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Media and Public Relations

Press release

CHMP recommends authorisation of new treatment for phenylketonuria, a rare inherited metabolic disease

EMA's human medicines committee (CHMP) has recommended the authorisation of Palynziq (pegvaliase), a new medicine for patients aged 16 and older with phenylketonuria, a rare but potentially serious inherited metabolic disease.

Patients suffering from this disorder do not have the enzyme that breaks down phenylalanine, an amino acid which is found in most foods containing protein. As a result, phenylalanine can build up in the blood, causing problems in the brain and nervous system. Patients with this disease may experience neurological and psychiatric disorders, including intellectual disability, anxiety, depression, and neurocognitive dysfunction.

This disease is not curable and patients need to follow a lifelong strict diet low in phenylalanine-containing foods (such as meat, fish, eggs, nuts, legumes and corn) to reduce phenylalanine intake. However, most adults and adolescents with phenylketonuria do not adhere to these dietary restrictions and phenylalanine levels in the blood are too high. Only one other treatment is currently authorised in the European Union to help manage the disease. Patients could therefore benefit from further treatment options for this condition which can impact heavily upon their quality of life.

Palynziq is for use by patients with excessive levels of phenylalanine in the blood (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options. It has a novel mode of action against phenylketonuria: it contains pegylated recombinant phenylalanine ammonia lyase, an enzyme that can break down phenylalanine and is expected to stop the accumulation of phenylalanine in the body, thus relieving the symptoms of the disease.

In phase 3 clinical trials, the majority of patients treated with the medicine had blood phenylalanine levels ≤ 600 micromol/l after 18 months of treatment. The data also showed that with continued long-term treatment patients' psychiatric and cognitive symptoms improved.

The most common side effects were arthralgia and injection site reactions including erythema and rash. These are considered to be linked to the allergic reactions seen in all patients; most were mild to moderate but acute hypersensitivity reactions occurred in a small fraction of patients (5.6%).

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Therefore, the CHMP required the company to take specific measures to minimise this risk, including making available additional educational material for prescribers and patients.

The opinion adopted by the CHMP is an intermediary step on Palynziq'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 the 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/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Palynziq is BioMarin International Limited.
3. As always at time of approval, EMA's committee for orphan medicines (COMP) will review the orphan designation to determine whether the information available to date allows maintaining Palynziq's orphan status and granting this medicine ten years of market exclusivity.
4. The only medicine currently authorised at EU level for phenylketonuria is Kuvan (sapropterin).
5. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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