12 December 2024
EMA/CHMP/561782/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Rytelo

imetelstat

On 12 December 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Rytelo[2], intended for the treatment of adults with transfusion-dependent anaemia due to very low, low or intermediate risk myelodysplastic syndromes (MDS).

The applicant for this medicinal product is Geron Netherlands B.V.

Rytelo will be available as 47 mg and 188 mg powder for concentrate for solution for infusion. The active substance of Rytelo is imetelstat, an other antineoplastic agent (ATC code: L01XX80). Imetelstat is an oligonucleotide telomerase inhibitor. By blocking telomerase activity, imetelstat causes telomere shortening, inhibits the proliferation of malignant stem and progenitor cells and induces cell death, ultimately leading to a reduction in malignant clones.

The benefit of Rytelo in patients with transfusion-dependent anaemia due to very low, low or intermediate risk MDS is a reduction in the need for red blood cell transfusions in the first 24 weeks of treatment compared to placebo, as observed in a double-blind controlled study. The most common side effects are thrombocytopenia, leukopenia, neutropenia, increased aspartate aminotransferase (AST), increased alanine aminotransferase (ALT), increased alkaline phosphatase (ALP), asthenia and headache.

The full indication is:

> Rytelo is indicated as monotherapy for the treatment of adult patients with transfusion-dependent anaemia due to very low, low or intermediate risk myelodysplastic syndromes (MDS) without an isolated deletion 5q cytogenetic (non-del 5q) abnormality and who had an unsatisfactory response to or are ineligible for erythropoietin-based therapy (see section 5.1).

Treatment with Rytelo should be administered and monitored under the supervision of physicians and healthcare professionals who are experienced in haematologic diseases and their 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] 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.