15 September 2022
EMA/CHMP/734156/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Pyrukynd

mitapivat

On 15 September 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Pyrukynd[2], intended for the treatment of an inherited condition called pyruvate kinase deficiency. The applicant for this medicinal product is Agios Netherlands B.V.

Pyrukynd will be available as 5 mg + 20 mg, 5 mg, 20 mg, 20 mg + 50 mg and 50 mg film-coated tablets. The active substance of Pyrukynd is mitapivat, a pyruvate kinase activator (ATC code: B06AX04). Pyrukynd works by binding to the enzyme pyruvate kinase, thus stabilising the defective enzyme and helping it work better. This results in a reduction in disease symptoms.

The benefits of Pyrukynd are an increased haemoglobin concentration, as observed in a randomised, double-blind, placebo-controlled, multicentre study in adult patients who were not regularly transfused, and a reduction in the number of transfusions required, as observed in a phase 3, single-arm, open-label study in adult patients who were regularly transfused. The most common side effect is insomnia.

The full indication is:

> Pyrukynd is indicated for the treatment of pyruvate kinase deficiency (PK deficiency) in adult patients (see section 4.4).

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.

[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] 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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