20 June 2011
EMA/COMP/249478/2010 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pomalidomide for the treatment of post-essential thrombocythaemia myelofibrosis

On 27 July 2010, orphan designation (EU/3/10/759) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for pomalidomide for the treatment of post-essential thrombocythaemia myelofibrosis.

What is post-essential thrombocythaemia myelofibrosis?

Myelofibrosis is a disease 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. It can develop as a reaction to essential thrombocythaemia (overproduction of platelets, components that help the blood to clot). 'Essential' means that the thrombocythaemia is not caused by any known condition.

In myelofibrosis, 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 myelofibrosis can develop several symptoms, including pain in the bones, fever, tiredness, weakness, weight loss, infections and bleeding.

Post-essential thrombocythaemia 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, post-essential thrombocythaemia myelofibrosis affected less than 0.01 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 500 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).

[*]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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What treatments are available?

At the time of designation, although hydroxyurea and busulfan were authorised in the EU for primary myelofibrosis (myelofibrosis of unknown cause), there were no treatments specifically authorised for post-essential thrombocythaemia myelofibrosis.

Treatments for this disease were aimed at relieving symptoms. They included androgens (male hormones), glucocorticoids (a 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.

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 post-essential thrombocythaemia 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 this 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 post-essential thrombocythaemia 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 post-essential thrombocythaemia myelofibrosis
Bulgarian	Помалидомид	Лечение на миелофиброза след есенциална тромбоцитемия
Czech	Pomalidomid	Léčba post-esenciální trombocytémické myelofibrózy
Danish	Pomalidomid	Behandling af post essentiel thrombocythæmi myelofibrose
Dutch	Pomalidomide	Behandeling van myelofibrosis volgend op essentiële trombocytemie
Estonian	Pomalidomiid	Postessentsiaalse trombotsüteemia müelofibroosi ravi
Finnish	Pomalidomidi	Essentiaalisen trombosytemian jälkeisen myelofibroosin hoito
French	Pomalidomide	Traitement de la myélofibrose consécutive à une thrombocytémie essentielle
German	Pomalidomid	Behandlung einer Myelofibrose nach essentieller Thrombozythämie
Greek	Πομαλιδομίδη	Θεραπεία της μυελοϊνωσης από ιδιοπαθή θρομβοκυττάρωση
Hungarian	Pomalidomid	Esszenciális thrombocytaemiát követő mielofibrózis kezelésére
Italian	Pomalidomide	Trattamento della mielofibrosi post-trombocitemia essenziale
Latvian	Pomalidomīds	Pēc-esenciālas trombocitēmijas mielofibrozes ārstēšana
Lithuanian	Pomalidomidas	Mielofibrozės gydymas po esencialinės trombocitemijos
Maltese	Pomalidomide	Kura tal-mjelofibrożi konsegwenti għal trombożitemija essenzjali
Polish	Pomalidomid	Leczenie mielofibrozy wywołanej nadpłytkowością samoistną
Portuguese	Pomalidomida	Tratamento da mielofibrose devida a trombocitémia essencial
Romanian	Pomalidomidă	Tratamentul mielofibrozei post-trombocitemie esenţială
Slovak	Pomalidomid	Liečba myelofibrózy po esenciálnej trombocytémii
Slovenian	Pomalidomid	Zdravljenje mielofibroze, nastale po esecialni trombocitemiji
Spanish	Pomalidomida	Tratamiento de la mielofibrosis secundaria a trombocitemia esencial
Swedish	Pomalidomid	Behandling av post-essentiell trombocytemi myelofibros
Norwegian	Pomalidomid	Behandling av myelofibrose sekundært til essensiell trombocytemi
Icelandic	Pómalídómíð	Meðferð á mýelófíbrósu í kjölfar eðlislægs blóðflagnadreyra

[1] At the time of designation