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Ogsiveo (*nirogacestat*)

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

What is Ogsiveo and what is it used for?

Ogsiveo is a medicine used to treat adults with progressing desmoid tumours who need systemic treatment (medicines that travel through the bloodstream and affect the whole body).

Desmoid tumours develop from the soft, supporting tissues of the body. They can grow rapidly and damage nearby tissues and organs.

Progressing desmoid tumours are rare, and Ogsiveo was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 October 2019. Further information on the orphan designation can be found on the EMA website.

Ogsiveo contains the active substance nirogacestat.

How is Ogsiveo used?

Ogsiveo can only be obtained with a prescription. Treatment should be started and monitored by a doctor experienced in the use of cancer treatments.

The medicine is available as tablets to be taken by mouth twice a day. Treatment can continue for as long as the patient benefits from it and no unacceptable side effects occur.

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

How does Ogsiveo work?

The active substance in Ogsiveo, nirogacestat, blocks the activity of an enzyme (a type of protein) called gamma secretase. This enzyme normally activates a protein called NOTCH, which is found on the surface of cells and is involved in cell growth.

By blocking gamma secretase, nirogacestat prevents NOTCH activation and slows down the growth of tumours.

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What benefits of Ogsiveo have been shown in studies?

In a main study involving 142 people with progressing desmoid tumours, treatment with Ogsiveo was compared to placebo (a dummy treatment). The main measure of effectiveness was progression-free survival (the time people lived without their disease getting worse).

Based on the data available, it was evaluated that after 12 months, about 85% of patients who received Ogsiveo had not experienced a worsening of their disease or died, compared with about 53% of those given placebo.

Additionally, the study looked at the effect of Ogsiveo on the size of tumours (overall response rate). The tumours shrunk in about 41% (29 out of 70) of people taking Ogsiveo, compared with about 8% (6 out of 72) of people taking placebo.

What are the risks associated with Ogsiveo?

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

The most common side effects with Ogsiveo (which may affect more than 4 in 10 people) include diarrhoea, rash, nausea (feeling sick), tiredness, hypophosphataemia (low blood levels of phosphates), headache, stomatitis (inflammation of the lining of the mouth) and, in women who are able to have children, damages to their ovaries.

Some side effects can be serious. The most common with Ogsiveo (which may affect more than 1 in 10 people) is premature menopause.

Ogsiveo must not be used during pregnancy or in women who could become pregnant and are not using highly effective contraception, or those who are breastfeeding.

Why is Ogsiveo authorised in the EU?

Ogsiveo was shown to slow down the worsening of progressing desmoid tumours, a condition for which there was no other approved medicine and no standard treatment at the time of authorisation.

Regarding safety, Ogsiveo may cause harm to the unborn baby if taken during pregnancy. However, this risk is minimised by strict measures in place, including the requirement for highly effective contraception. There are uncertainties on how Ogsiveo may damage ovaries and testicles and its potential effect on fertility. This risk is mentioned in the product information and the company must provide further data to further evaluate it. Overall, the side effects of Ogsiveo are considered acceptable considering the lack of therapeutic options and can be managed with dose adjustments.

The European Medicines Agency therefore decided that Ogsiveo’s benefits are greater than its risks and it can be authorised for use in the EU.

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

To help ensure Ogsiveo is used safely, the company that markets the medicine must provide a guide to healthcare professionals and a card to patients. These materials explain that taking Ogsiveo during pregnancy can harm an unborn baby and provide guidance on how to prevent this risk. They will inform healthcare professionals and people using the medicine that a women who can become pregnant, as well as males with female partners who can become pregnant, must use highly effective contraception during treatment with Ogsiveo and for one week after the last dose.

The guide will inform healthcare professionals that a negative pregnancy test is required before starting treatment with Ogsiveo. It will also remind them that Ogsiveo may reduce the effectiveness of hormonal contraceptives. The patient card will advise people to contact their doctor immediately if they suspect they are pregnant while taking Ogsiveo.

The company that markets Ogsiveo must also optimise the formulation of the medicine and the way it is produced to ensure that impurity levels remain within the acceptable range.

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

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

Other information about Ogsiveo

Ogsiveo received a marketing authorisation valid throughout the EU on 14 August 2025

Further information on Ogsiveo can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/ogsiveo.

This overview was last updated in 08-2025.