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Recommendation for maintenance of orphan designation at the time of marketing authorisation

Besponsa (inotuzumab ozogamicin) for the treatment of B-cell acute lymphoblastic leukaemia

During its meeting of 10 to 12 May 2017, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/13/1127 for Besponsa (inotuzumab ozogamicin), as an orphan medicinal product for the treatment of B-cell acute lymphoblastic leukaemia (ALL). The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. As other methods of treatment are authorised in the European Union (EU), the COMP also considered whether the medicine is of significant benefit to patients with B-cell ALL. The COMP recommended that the orphan designation of the medicine be maintained[1].

Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Besponsa ' as monotherapy the treatment of adults with relapsed or refractory CD22-positive B-cell precursor acute lymphoblastic leukaemia (ALL)'.

This falls within the scope of the product's designated orphan indication, which is B-cell ALL.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2013. B-cell ALL remains a condition that is debilitating in the long term and life threatening, particularly due to its effects on normal white blood cells, reducing the patient's ability to fight infections and causing organ damage.

Prevalence of the condition

The sponsor provided updated information on the prevalence of B-cell ALL based on data from the Globocan 2012 database and the scientific literature.

[1] The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with the same therapeutic indication cannot be placed on the market.

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On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of B-cell ALL remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was estimated to be less than 1 in 10,000 people. This is equivalent to a total of fewer than 51,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, several chemotherapy medicines were authorised in the EU for the treatment of ALL. In addition, Blincyto (blinatumomab) had been approved in the EU in November 2015 for the treatment of adults with B-cell precursor ALL.

Significant benefit of Besponsa

The COMP concluded that the claim of a significant benefit of Besponsa in B-cell ALL is justified based on data from a main study with Besponsa in patients whose disease had come back after previous treatment or had not responded to previous treatment. Results showed that Besponsa was more effective than chemotherapy alone.

Compared with Blincyto, the COMP considered that Besponsa is easier to give (one-hour infusion as opposed to a continuous infusion which requires the patient to stay in hospital for several days).

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Besponsa is of significant benefit to patients affected by B-cell ALL.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Besponsa still meets the criteria for designation as an orphan medicinal product and that Besponsa should remain in the Community Register of Orphan Medicinal Products.

More information on the COMP assessment can be found in the May 2017 COMP minutes.

Further information on Besponsa (inotuzumab ozogamicin) can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.