the field of respiratory and sleep medicine and has won numerous awards and nominations in the field of teaching and research. More importantly, Dr Singh is devoted to teaching and empowering trainees, GPs and aspiring paediatricians in the realms of respiratory and sleep medicine. In addition to performing his own research, Dr Singh also supervises and guides junior researchers in the field of paediatrics.

About Cure4 Cystic Fibrosis Foundation

Since 2009, Cure4CF has funded over \$5 million in cure-focused research. It is the largest private funder of cystic fibrosis research and development in Australia. Cure4CF believes that cystic fibrosis is solvable problem, and that research is the answer.

The organisation exclusively funds research into therapies and treatments that will either cure cystic fibrosis or significantly extend the life expectancy of people living with cystic fibrosis. Donations to Cure4CF will help to increase the volume of research that the organisation can fund.

About Sydney Children's Hospitals Foundation

Sydney Children's Hospitals Foundation is all in for kids' health. We exist to promote charitable investment in the prevention and management of childhood illness, with a focus on kids and families receiving treatment or care in services provided by NSW's network of children's hospitals and paediatric health services. One of the largest and most trusted kid's health charities in the country, with one foot at the front line and one in the future of kids' health, we make sure funds raised go directly to support children's healthcare - from the hospital wards to the treatment rooms, the research labs to the outreach programs - to help all sick kids, no matter where, no matter what. www.schf.org.au