

Medicine: lumacaftor-ivacaftor (brand name: Orkambi®) for cystic fibrosis

Vertex Pharmaceuticals (Europe) Limited

What is lumacaftor-ivacaftor for?

Lumacaftor-ivacaftor is used for the treatment of cystic fibrosis (CF). CF is an inherited life-limiting condition which mainly affects the lungs and gut, as well as the pancreas and kidneys. In people with CF, genetic changes (mutations) cause thick sticky mucus to build up in the body. This leads to a number of problems including inflammation and long term infections in the lungs and problems with digestion and the absorption of nutrients in the gut.

Lumacaftor-ivacaftor is for patients who are 2 years and older with a certain type of mutation that affects the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

How does lumacaftor-ivacaftor work?

Lumacaftor and ivacaftor are two active substances that may improve the balance of water and salt in the body's cells to make the mucus thinner. This could help relieve the symptoms of CF.

What has SMC said?

SMC has not recommended lumacaftor-ivacaftor for the treatment of CF as described above.



Why has SMC said this?

SMC looks at how well new medicines work compared with current treatments available in Scotland and in relation to how much they will cost to buy and administer (for example, if the medicine has to be given at a clinic or side effects have to be monitored).

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland. When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not just those who may be treated with the medicine under consideration.

To do this SMC consider the following:

- Clinical trial and economic evidence from the company that makes the medicine.

- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.
- Information from patient groups about the potential impact of the medicine on patients and carers.

After careful consideration, SMC decided the evidence from the company was not strong enough to be certain that the overall health benefits from lumacaftor-ivacaftor, in relation to its cost, offers value for money to NHSScotland. This was despite applying extra flexibility as lumacaftor-ivacaftor is a medicine for a rare condition.

What does SMC's decision mean for me?

Lumacaftor-ivacaftor should not normally be prescribed on the NHS in Scotland for use as described above. Your healthcare professional should talk to you about other treatment options available to you or your child. If your healthcare professional believes that lumacaftor-ivacaftor would be of benefit, he or she can make a request to prescribe it for you or your child. All health boards have procedures in place to consider these requests. For further information see:

Medicines in Scotland: What's the right treatment for me?

www.healthcareimprovementscotland.org/medicinesbooklet.aspx



More information

The organisations below can provide more information and support for people with cystic fibrosis and their families. SMC is not responsible for the content of any information provided by external organisations.

The Cystic Fibrosis Trust

 <https://www.cysticfibrosis.org.uk/>

 0300 373 1000

Quest for a CF Cure

 questforacfcure@gmail.com

You can find out more about lumacaftor-ivacaftor (Orkambi) in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.

 <http://www.ema.europa.eu>

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