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Ezmekly (*mirdametinib*)

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

What is Ezmekly and what is it used for?

Ezmekly is a medicine used to treat plexiform neurofibromas, benign (non-cancerous) tumours that grow along nerves, in adults and children 2 years and older with neurofibromatosis type 1 (NF1). It is used when the tumours cause symptoms (such as pain and weakness) and cannot be removed by surgery.

Neurofibromatosis is rare, and Ezmekly was designated an 'orphan medicine' (a medicine used in rare diseases) on 25 July 2019. Further information on the orphan designation can be found on the EMA website.

Ezmekly contains the active substance mirdametinib.

How is Ezmekly used?

Ezmekly can only be obtained with a prescription and treatment should be started by a doctor who has experience in the diagnosis and treatment of patients with tumours caused by NF1.

Ezmekly is available as capsules to be swallowed, or dispersible tablets which can be dispersed in water. It is taken twice a day, 12 hours apart. Patients take the medicine for 3 weeks, then stop for 1 week, and treatment can continue in repeated cycles until the disease gets worse or side effects become unacceptable.

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

How does Ezmekly work?

The active substance in Ezmekly, mirdametinib, is a small molecule which works by blocking a protein called MEK, which helps control how cells grow and survive. In people with NF1, MEK is overactive, leading to the growth of plexiform neurofibromas on nerve cells. By blocking MEK, mirdametinib helps to prevent the growth of these tumours.

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What benefits of Ezmekly have been shown in studies?

A main study involved 114 patients aged 2 years and older with plexiform neurofibromas caused by NF1 whose tumours could not be removed by surgery and caused symptoms. In this study, Ezmekly was not compared to any other treatment or a placebo.

All patients received Ezmekly in 28-day cycles (21 days on treatment followed by 7 days off), for up to 24 cycles (about 22 months).

The main measure of effectiveness was the proportion of patients who responded to treatment with Ezmekly. Patients were considered to respond when there were no signs of tumours (complete response) or if tumours shrank by at least 20% in size (partial response), and the response was maintained for at least 2 to 4 months.

In children, 52% (29 out of 56) had a partial response. Of those, 90% maintained their response for at least 12 months, and 48% for at least 24 months.

In adults, 41% (24 out of 58) had a partial response. Of those, 88% maintained their response for at least 12 months, and 50% for at least 24 months.

What are the risks associated with Ezmekly?

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

In adults, the most common side effects with Ezmekly (which may affect more than 1 in 3 people) include dermatitis acneiform (inflammation of the skin resembling acne), diarrhoea, nausea (feeling sick), elevated blood levels of the enzyme creatine phosphokinase, musculoskeletal pain (pain in the muscles and bones), vomiting (feeling sick) and fatigue. The most common serious side effects (which may affect up to 1 in 10 people) include abdominal (belly) pain, musculoskeletal pain and retinal vein occlusion (a blockage if the blood flow in a vein of the retina, the light sensitive membrane at the back of the eye).

In children, the most common side effects with Ezmekly (which may affect more than 1 in 3 people) include elevated blood levels of the enzyme creatine phosphokinase, diarrhoea, dermatitis acneiform, musculoskeletal pain, abdominal pain, vomiting and headache. Serious side effects (which may affect up to 1 in 10 people) include musculoskeletal pain.

Patients who are pregnant or may become pregnant must not take Ezmekly.

Why is Ezmekly authorised in the EU?

At the time of approval, only one medicine was authorised in the EU for plexiform neurofibromas in patients aged three years and older with NF1. Ezmekly provides an additional treatment option and has been shown to be effective in patients from a slightly younger age, starting at two years. However, the extent of its benefits remains uncertain, as the medicine has not been compared with any other treatment or a placebo.

In terms of safety, most side effects seen with Ezmekly were mild to moderate. However, some rare but serious side effects may affect the eyes and heart. There are also uncertainties about whether the medicine could damage DNA and potentially contribute to the development of cancer, as well as about its long-term safety.

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

Ezmekly 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 Ezmekly. It must submit results from a study evaluating the long-term safety and effectiveness of the medicine, as well as results from a study assessing its safety when used in clinical practice. 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 Ezmekly?

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

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

Other information about Ezmekly

Ezmekly received a conditional marketing authorisation valid throughout the EU on 17 July 2025.

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

This overview was last updated in 07-2025.