28 February 2019
EMA/CHMP/139000/2019
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Palynziq

pegvaliase

On 28 February 2019, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Palynziq, intended for the treatment of phenylketonuria. Palynziq was designated as an orphan medicinal product on 28 January 2010. The applicant for this medicinal product is BioMarin International Limited.

Palynziq will be available as 2.5 mg, 10 mg and 20 mg solutions for injection. The active substance of Palynziq is pegvaliase, a PEGylated recombinant phenylalanine ammonia lyase enzyme that converts phenylalanine to ammonia and *trans*-cinnamic acid, which are then eliminated from the body primarily by liver metabolism.

The benefits with Palynziq are its ability to reduce levels of phenylalanine in the blood. The most common side effects are injection site reactions, arthralgia and hypersensitivity reactions.

The full indication is: “treatment of patients with phenylketonuria (PKU) aged 16 years and older who have inadequate blood phenylalanine control (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options”. It is proposed that Palynziq be prescribed by physicians experienced in the the management of PKU.

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.

[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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