EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Press Office

Press release

First HDAC inhibitor for treatment of multiple myeloma recommended for approval in EU

Farydak shown to slow progression of rare blood cancer

The European Medicines Agency (EMA) has recommended granting a marketing authorisation for Farydak (panobinostat) for the treatment of multiple myeloma.

Farydak is the first cancer medicine that targets enzymes known as histone deacetylases or HDACs, which are involved in turning genes 'on' and 'off' within cells. It is intended for patients with relapsed and/or refractory multiple myeloma who have received at least two prior standard therapies, including bortezomib and an immunomodulatory agent. It is to be used in combination with bortezomib and the anti-inflammatory medicine dexamethasone.

Multiple myeloma is a rare cancer of a type of white blood cells called plasma cells. In multiple myeloma, the division of plasma cells becomes out of control, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. The abnormal cells interfere with the production of normal white blood cells, red blood cells and platelets, and patients develop complications such as infections, anaemia, bone pain and fractures, raised blood calcium levels and kidney dysfunction.

Multiple myeloma is generally an incurable disease that leads to bone destruction and kidney failure. In 2012, around 39,000 people had multiple myeloma in the European Union (EU). Around 50% of patients diagnosed with multiple myeloma under current treatment are still alive after five years.

The recommendation from EMA's Committee for Medicinal Products for Human Use (CHMP) is based on a study of Farydak in combination with bortezomib and dexamethasone in 768 patients with multiple myeloma, 147 of whom had received at least two prior treatments that included bortezomib and an immunomodulatory agent. Participants were randomly assigned to receive a combination of Farydak, bortezomib and dexamethasone, or bortezomib and dexamethasone alone. The study found that among patients who had received at least two prior treatments with bortezomib and an immunomodulatory agent, the disease progressed more slowly (by a median of 7.8 months) with Farydak treatment. The most common side effects of Farydak were blood disorders, haemorrhage, diarrhoea, nausea, vomiting and fatigue.

[1] The data in paragraph 5 has been updated.

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In the context of these sometimes serious side effects, the CHMP considered that the overall benefit-risk balance for Farydak is only positive in patients with relapsed and/or refractory multiple myeloma who have received at least two prior regimens including bortezomib and an immunomodulatory agent. A follow-up plan to monitor the safety of Farydak and lower its risks was agreed by the CHMP.

Because multiple myeloma is rare, Farydak was designated as an orphan medicine by the Committee for Orphan Medicinal Products (COMP) in 2012. Orphan designation gives medicine developers access to incentives such as fee reductions for scientific advice and is the key instrument available in the EU to encourage the development of medicines for patients with rare diseases.

The opinion adopted by the CHMP at its June 2015 meeting is an intermediary step on Farydak's path to patient access. The CHMP 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/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 Farydak is Novartis Europharm Ltd.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu.

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