Medical Xpress

Scientists develop new treatment to prolong life of those with cystic fibrosis

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Scientists at Queen's University Belfast have discovered a new molecule which has the potential to prolong the life of individuals with cystic fibrosis (CF).

The molecule represents a possible future treatment and works by altering cellular ion channels resulting in improved airway hydration and significantly increased mucous clearance.

For individuals with CF, normal airway clearance mechanisms which keep the lungs free of infection are defective. As a result, a build-up of mucous occurs and predisposes the individual to chronic bacterial infection. The ensuing cycles of chronic infection and airway inflammation cause progressive destruction of the airways, which is ultimately fatal.

The innovative approach could reduce the frequency of these infections through a novel protease inhibitor which prevents activation of the epithelial sodium channel; ENaC.

The research involved a team of scientists from Queen's University alongside colleagues at the Royal College of Surgeons in Ireland and the University of North Carolina, and was funded by The Cystic Fibrosis Trust.

Speaking about the breakthrough, Dr Lorraine Martin from the School of Pharmacy at Queen's University Belfast, said: "This is an important

finding which could provide a novel therapeutic opportunity relevant to all individuals with CF, as the targeting of ENaC is independent of their underlying CF mutation. This strategy could prevent the significant lung damage that results from chronic cycles of infection and inflammation, with potential impact on quality of life as well as life expectancy. This is a further example of Queen's University's research advancing knowledge and changing lives."

Ed Owen, Chief Executive at the Cystic Fibrosis Trust, said "We are thrilled with these initial findings and are excited to see how the next stage of preclinical testing progresses. We are pleased to have been able to fund this world class project at its early stage and welcome the drug development programme planned over the coming years. Research is the biggest single area of investment for the Cystic Fibrosis Trust and it's wonderful to see projects like this making such positive progress in our fight for a life unlimited." Currently available pharmacological alternatives are only suitable for a small subset of patients depending on the disease-causing genetic mutation.

More information: James A Reihill et al. Inhibition of Protease-ENaC Signaling Improves Mucociliary Function in Cystic Fibrosis Airways, *American Journal of Respiratory and Critical Care Medicine* (2016). DOI: 10.1164/rccm.201511-2216OC

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