Pomalidomide Celgene® (pomalidomide)

Introduction

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 pomalidomide?

Pomalidomide is a drug that is being used to treat myeloma and belongs to a group of drugs known as immunomodulatory drugs (IMiDs).

Pomalidomide is the third type of IMiD to be used to treat myeloma after thalidomide and Revlimid® (lenalidomide). Pomalidomide is a chemical derivative of thalidomide.

How does pomalidomide work?

Pomalidomide works in a similar manner to thalidomide and Revlimid and therefore is likely to have several mechanisms of action against myeloma cells.

It acts mainly by encouraging the patient’s immune system to attack and destroy myeloma cells. It also prevents the growth of new blood vessels, thereby reducing the supply of oxygen and nutrients to the myeloma cells.

The fact that pomalidomide works in many ways is thought to be why it is so active against myeloma. Its possible mechanisms of action are shown in Figure 1.

How is pomalidomide given?

Pomalidomide is given as a capsule. The recommended starting dose of pomalidomide is 4mg taken once daily orally on days 1 – 21 of repeated 28-day cycles until disease progression.

Pomalidomide can be given alone or in combination with other anti-myeloma treatments such as dexamethasone.
What are the possible benefits of pomalidomide over existing drugs?

Pomalidomide is an IMiD and has similar mechanisms of action as other IMiDs such as thalidomide and Revlimid. However, pomalidomide is thought to have advantages over both thalidomide and Revlimid. So far, pomalidomide has been found to be much more potent than either thalidomide or Revlimid and therefore can be given at much lower doses.

Several international trials have shown that pomalidomide may be particularly effective for pre-treated patients who have become resistant (refractory) to some of the current drugs, including Revlimid and Velcade® (bortezomib).

In multiple clinical trials, response rates in patients who have had between one and five previous standard treatment combinations have been between 29 – 65%.

This indicates that pomalidomide may be a useful drug for patients who have been treated with several standard treatments already. No trials have yet investigated the use of pomalidomide in newly diagnosed patients.

What are the possible known side-effects of pomalidomide?

In clinical trials to date, pomalidomide has been shown to be generally well tolerated when used as a treatment for relapsed and/or refractory myeloma.

Some of the more common side-effects of pomalidomide reported include: low blood counts, increased blood clots, skin rash, constipation and peripheral neuropathy.

Is pomalidomide currently involved in any UK clinical trials?

Part of a large scale Phase III international clinical trial called the STRATUS study is currently ongoing in a number of hospitals in the UK and Ireland*.

This study is an evaluation of the safety of pomalidomide in combination with low-dose dexamethasone in myeloma patients who have relapsed from their previous treatment or who are refractory to treatment.

Patients who are interested in taking part should speak to their doctor in the first instance.
Currently, only relapsed and refractory myeloma patients are being recruited for pomalidomide trials. It is likely, however, that there will be further trials into pomalidomide in different patient groups taking place in the UK in the future.

*Recruiting or soon-to-be recruiting hospitals: Leeds Teaching Hospitals NHS Trust; New Cross Hospital, Wolverhampton; The Royal Marsden Hospital, Sutton, Surrey; The Newcastle upon Tyne Hospitals NHS Foundation Trust; The Christie NHS Foundation Trust, Manchester; Kent and Canterbury Hospital; The Royal Liverpool University Hospital; Southmead Hospital, Bristol; Belfast City Hospital; Cork University Hospital; Mater Misericordiae University Hospital, Dublin

Are there any patients pomalidomide is unsuitable for?

As with any treatment, pomalidomide may not be suitable for all patients. Your doctor will be able to discuss this with you if you are considering taking part in a pomalidomide trial.

UK availability of pomalidomide

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).

Pomalidomide was granted a licence for use in myeloma patients across Europe by the European licensing body in August 2013. It has been licensed for myeloma patients who have received at least two prior treatments, including Revlimid and Velcade, and whose myeloma has progressed whilst taking the last treatment.

Now that a European licence has been granted, pomalidomide will pass to the UK drug appraisal bodies to be assessed. A NICE assessment of a newly licensed drug typically takes around a year to complete, whereas the SMC takes around six months. At the end of this process, NICE and the SMC can issue either positive or negative guidance depending on their assessment of the clinical and cost-effectiveness of the drug. If the appraisal bodies issue positive guidance on a drug, it is usually made available by the NHS within three months.

The future

There are several ongoing clinical trials further investigating the effectiveness of pomalidomide in patients with relapsed and/or refractory myeloma.

Pomalidomide is also being studied in combination with other treatments such as dexamethasone, cyclophosphamide, prednisolone, Velcade and dexamethasone.

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

Trials investigating pomalidomide as an initial myeloma treatment are expected to follow soon.
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 call our Myeloma Infoline on 0800 980 3332 or 1800 937 773 from Ireland. This information is also available on our website at www.myeloma.org.uk

To talk to one of our Myeloma Information Specialists about any aspect of myeloma, call the Myeloma Infoline on 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, call +44 (0)131 557 9988 (charged at normal rate). Information and support about myeloma is also available around the clock at www.myeloma.org.uk

About Myeloma UK

Myeloma UK is the only organisation in the UK dealing exclusively with myeloma.

Our broad and innovative range of services cover every aspect of myeloma from information and support to improving standards of treatment and care through research, education, campaigning and raising awareness.

Our strategy is to take an integrated approach to systematically address the barriers and challenges that are slowing down myeloma research and the development of, and access to, new treatments, optimal care, information and support.

We receive no government funding and rely almost entirely on voluntary donations and fundraising activities.

With Myeloma UK you can...

- Call our Myeloma Infoline on 0800 980 3332 or 1800 937 773 from Ireland for information, practical advice, emotional support and a listening ear
- Get free Infopacks, Infoguides and Infosheets about myeloma
- Learn about myeloma from experts and meet others affected by myeloma by attending Patient and Family Myeloma Infodays
- Subscribe to our newsletter Myeloma Matters
- Visit our website www.myeloma.org.uk
- Join a Myeloma Support Group

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