Scientists develop first ever drug to treat 'Celtic gene' in cystic fibrosis sufferers

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An international research team led by Queen's University have developed a ground breaking treatment for Cystic Fibrosis sufferers. The new drug will benefit sufferers who have the 'Celtic Gene', a genetic mutation which is particularly common in Ireland.

The study, which was carried out by scientists at Queen's University Belfast, the University of Ulster, the Belfast Health and Social Care Trust and teams of researchers in Europe, USA and Australia found significant improvement in lung function, quality of life and a reduction in disease flare ups for those receiving the new treatment.

The drug (VX-770) is a significant breakthrough not only for those with the 'Celtic Gene', known as G551D, but also for all other Cystic Fibrosis sufferers as it indicates that the basic defect in Cystic Fibrosis can be treated. This is the first drug aimed at the basic defect in Cystic Fibrosis to show an effect. It is still too early to determine whether this treatment will improve life expectancy but the improvements in the breathing tests and the reduction in flare-ups would suggest survival will be better.

Stuart Elborn, Centre Director in the Centre for Infection and Immunity at Queen's University and co-leader of the study said: "The development of this drug is significant because it is the first to show that treating the underlying cause of Cystic Fibrosis may have profound effects on the disease, even among people who have been living with it for decades. The remarkable reductions in sweat chloride observed in this study support the idea that VX-770 improves protein function thereby

addressing the fundamental defect that leads to CF."

Dr Judy Bradley, from the University of Ulster said: "This drug opens the defective channel in the lung cells of people with Cystic Fibrosis and allows proper lung clearance of bacteria. This is a ground breaking treatment because it treats the basic defect caused by the gene mutation in patients. Correcting the cells with this mutation shows that treatments aimed at the basic mutation can work leading to improvements in lung function and symptoms."

Dr Damien Downey, from the Belfast Health and Social Care Trust said: "The success of this study illustrates the benefits that come from collaborative work here in Northern Ireland. Not only will this breakthrough help patients in Ireland and the UK but it has the potential to change the lives for those with Cystic Fibrosis around the world. As a result of the recent work researchers from Queen's University, University of Ulster and clinicians from Belfast Health and Social Care Trust have been selected to join the European Cystic Fibrosis Society Clinical Trials Network. This means Cystic Fibrosis researchers in Northern Ireland will be collaborating with their European counterparts to work toward improved treatments for Cystic Fibrosis on a global level. "

The new drug will be submitted for licensing in the Autumn of this year and is expected to be available to patients by as early next year.

Cystic Fibrosis affects approximately 70,000 people worldwide. It is Ireland's most common life-threatening genetically inherited disease, and one of the most common in the UK. Over 9000 people in the UK have Cystic Fibrosis and over 1100 people in Ireland. Ireland has the highest proportion of CF people in the world.

Provided by Queen's University Belfast