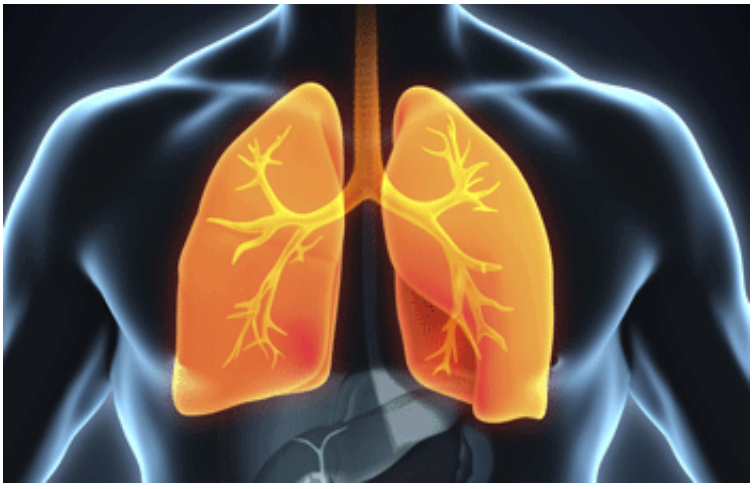
MedicalXpress

Breakthrough could help sufferers of fatal lung disease

May 24 2016



Pioneering research conducted by the University of Sheffield is paving the way for new treatments which could benefit patients suffering from the fatal lung disease pulmonary arterial hypertension (PAH).

For the first time scientists have identified a molecule that promotes the progression of the disease which affects 6,500 people in the UK.

The ground breaking study has identified that replacing the microRNA molecule can not only stop but reverse the progression of the disease.

Dr Allan Lawrie, from the University's Department of Infection,

Immunity and Cardiovascular Disease, who led the research said: "This research opens up a new insight into regulation of gene signalling and PAH.

"It opens up a much needed new avenue for drug development to treat this condition."

PAH is a chronic and debilitating condition that affects the blood vessels in the lungs and leads to heart failure.

The devastating disorder, which causes sufferers to be breathless and tired, often affects young people in their 30s and 40s and is more common in women.

The disease is caused by a sustained construction and a progressive narrowing of small arteries within the lung due to abnormal cell growth called vascular remodelling. This remodelling causes an elevation in blood pressure within the lungs which places significant strain on the right side of the heart. The strain of the pressure causes fatal right ventricle failure.

Current treatments for PAH target the constriction of the arteries but fail to fully reverse the vascular remodelling caused by the condition.

The new study, funded by the Medical Research Council (MRC), identified a specific small Ribonucleic acid (RNA) molecule that is downregulated in the blood of PAH patients – meaning the quality of a cellular component decreases.

The microRNA normally supresses SMURF1, a molecule that degrades the Bone Morphogenetic Protein Receptor 2 (BMPR2) which belongs to a family of genes originally identified for its role in regulating the growth and differentiation of bone and cartilage.

The loss of function in the BMPR2 gene is found to affect 80 per cent of families and around 20 per cent of individuals with idiopathic or familial PAH – but the mutations of the gene alone do not explain the disease development.

Now, the research team led by Dr Lawrie, a British Heart Foundation Senior Research Fellow, have discovered that the loss of the microRNA promotes the progression of PAH, and the replacement of the microRNA stopped and reversed the progression of disease in experimental models.

Dr Alex Rothman, an MRC Clinical Research Training Fellow, said: "The study suggests that either the delivery of the microRNA gene or the inhibition of SMURF1 is a viable therapeutic target.

"We are currently exploring opportunities to develop a drug that can block SMURF1 activity and hope to take this forward into clinical studies in the future."

Provided by University of Sheffield

Citation: Breakthrough could help sufferers of fatal lung disease (2016, May 24) retrieved 4 May 2024 from https://medicalxpress.com/news/2016-05-breakthrough-fatal-lung-disease.html

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