

# Cystic Fibrosis Research News

## Title:

Adherence to lumacaftor-ivacaftor therapy in patients with cystic fibrosis in France

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## What was your research question?

We wanted to measure whether people with cystic fibrosis (CF) adhered to Orkambi<sup>®</sup> therapy during the first year of treatment.

## Why is this important?

CFTR modulator therapies represent a real improvement in managing CF. These are the first treatments that target the underlying defect of the disease. Orkambi<sup>®</sup> has been approved for people with the F508del mutation, which is the most common mutation. People need to stick to Orkambi<sup>®</sup> treatment to get the most clinical benefit of it<sup>®</sup>. Considering the burden of treatment in people with CF, adherence to this innovative therapy is of major interest.

## What did you do?

People were recruited from five French CF centers. Their pharmacy refills were obtained from hospital pharmacies providing their Orkambi<sup>®</sup>. We calculated the adherence rates using the proportion of days covered (PDC) at 6 and 12 months after the date treatment was started. We considered the following factors which may be associated with adherence: age, gender, travel time from the place of residence to the hospital pharmacy and pulmonary function before treatment start.



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## What did you find?

96 people were included in the final group that we analysed. The average PDC was 96% at 6 months and 91% at 12 months. The proportion of people adhering to treatment, which we defined as PDC at least 80%, was 89% at 6 months and 83% at 12 months. In the analysis model we used which allowed us to adjust for potential confounders, the probability of sticking to treatment was significantly higher with the increase of age and pulmonary function. Proportion of adherent was significantly lower in people between 18 and 25 years old than in those between 12 and 17 years old.

## What does this mean and reasons for caution?

The high levels of adherence to Orkambi® which we found in this study are encouraging for the future of CFTR modulator therapies. Our results support the fact that the transition from adolescence to young adulthood is a critical period, associated with a risk that treatment may not be taken. One limitation of this study was the small number of people analysed. Moreover, Orkambi® is a lifelong treatment which does not mean that the burden of other CF therapies are reduced. This study only measured adherence during the first year of treatment and did not assess either adherence to other CF treatment or clinical outcomes.

## What's next?

Further research is needed to measure the long-term adherence to CFTR modulator therapies, their impact on adherence to other CF treatments and the clinical impact when people do not adhere to CFTR therapy.

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