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# Cystic Fibrosis Research News

## Title:

**Ivacaftor and symptoms of extra-oesophageal reflux in patients with cystic fibrosis and *G551D* mutation**

## Authors:

Gemma L Zeybel PhD [1a], Jeffrey P Pearson PhD [1a], Amaran Krishnan MD [2], Stephen J Bourke MD [3], Simon Doe MD [3], Alan Anderson MSc [3], Shoaib Faruqi MD [4], Alyn H Morice MD [4], Rhys Jones MD [2], Mellissa McDonnell MD [1a], Mujdat Zeybel MD [1b,5], Peter W Dettmar PhD [6], Malcolm Brodlie MD [1b,7], Chris Ward PhD [1b,2]

## Affiliations:

[1a] Institute for Cell and Molecular Bioscience and [1b] Institute for Cellular Medicine, Medical School, Newcastle University, Catherine Cookson Building, Framlington Place, Newcastle Upon Tyne NE2 4HH, United Kingdom;
[2] Northern Aerodigestive group, Royal Victoria Infirmary, Newcastle upon Tyne NE1 4LP, United Kingdom;
[3]Department of Respiratory Medicine, Royal Victoria Infirmary, Newcastle upon Tyne NE1 4LP, UK;
[4] Academic Department of Respiratory Medicine, Hull York Medical School, University of Hull, Castle Hill Hospital, Cottingham, United Kingdom;
[5]School of Medicine, Koç University, Istanbul, Turkey;
[6] RD Biomed Ltd, Castle Hill Hospital, Cottingham, United Kingdom;
[7] Department of Paediatric Respiratory Medicine, Great North Children's Hospital, Queen Victoria Road, Newcastle upon Tyne, NE1 4LP, United Kingdom.

## What was your research question?

Ivacaftor (Kalydeco™) improves lung function in people with certain types of cystic fibrosis (CF). The drug was successful in clinical trials. Therefore, it is now available for general use within the CF clinic. In a real world study of individuals starting ivacaftor we investigated whether this treatment also helped gullet (oesophagus) problems that are known to affect the lives of people with CF.

## Why is this important?

CF is a disease which affects the lungs and digestive system, and symptoms of both are a burden. People with CF frequently have reflux symptoms, known to occur when fluid from

the stomach goes back up the gullet the wrong way. Reflux symptoms include cough and sore throat. Reflux has been linked to worsening lung function.
Ivacaftor treatment corrects the effects of the genetic problem seen in about 5% of people with CF, but this has mostly been shown in carefully managed clinical trials that measured lung function and weight gain. It is therefore important to know how treatments work in everyday use, and whether an improvement in reflux symptoms occurs with ivacaftor.

## What did you do?

We studied gullet and reflux symptoms in 12 individuals from a CF clinic before and after sustained treatment with ivacaftor. We obtained data for a year after ivacaftor treatment was started and changes in symptoms of reflux were measured by interviewing the 12 individuals. Sweat salt chloride levels, lung function and weight gain were also measured, to see whether typical problems associated with CF were improving.

## What did you find?

Our results, in real life, agreed with previous carefully managed trials; the CF participants involved gained weight and lung function. Sweat salt chloride changes indicated that the treatment had reduced the effects of the defective CF gene. Participants also reported a long-term improvement of reflux symptoms, benefits that have not been previously shown.

## What does this mean and reasons for caution?

Our study means that ivacaftor treatment is beneficial for those people with CF with symptoms of reflux and problems in the gullet, which are known to be troublesome. We have also shown that such real life studies are possible and informative in CF clinics. Ivacaftor treatment is long term, so longer studies in larger groups of people are needed. We could only report on the individual's experience of symptoms in the gullet, but other measurements would also be useful.

## What's next?

This is the first time that ivacaftor has been shown to reduce reflux symptoms in the gullet. Further, longer-term work is therefore required. As we measure lung health in people with CF we need to measure digestive health; building on the multi-disciplinary health care team approach known to help people with CF.

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