EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 July 2020
EMA/CHMP/392892/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Ayvakyt

avapritinib

On 23 July 2020, 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 Ayvakyt,[3] intended for the treatment of adult patients with unresectable or metastatic gastrointestinal stromal tumours (GIST) harbouring the platelet-derived growth factor receptor alpha (PDGFRA) D842V mutation. The applicant for this medicinal product is Blueprint Medicines (Netherlands) B.V.

Ayvakyt will be available as film-coated tablets (100 mg, 200 mg and 300 mg). The active substance of Ayvakyt is avapritinib, a protein kinase inhibitor (ATC code: L01EX18) designed to potently and selectively inhibit oncogenic KIT and PDGFRA mutants by targeting the active conformation of the kinase.

The benefits with Ayvakyt are its ability to provide durable responses in patients with GIST harbouring the PDGFRA D842V mutation. The most common side effects (in ≥20% of patients) are nausea, fatigue, anaemia, periorbital oedema, face oedema, hyperbilirubinaemia, diarrhoea, vomiting, oedema peripheral, lacrimation increased, decreased appetite and memory impairment.

The full indication is:

> AYVAKYT is indicated as monotherapy for the treatment of adult patients with unresectable or metastatic gastrointestinal stromal tumours (GIST) harbouring the platelet-derived growth factor receptor alpha (PDGFRA) D842V mutation.

It is proposed that Ayvakyt be prescribed by physicians experienced in the treatment of anticancer therapy.

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

[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 likely to provide comprehensive clinical data at a later stage.
[3] This product was designated as 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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