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EPAR summary for the public

Farydak

panobinostat

This is a summary of the European public assessment report (EPAR) for Farydak. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Farydak.

For practical information about using Farydak, patients should read the package leaflet or contact their doctor or pharmacist.

What is Farydak and what is it used for?

Farydak is a cancer medicine used in combination with two other medicines, bortezomib and dexamethasone, to treat multiple myeloma (a cancer of the bone marrow). It is given to adults whose disease has come back or got worse after at least two previous treatments, including bortezomib and an immunomodulatory medicine (a medicine that acts on the immune system).

Farydak contains the active substance panobinostat.

Because the number of patients with multiple myeloma is low, the disease is considered 'rare', and Farydak was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 November 2012.

How is Farydak used?

Treatment with Farydak must be started by a doctor experienced in the treatment of cancer and the medicine can only be obtained with a prescription.

Farydak is available as capsules (10, 15 and 20 mg) and it is given in 21-day treatment cycles, together with bortezomib and dexamethasone. The recommended starting dose of Farydak is 20 mg, taken on days 1, 3, 5, 8, 10 and 12 of the cycle. Patients are given the medicine for 8 cycles, and further 8 cycles of treatment are recommended in those that benefit. The doctor may have to adjust or

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delay the dose in patients who experience severe side effects. For further information, see the summary of product characteristics (part of the product information).

How does Farydak work?

The active substance in Farydak, panobinostat, is a type of medicine called a histone deacetylase (HDAC) inhibitor. It blocks the activity of enzymes called histone deacetylases (HDACs), which are involved in switching the activity of genes on and off within cells. In multiple myeloma, panobinostat is expected to keep genes that suppress the division and growth of the cancer cells switched 'on'. This is expected to stop the cancer cells from multiplying and to activate processes that kill the cell, thereby slowing down the growth of the cancer.

What benefits of Farydak have been shown in studies?

The benefits of Farydak have been shown in one main study involving 768 patients with multiple myeloma that had come back after previous treatments. The medicine was compared with placebo (a dummy treatment) as an addition to treatment with bortezomib and dexamethasone. The main measure of effectiveness was the average length of time before the patient's disease got worse again (progression-free survival), which was 12 months in patients given Farydak, compared with around 8 months in those given placebo.

When results were analysed just for the group of patients who had previously received at least two previous treatments, including bortezomib and an immunomodulatory medicine (thalidomide, lenalidomide or pomalidomide), the average time until the myeloma got worse was 12.5 months with Farydak, versus 4.7 months with placebo.

What are the risks associated with Farydak?

The most common side effects with Farydak (which may affect more than 1 in 10 people) are diarrhoea, tiredness, nausea (feeling sick) and vomiting, and effects on the blood such as thrombocytopenia (low levels of blood platelets which are important for blood clotting), anaemia and neutropenia and lymphopenia (low levels of certain white blood cells). The most significant effects that led to patients having to stop treatment (which happened in about 4 patients in 10) were diarrhoea, weakness and tiredness, and pneumonia (lung infection). Effects on the heart occurred in between 1 and 2 patients in 10 and included tachycardia (increased heart rate), palpitations, and irregular heart rhythms (atrial fibrillation, sinus tachycardia); more rarely patients had changes in electrical conduction in the heart (prolonged QTc interval). For the full list of all side effects reported with Farydak, see the package leaflet.

Farydak must not be used in women who are breast-feeding. For the full list of restrictions, see the package leaflet.

Why is Farydak approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) considered the increase in progression free survival to be clinically significant although it noted that a benefit in overall survival had not yet been shown. In addition, panobinostat works in a different way to existing treatments. This means that for patients who have previously received at least two previous treatments, including bortezomib and immunomodulatory agents, who have limited treatment options and therefore a high unmet medical need, it offers a new alternative. Although the side effects were of concern and could not be justified in patients who could be given less toxic treatments, the CHMP considered that they were acceptable in this previously treated subgroup in view of the lack of alternatives, and could be

managed. The CHMP therefore decided that Farydak's benefits are greater than its risks in this group and recommended that it be approved for use in the EU.

What measures are being taken to ensure the safe and effective use of Farydak?

A risk management plan has been developed to ensure that Farydak is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Farydak, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that markets Farydak will provide educational materials for patients, including a patient card, to help them take the medicine correctly. It will also provide a final analysis from the main study on how long patients who have been treated with the medicine survive.

Further information can be found in the summary of the risk management plan.

Other information about Farydak

The European Commission granted a marketing authorisation valid throughout the European Union for Farydak on 28 August 2015.

The full EPAR and risk management plan summary for Farydak can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Farydak, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Farydak can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 08-2015.