



Monday, 4 May 2009

## Major Breakthrough for People with Cystic Fibrosis

The Children's Hospital at Westmead will today announce the results of a clinical trial where they have seen a major therapeutic advance into Cystic Fibrosis (CF), a major cause of shortened life span in young people in Australia. Despite extensive research by many groups, there have been no new drugs specifically registered for CF in the last ten years.

The trial has been sponsored by Australian based pharmaceutical company Pharmaxis. It has compared Bronchitol, a novel mucus clearance agent invented and developed in Australia by an Australian company, on lung function and quality of life for children and adults living with CF. Bronchitol is a dry powder that works to break the vicious cycle of impaired clearance, infection, inflammation and lung damage that is present in patients who have CF.

The clinical trial was successful, meeting both its primary and key secondary endpoints comfortably. Patients taking Bronchitol had a consistently better lung function than those taking placebo. The improvement reached a level of 6.6% compared to that measured at the start of the study. The effect commenced early in the study and was sustained for the full 6 months patients were receiving drug. Furthermore Bronchitol was effective in improving lung function in trial participants whether or not they were taking the most commonly prescribed existing treatment for cystic fibrosis, Dornase alpha. If approved Bronchitol will be among the first inhaled dry powder products registered for cystic fibrosis and offers patients a treatment option with less drug administration time and the equipment maintenance issues associated with nebulised liquids.

Dr Peter Cooper, Director of Cystic Fibrosis at The Children's Hospital at Westmead and lead investigator on the trial said, "The Children's Hospital at Westmead performs an important role in world class research that makes a real direct difference to the lives of our young patients. The Bronchitol trial has shown a genuine increase in lung function for CF patients using the drug. With better clearance of mucus from the lungs, CF patients are likely to live a better and longer life."

This trial is one of the largest clinical trials ever conducted in this disease. The trial has helped to improve the quality of life for people with Cystic Fibrosis such as Georgia Jones. Georgia is 11 years old and was diagnosed with Cystic Fibrosis at birth. Being involved in the trial has shown a great deal of improvement for Georgia. Sue, Georgia's mother has noticed a distinct change in her daughter, "The results in our minds, speak for themselves. For our family the results achieved were extremely pleasing to the degree when our trial period ended we made a formal request for Georgia to continue due to the benefits achieved."

CF is the most common life threatening, recessive genetic condition affecting Australian children; the average life expectancy for someone with CF is 37 years. A child is born with CF every four days. In people with CF a defective gene causes the body to produce unusually thick, sticky mucus that clogs the lungs and leads to life-threatening lung infections.

The trial was conducted across 40 centers in Australia, Ireland, New Zealand and the United Kingdom. The randomised, parallel, placebo controlled, double-blind study examined the safety and efficacy of Bronchitol in the symptomatic treatment of CF. 325 patients took part in the trial; some of the children of which were patients of The Children's Hospital at Westmead and Dr Peter Cooper. One of the largest trial sites was The Children's Hospital at Westmead.

**Media Opportunity: 10am, Tuesday 5 May, The Children's Hospital at Westmead**

Dr Peter Cooper (The Children's Hospital at Westmead) will be announcing the findings of this clinical trial.

Also available is Sue Jones (Mother of 11yo participant Georgia), Dr Alan Robertson (CEO, Pharmaxis) and Terry Stewart (CEO, Cystic Fibrosis Australia)

About The Children's Hospital at Westmead

The Children's Hospital at Westmead is the largest paediatric centre in NSW, providing the most advanced care and treatment options for children from NSW, Australia and across the Pacific Rim.

Established in 1880, The Children's Hospital at Westmead is a stand-alone public Hospital and registered charity with 3,000 staff working in 150 departments. Over 60,000 sick children and their families are cared for each year in a family-focused, healing environment. Funded by the NSW Government, The Children's Hospital at Westmead provides significant enhancements to services aided by the generosity of individual and corporate donors. For more information visit [www.chw.edu.au](http://www.chw.edu.au)

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