20 July 2017
EMA/CHMP/263985/2017
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Rydapt

midostaurin

On 20 July 2017, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Rydapt, intended for the treatment of adult patients with newly diagnosed acute myeloid leukaemia (AML) who are FLT3 mutation-positive and for the treatment of adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated haematological neoplasm (SM AHN), or mast cell leukaemia (MCL). Rydapt was designated as an orphan medicinal product on 29 July 2004. The applicant for this medicinal product is Novartis Europharm Ltd.

Rydapt will be available as 25 mg soft capsules. The active substance of Rydapt is midostaurin, a protein kinase inhibitor (ATC code: L01XE39) which inhibits multiple receptor tyrosine kinases, including FLT3 and KIT kinase.

The benefit of Rydapt in AML is its ability to improve overall survival rates when used in combination with standard chemotherapy. The most common side effects observed in patients with AML treated with midostaurin are febrile neutropenia, nausea, exfoliative dermatitis, vomiting, headache, petechiae and pyrexia.

In ASM, SM AHN and MCL, Rydapt was shown to achieve an overall response in the majority of patients. The most common side effects observed in patients with ASM, SM AHN and MCL are nausea, vomiting, diarrhoea, peripheral oedema and fatigue.

The full indication is:

‘‘Rydapt is indicated:

- in combination with standard daunorubicin and cytarabine induction and high dose cytarabine consolidation chemotherapy, and for patients in complete response followed by Rydapt single agent maintenance therapy, for adult patients with newly diagnosed acute myeloid leukaemia (AML) who are FLT3 mutation positive (see section 4.2);
- as monotherapy for the treatment of adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated haematological neoplasm (SM AHN), or mast cell leukaemia

[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

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(MCL)''.

It is proposed that Rydapt be prescribed by physicians experienced in the use of anti-cancer therapies.

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.