



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Koselugo (*selumetinib*)

An overview of Koselugo and why it is authorised in the EU

What is Koselugo and what is it used for?

Koselugo is a medicine used to treat plexiform neurofibromas, benign (non-cancerous) tumours along the nerves, when they cause symptoms and cannot be removed by surgery in children from 3 years of age with neurofibromatosis type 1 (NF1).

NF1 is rare, and Koselugo was designated an 'orphan medicine' (a medicine used in rare diseases) on 31 July 2018. Further information on the orphan designation can be found here:

ema.europa.eu/medicines/human/orphan-designations/eu3182050.

Koselugo contains the active substance selumetinib.

How is Koselugo used?

Koselugo is available as capsules and can only be obtained with a prescription. Treatment should be started by a doctor experienced in the diagnosis and treatment of NF1-related tumours.

The recommended dose of Koselugo is based on the patient's weight and height. The medicine is taken twice a day, about 12 hours apart, on an empty stomach. The medicine should not be given to patients who cannot swallow the capsule whole.

Before and during treatment with Koselugo, the doctor will check how well the patient's heart, eyes and liver are working. Treatment should be continued for as long as the patient improves or remains stable and the side effects are tolerable. The doctor may reduce the dose or stop treatment temporarily or permanently if certain side effects occur.

For more information about using Koselugo, see the package leaflet or contact your doctor or pharmacist.

How does Koselugo work?

The active substance in Koselugo, selumetinib, blocks enzymes called MEK1 and MEK 2 (MEK1/2) which are involved in stimulating cells to grow. MEK1/2 are overactive in NF1, making tumour cells grow uncontrollably. By blocking these enzymes, Koselugo helps slow down growth of the tumour cells.

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What benefits of Koselugo have been shown in studies?

A main study found that Koselugo is effective at treating plexiform neurofibromas in children with NF1 by shrinking the size of these tumours. In the study, tumour size decreased by at least 20% in 33 out of 50 (66%) children aged 3 years and older with NF1 and plexiform neurofibromas that could not be removed by surgery. In the study, Koselugo was not compared with any other medicine.

What are the risks associated with Koselugo?

The most common side effects with Koselugo (which may affect more than 4 in 10 people) are vomiting, rash, increased blood creatine phosphokinase (an enzyme released into the blood when muscle is damaged), diarrhoea, nausea (feeling sick), asthenic events (feeling weak), dry skin, fever, acneiform rash (a rash resembling acne), hypoalbuminaemia (low levels of albumin, a blood protein), increased aspartate aminotransferase (an enzyme indicating a possible sign of liver problems) and paronychia (nail bed infection).

The most common serious side effects with Koselugo (which may affect up to 1 in 10 people) are diarrhoea, anaemia (low red blood cell count), fever, increased blood creatine phosphokinase and increased blood creatinine (a sign of kidney problems).

Koselugo must not be used in patients with severe liver disease.

For the full list of side effects and restrictions of Koselugo, see the package leaflet.

Why is Koselugo authorised in the EU?

Plexiform neurofibromas can cause disfigurement, movement difficulties, pain and nerve problems. For children with plexiform neurofibromas that cannot be removed by surgery, there are no authorised treatment options; therefore, there is an important unmet medical need. Koselugo has been shown to be effective at shrinking tumour size in such children aged 3 years and older. The number of children included in the trial was small due to the rarity of the disease. Although data on safety were limited, they indicate that Koselugo's side effects are manageable. The European Medicines Agency therefore decided that Koselugo's benefits are greater than its risks and it can be authorised for use in the EU.

Koselugo has been given 'conditional authorisation'. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Koselugo?

Since Koselugo has been given conditional authorisation, the company that markets the medicine will provide further information from two ongoing studies that aim to confirm the benefits and safety of Koselugo in children with NF1 aged 3 years and above. The company will also carry out a third study in children to provide more information on the long-term safety of the medicine.

What measures are being taken to ensure the safe and effective use of Koselugo?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Koselugo have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Koselugo are continuously monitored. Side effects reported with Koselugo are carefully evaluated and any necessary action taken to protect patients.

Other information about Koselugo

Koselugo received a conditional marketing authorisation valid throughout the EU on 17 June 2021.

Further information on Koselugo can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/koselugo.

This overview was last updated in 06-2021.