Benefits of cystic fibrosis drug ivacaftor reported in pre-school children for the first time

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The oral drug ivacaftor appears to be safe and could be beneficial to young children between the ages of 2 and 5 with a specific type of cystic fibrosis, according to new research published in *The Lancet Respiratory Medicine* journal. The findings also suggest a potential window of opportunity in early life when organ damage could be mitigated.

Cystic fibrosis is an inherited disease caused by a defective gene that slowly destroys the lungs and digestive system. The defective gene disrupts the activity of a protein called cystic fibrosis transmembrane conductance regulator (CFTR) which regulates salt transport in the body's cells. This causes thick, sticky mucus to build up in organs, especially the lungs and digestive system, and leads to recurring infections and irreversible lung damage. Around 10000 people are living with cystic fibrosis in the UK and more than 70000 worldwide. Currently, there is no cure.

Ivacaftor targets the basic defect of cystic fibrosis, or CFTR, seen in about 4% of cystic fibrosis patients with at least one mutation in the CFTR gene. Earlier studies confirmed the drug to be a safe and effective treatment in children aged 6 and older, adolescents, and adults with these so called "gating" mutations, and the drug has been approved for use in these groups. But, until now, no studies have examined its effects in younger children.

This trial, involving 34 pre-school children with cystic fibrosis aged between 2 and 5 years with at least one copy of a mutation in the CFTR gene, showed that taking the oral drug at one of two doses (50mg for children with bodyweight