22 April 2021
EMA/CHMP/210566/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Koselugo

selumetinib

On 22 April 2021, 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 Koselugo[3], intended for the treatment of paediatric patients with neurofibromatosis type 1 (NF1) plexiform neurofibromas (PN).

The applicant for this medicinal product is AstraZeneca AB.

Koselugo will be available as 10 mg and 25 mg hard capsules. The active substance of Koselugo is selumetinib, a selective inhibitor of mitogen activated protein kinase kinases 1 and 2 (MEK 1/2) (ATC code: L01EE04). MEK inhibition can block the proliferation and survival of tumour cells in which the RAF-MEK-ERK pathway is activated.

The benefits of Koselugo are its ability to reduce the volume of plexiform neurofibromas and thereby improve clinical outcomes such as pain intensity, mobility and/or disfigurement in patients. The most common side effects are vomiting, rash, increased blood creatine phosphokinase, diarrhoea, nausea, dry skin, asthenic events, pyrexia, acneiform rash, hypoalbuminaemia, stomatitis, increased aspartate aminotransferase and paronychia.

The full indication is:

> Koselugo as monotherapy is indicated for the treatment of symptomatic, inoperable plexiform neurofibromas (PN) in paediatric patients with neurofibromatosis type 1 (NF1) aged 3 years and above.

Koselugo should be prescribed by physicians experienced in the diagnosis and the treatment of patients with NF1 related tumours.

[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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Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.