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Outcome of assessment on use of Revolade in treatment of severe aplastic anaemia in children

The European Medicines Agency has finalised its assessment of an application to extend the use of Revolade to include children with severe aplastic anaemia (SAA). Although EMA did not recommend this use, it agreed that relevant data from the study submitted with the application be included in the medicine's product information so that healthcare professionals have access to up-to-date data on the effects of Revolade in people with SAA.

What is Revolade and what is it used for?

Revolade is a medicine used to treat:

- primary immune thrombocytopenia (ITP), a disease in which the patient's immune system destroys platelets (components in the blood that help it to clot). Patients with ITP have low platelet counts in the blood (thrombocytopenia) and are at risk of bleeding. Revolade is used in patients from 1 year of age for whom treatment with medicines such as corticosteroids or immunoglobulins has not worked. In children and adolescents, the medicine is used when they have had the disease for at least 6 months;
- thrombocytopenia in adults with chronic (long-term) hepatitis C, a liver disease caused by the hepatitis C virus. Revolade is used when the thrombocytopenia is too severe to allow interferon-based therapy (a type of treatment for hepatitis C);
- acquired SAA (a disease in which the bone marrow does not make enough blood cells or platelets). Acquired means that the disease is not inherited. Revolade is used in adults whose disease is not controlled by immunosuppressive therapy (medicines that lower the body's immune defences) and cannot receive haematopoietic stem cell transplantation (where the patient's bone marrow is replaced by stem cells from a donor to form new bone marrow).

Revolade has been authorised in the EU since March 2010. It contains the active substance eltrombopag and is available as tablets and as a powder to prepare a suspension (a liquid) to be taken by mouth.

Further information on Revolade's current uses can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/revolade.

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What change had the company applied for?

The company applied to extend the use of Revolade to children aged 2 years and older with SAA whose disease is not controlled by, or has come back after, immunosuppressive treatment and who cannot receive haematopoietic stem cell transplantation.

How does Revolade work?

In the body, a natural hormone called thrombopoietin stimulates the production of platelets by attaching to and activating certain receptors (targets) in the bone marrow. Like thrombopoietin, the active substance in Revolade, eltrombopag, also attaches to and stimulates thrombopoietin receptors. This increases the production of platelets, improves platelet counts and reduces the risk of bleeding. In some patients with SAA, Revolade may also increase the production of blood cells. In children aged 2 years and older with severe aplastic anaemia, Revolade is expected to work in the same way as it does in adults with this condition.

What did the company present to support its application?

The company presented results from an ongoing main study involving 51 children aged 2 years and older with SAA who could not receive haematopoietic stem cell transplantation. In this study, 37 children had not received prior treatment, and 14 had received immunosuppressive therapy, but their disease was not controlled or had come back. All children received Revolade in combination with immunosuppressive therapy for 26 weeks. The medicine was not compared with any other treatment or placebo (a dummy treatment). The main objective of the study was to assess how Revolade behaves in children. Secondary objectives included assessing the medicine's safety and effectiveness.

What were EMA's conclusions?

EMA noted that, although the results of the study provided by the applicant suggest that children with SAA might benefit from treatment with Revolade, only 14 patients in the study matched the intended use. This number was considered too small to draw firm conclusions on the effectiveness and safety of Revolade in these children.

EMA therefore concluded that the safety and effectiveness of Revolade have not been sufficiently established in children with SAA, and that it should not be authorised in these patients. Nevertheless, the prescribing information for Revolade will be updated to include relevant data, so that healthcare professionals have access to up-to-date data on the effects of Revolade in children with SAA.

Does this outcome affect patients in clinical trials/ compassionate use programmes?

The company informed the Agency that the study of Revolade in children with SAA has recently been completed, and that no other studies are ongoing in these children.