



14 October 2014  
EMA/351975/2013 Rev.1  
Committee for Orphan Medicinal Products

## Recommendation for maintenance of orphan designation at the time of marketing authorisation

Imnovid (pomalidomide) for the treatment of multiple myeloma

First publication	13 August 2013
Rev.1: administrative update	14 October 2014
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

During its meeting of 11-13 June 2013, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/09/672 for Imnovid, previously Pomalidomide Celgene, (pomalidomide) as an orphan medicinal product for the treatment of multiple myeloma. The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other satisfactory methods of treatment. As other methods of treatment for patients with this condition are authorised in the European Union (EU), the COMP also looked at the significant benefit of the product over existing treatments. The COMP recommended that the orphan designation of the medicine be maintained<sup>1</sup>.

### Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Imnovid:

‘in combination with dexamethasone for the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior treatment regimens, including both lenalidomide and bortezomib, and have demonstrated disease progression on the last therapy’.

<sup>1</sup> The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with a comparable therapeutic indication cannot be placed on the market.



This falls within the scope of the product's designated orphan indication, which is 'treatment of multiple myeloma'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Multiple myeloma remains a debilitating and life-threatening disease because it disrupts the normal functioning of the bone marrow, leads to bone destruction and causes kidney failure.

### **Prevalence of the condition**

The sponsor provided updated information on the prevalence of multiple myeloma based on data from the Globocan 2008 database.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of multiple myeloma remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was estimated to be approximately 1.3 people in 10,000. This is equivalent to a total of around 66,000 people in the EU.

### **Existence of other satisfactory methods of treatment**

At the time of the review of the orphan designation, several medicines were authorised for multiple myeloma in the EU, including bortezomib, lenalidomide and thalidomide. They were usually combined with steroids to reduce the activity of the immune system, the body's natural defences. Where these medicines did not work, some patients received an allogeneic stem-cell transplant (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

### **Significant benefit over existing treatments**

The COMP concluded that the claim of a significant benefit of Imnovid in multiple myeloma is justified because of its demonstrated effectiveness in patients whose disease did not respond to or had come back after previous treatment, including bortezomib and lenalidomide. These patients have very limited treatment options.

The COMP conclusions are supported by data from 3 studies in this group of patients, which showed that Imnovid was effective at delaying the progression of multiple myeloma.

Therefore, although other satisfactory methods for the treatment of multiple myeloma have been authorised in the EU, the COMP concluded that Imnovid is of significant benefit for patients with this condition.

### **Conclusions**

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Imnovid still meets the criteria for designation as an orphan medicinal product and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Imnovid can be found in the European public assessment report (EPAR) on the Agency's website: [ema.europa.eu/Find\\_medicine/Human\\_medicines/European\\_public\\_assessment\\_reports](http://ema.europa.eu/Find_medicine/Human_medicines/European_public_assessment_reports).