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First oral treatment against residual haemolytic anaemia in patients with paroxysmal nocturnal haemoglobinuria

23 February 2024

EMA has recommended granting a marketing authorisation in the European Union (EU) for Voydeya, the first oral treatment for patients with paroxysmal nocturnal haemoglobinuria (PNH) who continue to have haemolytic anaemia (low levels of red blood cells) despite treatment with a C5 complement inhibitor (eculizumab or ravulizumab).

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PNH is a rare genetic disorder and potentially life-threatening blood disease leading to the premature destruction of red blood cells (haemolytic anaemia) by the immune system. Disease symptoms include fatigue, body pain, blood clots, bleeding and shortness of breath. PNH generally worsens over time and patients often require red blood cell transfusions. The standard treatment for PNH is monoclonal antibodies (ravulizumab or eculizumab) known as complement inhibitors. Some patients who experience residual haemolytic anaemia need subcutaneous infusions of another complement inhibitor (pegcetacoplan) to prevent the damage to red blood cells.

The active substance of Voydeya is danicopan, a complement inhibitor which prevents the destruction of red

blood cells, thereby helping to relieve the symptoms of PNH. Complement inhibitors are a type of immunotherapy used in the treatment of many inflammatory conditions that could be caused by deficiencies of the complement system, a part of the body's innate immune system.

EMA’s recommendation is mainly based on the results of a randomised, double-blind, placebo-controlled phase III study in 86 patients with PNH and clinically significant evidence of residual haemolytic anaemia while on treatment with C5 complement inhibitors ravulizumab or eculizumab. Of these patients, 57 received danicopan and 29 were given a placebo. After 12 weeks, patients treated with danicopan had a clinically-relevant increase in haemoglobin (an average of 2.35 g/dl) compared to those treated with placebo.

Other benefits included a significant decrease in fatigue, the number of required blood transfusions and the amount of blood transfused, as well as a reduction in the amount of bilirubin (a yellow-brownish pigment generated by the breakdown of red blood cells). These positive outcomes remained at week 72 and support durability and maintenance of the effect over time.

The most frequent side effects in patients treated with Voydeya were headache, nausea, diarrhoea, arthralgia (pain in the joints), vomiting and fever. Some patients experienced elevated liver enzymes and breakthrough haemolysis (flare-up of red-blood-cell breakdown despite ongoing complement inhibition). Long-term data and data on the use of Voydeya in elderly patients are limited. As for other complement inhibitors, the potential risk of cancer and blood abnormalities cannot be excluded and should be monitored.

Voydeya was supported through EMA's PRIority MEdicines (PRIME) scheme, which provides early and enhanced scientific and regulatory support to medicines that have a particular potential to address patients' unmet medical needs.

The opinion adopted by the CHMP is an intermediary step on Voydeya’s path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a

marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role or use of this medicine in the context of the national health system of that country.

Notes

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1. The applicant for Voydeya is Alexion Europe.

2. Voydeya was designated as an orphan medicinal product on 12 December 2017 for the treatment of paroxysmal nocturnal haemoglobinuria. Following this positive CHMP opinion, the Committee for Orphan Medicinal Products (COMP) will assess whether the orphan designations should be maintained.

3. Voydeya was accepted into the PRIME scheme on for the treatment of paroxysmal nocturnal hemoglobinuria patients who are not adequately responding to a C5 inhibitor.

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