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EMA/COMP/168763/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Dexamethasone (40 mg tablet) for the treatment of multiple myeloma

On 9 June 2010, orphan designation (EU/3/10/745) was granted by the European Commission to Laboratoires C.T.R.S., France, for dexamethasone (40 mg tablet) for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called plasma cells. Plasma cells are found in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes out of control, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, multiple myeloma affected not more than 2.2 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of not more than 111,000 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, several medicines were authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with high-dose dexamethasone. Radiotherapy (treatment with radiation) was considered to be very

[*]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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useful in treating pain and weakened bones. Interferon alfa, a protein normally produced by the body during viral infections, was sometimes used in combination with chemotherapy.

The sponsor has provided sufficient information to show that dexamethasone (40 mg tablet) might be of significant benefit for patients with multiple myeloma because this high-dose formulation will allow patients to take only one tablet instead of the multiple tablets they take with the currently available formulations, which contain between 0.5 and 8 mg dexamethasone. In addition, it might offer a wider availability than the existing formulations, which are not available in some EU countries. These two elements are expected to result in a major contribution to patient care. These assumptions 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?

Dexamethasone belongs to a group of medicines known as corticosteroids, which reduce the activity of the immune system (the body's natural defences) by attaching to receptors in various types of immune cells. In multiple myeloma, high-dose dexamethasone is used together with chemotherapy to make chemotherapy more effective and to reduce certain side effects of cancer treatment, such as nausea (feeling sick) and vomiting.

What is the stage of development of this medicine?

The sponsor of this application has not conducted any studies in experimental models with dexamethasone (40 mg tablet). However, it provided the results of studies on the effect of high-dose dexamethasone from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, no clinical trials with dexamethasone (40 mg tablet) in patients with multiple myeloma had been started.

At the time of submission, dexamethasone (40 mg tablet) was not authorised anywhere in the EU for multiple myeloma 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 3 March 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

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

Language	Active ingredient	Indication
English	Dexamethasone (40 mg tablet)	Treatment of multiple myeloma
Bulgarian	Дексаметазон (Таблетка 40 мг.)	Лечение на мултиплен миелом
Czech	Dexametazon (40 mg tablety)	Léčba mnohočetného myelomu
Danish	Dexamethason (40 mg tablet)	Behandling af multipelt myelom
Dutch	Dexamethasone (40 mg tablet)	Behandeling van multipel myeloom
Estonian	Deksametasoon (40 mg tablett)	Multiibelse müeloomi ravi
Finnish	Deksametasoni (40 mg tabletti)	Multippeli myelooman hoito
French	Dexaméthasone (comprimé de 40 mg)	Traitement du myélome multiple
German	Dexamethason (40 mg Tablette)	Behandlung des multiplen Myeloms
Greek	Δεξαμεθαζόνη (δισκία 40mg)	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	Dexametazon (40 mg tabletta)	Myeloma multiplex kezelése
Italian	Desametasone (compressa da 40 mg)	Trattamento del mieloma multiplo
Latvian	Deksametazonas (40 mg tab)	Multiplās mielomas ārstēšana
Lithuanian	Deksametazonas (40mg tabletė)	Dauginės mielomos gydymas
Maltese	Dexamethasone (pillola ta' 40mg)	Kura tal-mjeloma multipla
Polish	Deksametazon (tabletka 40 mg)	Leczenie szpiczaka mnogiego
Portuguese	Dexametasona (comprimidos de 40mg)	Tratamento do mieloma múltiplo
Romanian	Dexametazonă (tablete a 40 mg)	Tratamentul mielomului multiplu
Slovak	Dexametazón (40 mg tablety)	Liečba mnohopočetného myelómu
Slovenian	Deksametazon (40 mg tableta)	Zdravljenje multiplega mieloma
Spanish	Dexametasona (40 mg comprimido)	Tratamiento del mieloma múltiple
Swedish	Dexametason (40 mg tablett)	Behandling av multipelt myelom
Norwegian	Deksametason (40 mg tablet)	Behandling av myelomatose
Icelandic	Dexametasóni (40 mg tafla)	Meðferð við mergfrumuæxli

[1] At the time of designation