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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pomalidomide for the treatment of primary myelofibrosis

On 27 July 2010, orphan designation (EU/3/10/757) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for pomalidomide for the treatment of primary myelofibrosis.

What is primary myelofibrosis?

Primary myelofibrosis is a disease of unknown cause in which the bone marrow (the spongy tissue inside the large bones) becomes dense and fibrous, and starts producing abnormal immature blood cells that replace the normal blood cells.

Some immature blood cells migrate from the bone marrow to other organs, such as the spleen and liver, where they mature. This causes the organs to become enlarged. Patients with primary myelofibrosis can develop several symptoms, including pain in the bones, fever, tiredness, weakness, weight loss, infections and bleeding.

Primary myelofibrosis is a debilitating disease that is long lasting and may be life threatening because it can lead to severe anaemia (low red blood cell counts) and infections, and can result in leukaemia (cancer of the white blood cells).

What is the estimated number of patients affected by the condition?

At the time of designation, primary myelofibrosis affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 15,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, hydroxyurea and busulfan (which are also used to treat cancer) were authorised in the EU for primary myelofibrosis. In addition, treatments aimed at relieving the symptoms of the disease were used. These included androgens (male hormones), glucocorticoids (a

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 506,500,000 (Eurostat 2010).

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type of steroid) and erythropoietin (a hormone that stimulates the production of red blood cells) to treat anaemia, and surgery or radiation to remove or shrink the enlarged spleen. In some patients, bone marrow transplantation was used. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with bone marrow from a matched donor.

The sponsor has provided sufficient information to show that pomalidomide might be of significant benefit for patients with primary myelofibrosis because it might improve the treatment of patients with this condition by relieving anaemia. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Pomalidomide is an immunomodulating agent. This means that it affects the activity of the immune system. The way that pomalidomide will work in primary myelofibrosis is not known, but it is expected to block the growth of the abnormal cells in the bone marrow, while allowing normal cells to grow, including the cells that produce red blood cells. This is expected to improve anaemia in patients with the condition.

What is the stage of development of this medicine?

The effects of pomalidomide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pomalidomide in patients with myelofibrosis were ongoing.

At the time of submission, pomalidomide was not authorised anywhere in the EU for primary myelofibrosis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 April 2010 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pomalidomide	Treatment of primary myelofibrosis
Bulgarian	Помалидомид	Лечение на първична миелофиброза
Czech	Pomalidomid	Léčba primárnímyelofibrózy
Danish	Pomalidomid	Behandling af primær myelofibrose
Dutch	Pomalidomide	Behandeling van primaire myelofibrose
Estonian	Pomalidomiid	Esmase müelofibroosi ravi
Finnish	Pomalidomidi	Primaarisen myelofibroosin hoito
French	Pomalidomide	Traitement de la myélofibrose primitive
German	Pomalidomid	Behandlung der primären Myelofibrose
Greek	Πομαλιδομίδη	Θεραπεία της πρωτογενούς μυελοσκλήρυνσης
Hungarian	Pomalidomid	Primer mielofibrózis kezelésére
Italian	Pomalidomide	Trattamento della mielofibrosi primitiva
Latvian	Pomalidomīds	Primāras mielofibrozes ārstēšana
Lithuanian	Pomalidomidas	Pirminės mielofibrozės gydymas
Maltese	Pomalidomide	Kura tal-mjelofibrożi primarja
Polish	Pomalidomid	Leczenie mielofibrozy pierwotnej
Portuguese	Pomalidomida	Tratamento da mielofibrose primária
Romanian	Pomalidomidă	Tratamentul mielofibrozei primitive
Slovak	Pomalidomid	Liečba primárnej myelofibrózy
Slovenian	Pomalidomid	Zdravljenje primarne mielofibroze
Spanish	Pomalidomida	Tratamiento de la mielofibrosis primaria
Swedish	Pomalidomid	Behandling av primär myelofibros
Norwegian	Pomalidomid	Behandling av primær myelofibrose
Icelandic	Pómalídómíð	Meðferð á beinmergsnetjuhersli

[1] At the time of designation