22 May 2025
EMA/CHMP/160270/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Ezmekly

mirdametinib

On 22 May 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a conditional[2] marketing authorisation for the medicinal product Ezmekly[3], intended for the treatment of plexiform neurofibromas (PN) in adults and children from 2 years of age with neurofibromatosis type 1 (NF1). The applicant for this medicinal product is SpringWorks Therapeutics Ireland Limited.

Ezmekly will be available as 1 mg and 2 mg hard capsules and 1 mg dispersible tablets. The active substance of Ezmekly, mirdametinib, is a selective, non-competitive mitogen-activated protein kinase 1 and 2 (MEK 1/2) inhibitor (ATC code: L01EE05). By inhibiting MEK, mirdametinib blocks the proliferation and survival of tumour cells in which the rapidly accelerated fibrosarcoma (RAF)-MEK-extracellular related kinase (ERK) pathway is activated.

The benefits of Ezmekly are its ability to provide durable responses (reduction of the volume of plexiform neurofibromas) in adults and children from 2 years of age with NF1-associated symptomatic, inoperable plexiform neurofibromas, as observed in a single arm trial. The most common side effects with Ezmekly in adults include acneiform dermatitis, diarrhoea, nausea, increased blood creatinine phosphokinase, musculoskeletal pain, vomiting, and fatigue. The most common side effects in children include blood creatine phosphokinase, diarrhoea, acneiform dermatitis, musculoskeletal pain, abdominal pain, vomiting, and headache.

The full indication is:

> Ezmekly as monotherapy is indicated for the treatment of symptomatic, inoperable plexiform neurofibromas (PN) in paediatric and adult patients with neurofibromatosis type 1 (NF1) aged 2 years and above.

Treatment with Ezmekly should be initiated by physicians experienced in the diagnosis and the treatment

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] A conditional marketing authorisation is granted to a medicinal product that fulfils an unmet medical need when the benefit to public health of immediate availability outweighs the risk inherent in the fact that additional data are still required. The marketing authorisation holder is expected to provide comprehensive clinical data at a later stage.
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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of patients with NF1 related tumours.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.