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MYELOMA PATIENTS EUROPE

BREAKING NEWS

POMALIDOMIDE (POMALYST®) APPROVED BY FDA FOR USE IN THE UNITED STATES

The United States (US) Food and Drug Administration (FDA) has approved pomalidomide (brand name Pomalyst®) for use in myeloma patients with relapsed and refractory myeloma. This is the second new treatment to be approved in this setting by the FDA in the past 6 months, with Kyprolis™ approved for use in August last year.

Pomalyst® is an immunomodulatory drug (IMiD) that works in a similar way to thalidomide and Revlimid®. It is specifically indicated for patients who have received at least two prior treatments, including Revlimid® (lenalidomide) and Velcade® (bortezomib) and whose myeloma did not respond to treatment and progressed within 60 days of the last treatment.

Speaking about the announcement, Sarper Diler, Chairman, MPE said,

"The approval of Pomalyst® is very positive news indeed. However, it is still not approved in Europe. What is more, some countries in Europe still don't have access to thalidomide or Revlimid®. This is clearly unacceptable. MPE will shortly announce its plans to try to ensure equity in access to effective myeloma treatments."

Q&A

Why was Pomalyst® approved by the FDA?

A licence for Pomalyst® was granted by the FDA through its accelerated approval process on the strength of positive results from the 'MM-003' Phase III clinical study. As part of the accelerated approval process, the FDA licence can be updated to take into account new data on Pomalyst® as more becomes available from ongoing studies.

The MM-003 study, involving 455 patients, compared the effectiveness of Pomalyst® in combination with patients who had low-dose dexamethasone to high-dose dexamethasone alone in relapsed and refractory myeloma patients who had previously been treated with both Revlimid® and Velcade®.

The results showed that Pomalyst® in combination with low-dose dexamethasone significantly improved progression-free survival and had an overall survival advantage compared to high-dose dexamethasone alone.

A conference report from ASH will be available soon to MPE members.

What are the benefits of Pomalyst®?

As described above, Pomalyst® is an immunomodulatory drug (IMiD) that has a similar mechanism of action to thalidomide and Revlimid® – both are commonly used anti-myeloma treatments.

However, data from a number of different studies show that it is more potent and is effective even in relapsed and refractory patients who no longer respond to thalidomide, Revlimid® and Velcade®. The approval of Pomalyst® is therefore very promising for this group of patients.

The MM-003 study also found that Pomalyst® is associated with a better side-effect profile with fewer incidences of thrombocytopenia (low platelet count) and peripheral neuropathy than with Revlimid® and thalidomide. There was, however, a higher incidence of neutropenia (low white blood cell count) in patients receiving Pomalyst® compared to those receiving high-dose dexamethasone alone with levels comparable to Revlimid®.

What does the FDA approval of Pomalyst® mean for myeloma patients in Europe?

The FDA approval of Pomalyst does not apply to myeloma patients in Europe, as the FDA only covers decisions on new treatments for patients in the US. In Europe, new treatments must be approved by the European Medicines Agency (EMA) before they can become available. However, a positive FDA decision is a strong indication of a positive EMA approval.

An application for a licence covering Pomalyst® was submitted to the EMA in June 2012 and a decision is expected in the first quarter of 2013. Look out for an update on our website as soon as a decision has been reached.

In the interim, how can I access Pomalyst®?

You should contact your doctor or local MPE member group to find out about availability in your country, region or hospital. As Pomalyst® is not yet licensed in Europe, access will most likely be via a clinical study. It might also be worth contacting the Celgene affiliate in your country. You can find more information about clinical trials with pomalidomide on the European clinical trials website: **www.clinicaltrialsregister.eu**

Where can I find more information about Pomalyst®?

If you have any questions or comments about Pomalyst® and the recent FDA approval, please contact: **sarper.diler@myelomapatientseurope.org** or **eric.low@myelomapatientseurope.org**