EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Media and Public Relations

Press release

First treatment for acute hepatic porphyria

EMA's human medicines committee (CHMP) has recommended granting a marketing authorisation in the European Union (EU) for Givlaari (givosiran), the first treatment for acute hepatic porphyria (AHP) in adults and adolescents aged 12 years and older.

Acute hepatic porphyria is a rare genetic condition in which patients lack certain enzymes needed to produce haem, a basic structure of haemoglobin that binds to oxygen and is characterised by an accumulation of porphyrins in the body to toxic amounts. This can cause attacks of severe abdominal pain, vomiting and nervous system disorders, such as seizures, depression and anxiety. AHP is life-threatening due to the possibility of paralysis and respiratory arrest during attacks.

At the time of designation, acute hepatic porphyria affected approximately 0.1 in 10,000 people in the EU and Norway, Iceland and Liechtenstein, which makes it a rare disease.

The new active substance givosiran is made of a short, synthetic strand of genetic material called 'small interfering RNA' that has been designed to interfere with the production of an enzyme involved in an early step in making haem. By blocking this early step of haem production in patients with acute hepatic porphyria, the medicine is expected to prevent the next steps which produce substances that accumulate in the body and cause the symptoms of the disease.

There are no approved treatments that directly ameliorate or prevent chronic symptoms experienced by many AHP patients and no approved treatments to reduce the risk of attacks. Intravenous hemin, a human blood-derived haem formulation, is the only therapy currently approved for the treatment of acute attacks. However, it is not approved as a chronic treatment to prevent attacks. Additional treatments include painkillers and antiemetics (to treat nausea and vomiting), chemically-induced menopause with hormonal suppression therapy, and liver transplantation.

The benefits and safety of Givlaari were demonstrated in a phase III clinical study which enrolled 94 patients with AHP who experienced at least two attacks in the past six months. Data from the study showed that the treatment resulted in a significant decrease of annual attacks, less pain and an improved quality of life.

Since Givlaari addresses an unmet medical need, it benefited from PRIME, EMA's platform for early and enhanced dialogue with developers of promising new medicines. This interaction led to a more robust application package to demonstrate the medicine's benefits and risks, which allowed the accelerated assessment of Givlaari in 150 days.

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Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Givlaari is Alnylam Netherlands B.V.
3. This product was designated as an orphan medicine during its development. Orphan designations are reviewed by EMA's Committee for Orphan Medicinal Products (COMP) at the time of approval to determine whether the information available to date allows maintaining the medicine's orphan status and granting the medicine ten years of market exclusivity.
4. Due to the lack of recruitment of adolescents in a clinical study, EMA's Paediatric Committee (PDCO) granted a product specific waiver for all subsets of the paediatric population in accordance with Article 12 of the Paediatric Regulation.
5. To maximise the use of expertise within the European regulatory network, a multinational assessment team with experts from the Netherlands, Portugal and Poland evaluated the dossier.
6. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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