Farydak® (panobinostat)

This Horizons Infosheet contains information on Farydak (also known as panobinostat), a drug being investigated for the treatment of myeloma.

The Horizons Infosheet series provides information relating to novel drugs that are currently being investigated for the treatment of myeloma. The series also aims to highlight the considerable amount of research currently taking place in the field of myeloma drug development.

The drugs described in the Horizons Infosheets may not be licensed and/or approved for use in myeloma. You may, however, be able to access them as part of a clinical trial.

What is Farydak?

Farydak is a new drug being investigated for the treatment of myeloma.

Farydak (originally known as LBH589) belongs to a group of drugs known as histone deacetylase (HDAC) inhibitors.

What are histone deacetylases?

A histone deacetylase is a protein which changes the way other proteins, called histones, bind to DNA within cells. In carrying out this function, a histone deacetylase causes genes to be "switched off".

Myeloma cells contain an excess of histone deacetylases. This causes many of the genes involved in controlling cell growth to be switched off, which in turn allows myeloma cells to rapidly grow and multiply out of control.

How does Farydak work?

Farydak is a histone deacetylase inhibitor. This means that it works by blocking the action of histone deacetylase in myeloma cells, preventing their growth and survival.

There is also some evidence that Farydak may prevent the growth of new blood vessels, thereby reducing the supply of oxygen and nutrients to the myeloma cells. See Figure 1.

How is Farydak given?

Farydak is given orally as a capsule. Based on the recommendation of your doctor, the dose of Farydak is either 20 mg, 15 mg or 10 mg, taken over 21 days.

Farydak is always used together with two other drugs, Velcade® (bortezomib) and dexamethasone.

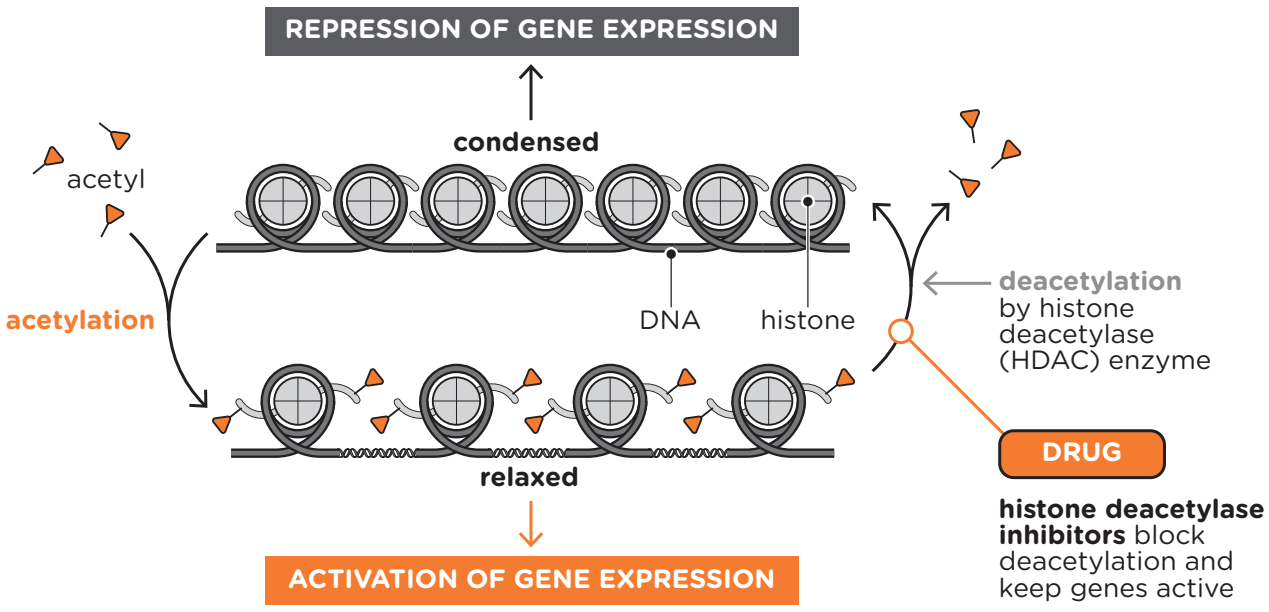

Figure 1. Mechanism of action of HDAC inhibitors

What evidence exists to support the use of Farydak?

