EMADOC-1829012207-31561
EMEA/H/C/005244

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 adults and 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 tumours caused by NF1.

The medicine is taken twice a day, about 12 hours apart. It 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 (proteins) called MEK1 and MEK2 which are involved in stimulating cells to grow. MEK1 and MEK2 are overactive in NF1, making tumour cells grow uncontrollably. By blocking these enzymes, selumetinib helps slow down the growth of the tumour cells.

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What benefits of Koselugo have been shown in studies?

Two main studies found that Koselugo is effective at treating plexiform neurofibromas in children and adults with NF1 by shrinking the size of these tumours. The main measure of effectiveness in both studies was the proportion of patients who responded to treatment with Koselugo. Patients were considered to have responded when there were no signs of tumours (complete response) or when tumours shrank by at least 20% in size (partial response), and the response was confirmed within 3 to 6 months.

In the first 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 this study, Koselugo was not compared with any other medicine or placebo (a dummy treatment).

In the second study involving 145 adults with NF1 and plexiform neurofibromas, tumour size decreased by at least 20% in 14 out of 71 (around 20%) people who received Koselugo, compared with 4 out of 74 (around 5%) of those given placebo.

What are the risks associated with Koselugo?

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

The most common side effects with Koselugo in children (which may affect more than 4 in 10 people) include vomiting, rash, increased blood creatine phosphokinase (an enzyme released into the blood when muscle is damaged), diarrhoea, nausea (feeling sick), stomatitis (inflammation of the lining of the mouth), asthenic events (feeling weak), dry skin, fever, acneiform dermatitis (a rash resembling acne), hypoalbuminaemia (low levels of albumin, a blood protein), increased aspartate aminotransferase (an enzyme indicating a possible sign of liver problems), decreased haemoglobin (the protein in red blood cells that carries oxygen around the body) and paronychia (nail bed infection).

Some side effects with Koselugo in children can be serious. The most frequent (which may affect up to 1 in 10 people) include diarrhoea, anaemia (low red blood cell count), fever, increased blood creatine phosphokinase, increased blood creatinine (a sign of kidney problems), peripheral oedema (swelling especially of the ankles and feet) and vomiting.

The most common side effects with Koselugo in adults (which may affect more than 2 in 10 people) include acneiform rash (a rash resembling acne), increased blood creatine phosphokinase, diarrhoea, rash and vomiting.

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

Why is Koselugo authorised in the EU?

Plexiform neurofibromas can cause disfigurement, movement difficulties, pain and nerve problems. Koselugo has been shown to shrink tumour size in adults and children aged 3 years and older with NF1-associated plexiform neurofibromas that cannot be removed by surgery. In terms of safety, Koselugo’s side effects are considered manageable.

Koselugo has been given conditional authorisation. This means that it has been authorised on the basis of less comprehensive data than are normally required because it fulfils an unmet medical need. The Agency considers that the benefit of having the medicine available earlier outweighs any risks associated with using it while awaiting further evidence.

The company must provide further data on Koselugo. It must submit the results from an ongoing study to confirm the long-term safety of Koselugo in children with NF1 aged 3 years and older. Every year, the Agency will review any new information that becomes available.

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 10-2025.