swissmedic

Summary report on authorisation dated 16 February 2026

Trikafta® (active substances: elexacaftor, ivacaftor, tezacaftor)

Indication extension in Switzerland: 5 November 2025

Film-coated tablets for the treatment of cystic fibrosis

About the medicinal product

The medicinal product Trikafta contains the active substances elexacaftor, ivacaftor, and tezacaftor.

Trikafta has already been authorised by Swissmedic, since 10 December 2020, for the treatment of patients aged 12 years and older with cystic fibrosis who either have a so-called F508del mutation (defect) on 2 chromosomes[1] or an F508del mutation on 1 chromosome together with a defect on the second chromosome that prevents the formation of a functional CFTR[2] protein (so-called "minimal function mutation").

On 14 September 2021, the authorised indication for Trikafta was extended so that patients aged 12 years and older with cystic fibrosis and an F508del mutation could be treated regardless of the mutation on the second chromosome.

On 5 January 2022, a further indication extension was approved for the treatment of patients aged 6 years and older.

With the third indication extension, approved by Swissmedic on 13 June 2024, Trikafta can now also be used for the treatment of patients aged 2 years and older with cystic fibrosis who have at least 1 F508del mutation in the CFTR gene.

A further indication extension for Trikafta was approved on 5 November 2025. Patients with cystic fibrosis who have other CFTR mutations which respond to Trikafta can now also be treated.

Cystic fibrosis is a genetic disease caused by a deficiency and/or a dysfunction of the CFTR gene. The CFTR gene controls the formation (coding) of a protein used for transporting water and salts. The CFTR protein is also termed a chloride channel and sits on the cell surface. Chloride can move out of the cell through the channel. A dysfunction of the CFTR protein can lead, for example, to the formation of thick mucus in the lungs or pancreas, as well as elevated chloride levels in sweat.

Various mutations of the CFTR gene can lead to cystic fibrosis, although not all mutations

[1] Chromosomes: Chromosomes are the carriers of genetic information and are located in the cell nuclei

[2] CFTR: Cystic Fibrosis Transmembrane Conductance Regulator

of the CFTR gene lead to illness with symptoms of cystic fibrosis. The most common defect is the lack of coding for phenylalanine (F508del). Around 45% of patients with cystic fibrosis have this type of defect on each chromosome of the double set of chromosomes, which leads to an extensive CFTR malfunction in sufferers and thus to severe cystic fibrosis.

In addition, there are a number of other mutations that impair CFTR function in various ways and to varying extents.

Since cystic fibrosis is a rare and life-threatening disease, the indication extension for the medicinal product Trikafta has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Mode of action

The active substances tezacaftor and elexacaftor contained in Trikafta are so-called CFTR correctors, which bind to various sites on the CFTR protein, enabling them to improve the formation and the transport of CFTR proteins to the cell surface. The active substance ivacaftor helps improve the function of the CFTR channel at the cell surface.

The combination of the 3 active substances, elexacaftor, tezacaftor, and ivacaftor improves the function, and increases the quantity of, the CFTR protein at the cell surface, thereby increasing CFTR activity.

Thanks to the mode of action of these 3 combined active substances, the medicinal product Trikafta alleviates the symptoms associated with cystic fibrosis.

However, the underlying genetic defect is not cured.

Administration

Trikafta is a prescription-only medicine and contains different film-coated tablets or sachets of granules (morning dose and evening dose). The morning dose contains the active substances elexacaftor, ivacaftor, and tezacaftor combined in a single tablet/sachet of granules. The evening dose contains only the active substance ivacaftor.

The dosage is adjusted to the patient's age and weight.

For adults and children aged 6 years and older, the usual dosage is 2 film-coated tablets for the morning dose and 1 film-coated tablet for the evening dose. For children aged between 2 and 5 years inclusive, the usual dosage is 1 sachet of granules for the morning dose and 1 sachet of granules for the evening dose. The morning and evening doses should be taken approximately 12 hours apart.

The tablets may not be broken, chewed, or dissolved and should be taken with a fat-containing meal.

Efficacy

The efficacy of Trikafta to treat patients without F508del mutation was investigated in a clinical study (study 445-124). This study included 307 male and female patients with cystic fibrosis. The participants had a CFTR mutation which responds to Trikafta and were given doses adjusted to their age and weight. The study found that compared to placebo, treatment with Trikafta improved lung function and sweat chloride levels.

Precautions, undesirable effects, & risks

Trikafta may not be used in those who are hypersensitive to 1 of the active substances or any of the excipients.

The most common undesirable effects of Trikafta (affecting more than 1 in 10 users) are rash, upper respiratory tract infections, nasal congestion, sore throat, abdominal or stomach pain, diarrhoea, dizziness, headache, change in the type of bacteria in mucus, and increased liver enzymes (signs of stress on the liver).

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

Trikafta therapy covers an important medical need, as it offers a treatment option for patients with certain rare CFTR mutations, for which there has been no authorised CFTR modulator therapy to date.

The clinical data show that the medicinal product improves lung function in patients with cystic fibrosis who have a CFTR mutation which responds to Trikafta.

Taking all the risks and precautions into account, and based on the available data, the benefits of Trikafta outweigh the risks. Swissmedic has therefore authorised the medicinal product Trikafta, containing the active substances elexacaftor, tezacaftor, and ivacaftor, for the treatment of patients aged 2 years and over with cystic fibrosis (CF) who have at least 1 F508del mutation or another mutation in the CFTR gene which responds to Trikafta.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Trikafta®

Information for patients (package leaflet): Information for patients Trikafta®
Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

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