Data from the Phase III PANORAMA-1 clinical trial in which Farydak in combination with Velcade and dexamethasone was compared to Velcade and dexamethasone alone, shows that patients receiving the Farydak combination had a longer progression free survival (the length of time following treatment before the myeloma starts to come back).

768 relapsed and/or refractory patients participated in the trial, and those who received the Farydak combination had an average progression free survival of 12 months compared to 7.8 months in patients who received Velcade and dexamethasone alone.

What are the possible known side-effects of Farydak?

The most common side-effects of Farydak that have been reported include: pneumonia, diarrhoea, low platelet counts, fatigue, low red blood cell counts, low white blood cell counts, and peripheral neuropathy (pain, tingling, or loss of feeling in the extremities).

Is Farydak currently available in any UK clinical trials?

For an up-to-date list of UK clinical trials involving Farydak, visit the **Myeloma Trial Finder** on **www.myeloma.org.uk.**

To be enrolled on a clinical trial, patients have to meet certain conditions known as eligibility criteria. You should speak to your doctor in the first instance if you are interested in taking part in a trial.

UK availability of Farydak

Before a drug can be widely used, it must first be licensed as a safe and effective treatment. This is usually done by regulatory authorities at a European level and involves a review of evidence from large-scale clinical trials.

Normally, the licensed drug must then be approved by a UK drug appraisal body before it can be routinely prescribed by NHS doctors. The drug appraisal process differs from licensing - it compares how effective the newly-licensed drug is to existing drugs already in use on the NHS and decides whether it offers the NHS good value for money. The main body responsible for carrying out drug appraisals

in England and Wales is the National Institute for Health and Care Excellence (NICE). NICE recommendations are usually adopted in Northern Ireland. Scotland's drug appraisal body is the Scottish Medicines Consortium (SMC).

Farydak has been approved by the European licensing body, the European Medicines Agency (EMA). In December 2015, Farydak was approved by NICE in combination with Velcade and dexamethasone for relapsed and/or refractory myeloma patients who have received at least two prior treatments, including Velcade and an immunomodulatory drugs (such as thalidomide or Revlimid®). It was approved by the SMC in February 2016.

Future directions

In addition to Velcade, thalidomide and dexamethasone, Farydak is also being studied in combination with other myeloma drugs such as Revlimid® (lenalidomide) and Kyprolis® (carfilzomib) in relapsed and/or refractory patients. It is also being studied in newly diagnosed patients in combination with Revlimid, Velcade and dexamethasone as an induction treatment prior to autologous stem cell transplantation.

These clinical trials will provide information about the safest and most effective way to use Farydak in myeloma.

About this Horizons Infosheet

The information in this Horizons Infosheet is not meant to replace the advice of your medical team. They are the people to ask if you have questions about your individual situation. All Myeloma UK publications are extensively reviewed by patients and healthcare professionals prior to publication.

Other information available from Myeloma UK

Myeloma UK has a range of Essential Guides, Infoguides and Infosheets available covering many areas of myeloma, its treatment and management.
To order your free copies or to talk to one of our Myeloma Information Specialists about any aspect of myeloma, call the **Myeloma Infoline: 0800 980 3332** or **1800 937 773** from Ireland.

The Myeloma Infoline is open from Monday to Friday, 9am to 5pm and is free to phone from anywhere in the UK and Ireland. From outside the UK and Ireland, call **0131 557 9988** (charged at normal rate).

Information and support about myeloma is also available around the clock at **www.myeloma.org.uk**

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