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Cystic fibrosis: the number of patients eligible for free access to new CFTR modulator medicines has been expanded

The Board of Directors' approval covered two orphan drugs, two new molecules, seven generics, three biosimilars and five therapeutic indication extensions.

All cystic fibrosis patients who respond to the new indications authorised by the EMA for CFTR gene modulators will be able to benefit from free access to the medicine **Kaftrio** (ivacaftor/tezacaftor/elexacaftor) in combination with **Kalydeco** (ivacaftor).

This was decided by the AIFA Board of Directors at its meeting on 28 January, following intense negotiations between the Agency's Scientific and Economic Committee (CSE) and the pharmaceutical company that owns the medicine.

The medicine was already reimbursed in Italy for patients aged 2 years and older with the most common mutations, which affect approximately 80% of patients (over 3,000 in Italy, according to estimates). With the extension to rarer mutations, the group of patients who will have access to treatment covered by the National Health Service will also include almost all patients not yet included in the reimbursement scheme (approximately 1,600), who until now had no valid treatment alternatives.

The long-awaited decision was made possible thanks to the significant (confidential) discount obtained by the Agency on the price proposed by the company at the start of negotiations in July 2025.

Cystic fibrosis is a rare, progressive genetic disease that mainly affects the respiratory and digestive systems. It is caused by a mutated gene, called the CFTR (Cystic Fibrosis Transmembrane Regulator) gene, which causes the production of excessively thick mucus. This mucus closes the bronchi, leading to repeated respiratory infections, and obstructs the pancreas, preventing proper digestion and assimilation of food. CFTR protein modulator drugs, such as Kaftrio and Kalydeco, act on respiratory function, significantly improving patients' quality of life.

The Board also approved reimbursement for **Alyftrek** (vanzacaftor/tezacaftor/deutivacaftor), another new-generation CFTR modulator for cystic fibrosis, administered in two tablets to be taken once a day in patients aged 6 years and older.

Overall, the Board of Directors gave the green light to two orphan drugs, two new molecules, seven generics, three biosimilars and five therapeutic indication extensions.

'There was a lot of anticipation among patients for some of these medicines, such as those for cystic fibrosis,' said President Robert Nisticò, 'and we are delighted that the Agency's commitment to dialogue with the company has enabled the negotiation process to be concluded

successfully, as it is our priority to make essential medicines available to citizens. Clearly, it is AIFA's responsibility to ensure that the costs of these new therapies are sustainable for the National Health Service. For this reason, it is essential to evaluate innovation in terms of its real added value for patients and as an investment for the healthcare system as a whole. Medicines that radically change the history of a disease and help to increase life expectancy in better health are also a resource for the NHS because they make it possible, for example, to reduce the impact of hospitalisation costs and all those treatments that are necessary to deal with the frequent and serious complications that patients face as a result of these diseases.'

The second orphan drug is **Lyvdelzi** (seladelpar), indicated for the treatment of primary biliary cholangitis.

The two new molecules approved for reimbursement are monoclonal antibodies:

- **Alhemo** (concizumab), for routine prophylaxis of haemorrhagic episodes in patients with haemophilia A or B
- **Andembry** (garadacimab) for routine prevention of recurrent attacks of hereditary angioedema

Two other biosimilars of aflibercept (**Baiama** and **Eydenzelt**), the medicine for age-related neovascular macular degeneration, and golimumab (**Gobivaz**), the monoclonal antibody used to treat moderate to severe forms of rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and ulcerative colitis, will also be reimbursed by the National Health Service.

The first generics of nintedanib (**Nintedanib Teva** and **Viatris**, **Nipfilan**, **Puqod**), of nintedanib hexylate (**Nintedanib Accord** and **Sandoz**), indicated for the treatment of idiopathic pulmonary fibrosis and interstitial lung diseases, and of the antiepileptic brivaracetam (**Brivaracetam Teva**) will also be eligible for reimbursement.

Other therapeutic indications that will be covered by the NHS are:

- **Fabhalta** (iptacopan), for the treatment of C3 glomerulopathy, a rare and serious kidney disease
- **Jivi** (factor VIII), for the treatment and prophylaxis of bleeding in previously treated patients aged ≥7 years with haemophilia A
- **Kisqali** (ribociclib), for early-stage breast cancer.