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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Daratumumab for the treatment of plasma cell myeloma

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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.	

On 17 July 2013, orphan designation (EU/3/13/1153) was granted by the European Commission to Janssen-Cilag International N.V., Belgium, for daratumumab for the treatment of plasma cell myeloma.

What is plasma cell myeloma?

Plasma cell myeloma is a cancer of a type of white blood cells called plasma cells. Plasma cells originate from the bone marrow, the spongy tissue inside the large bones in the body. In plasma cell 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.

Plasma cell myeloma is a debilitating and life-threatening disease because it disrupts the normal functioning of the bone marrow, leads to bone lesions and causes kidney failure.

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

At the time of designation, plasma cell myeloma affected approximately 1.75 in 10,000 people in the European Union (EU). This was equivalent to a total of around 90,000 people*, and is below the ceiling 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 28), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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What treatments are available?

At the time of designation, several medicines were already authorised for plasma cell myeloma in the EU. The main treatment for plasma cell myeloma was chemotherapy (medicines to treat cancer) usually combined with steroids to reduce the activity of the immune system, the body's natural defences. Where chemotherapy 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). Radiotherapy (using radiation to kill cancer cells) was used to treat pain and weakened bones. Interferon alfa was sometimes used in combination with chemotherapy.

The sponsor has provided sufficient information to show that daratumumab might be of significant benefit for patients with plasma cell myeloma because early studies have shown that it may improve the outcome of patients whose disease does not respond to or has come back after other treatments. 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?

Daratumumab is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (an antigen) on the myeloma cells called 'CD38'. Once attached, it is expected to activate the immune system to attack and kill the myeloma cells.

What is the stage of development of this medicine?

The effects of daratumumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with daratumumab in patients with plasma cell myeloma were ongoing.

At the time of submission, daratumumab was not authorised anywhere in the EU for plasma cell myeloma. Orphan designation of daratumumab had been granted in the United States for treatment of the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 June 2013 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	Daratumumab	Treatment of plasma cell myeloma
Bulgarian	даратумумаб	Лечение на плазмоцитен миелом
Croatian	Daratumumab	Liječenje multiplog mijeloma
Czech	Daratumumab	Léčba myelomu
Danish	Daratumumab	Behandling af plasmacellsmyelom
Dutch	Daratumumab	Behandeling van plasmacel myeloom
Estonian	Daratumumab	Plasmarakulise müeloomi ravi
Finnish	Daratumumabi	Plasmasolumyelooman hoito
French	Daratumumab	Traitement du myélome des cellules plasmatiques
German	Daratumumab	Behandlung des Plasmazell Myeloms
Greek	Δαρατουμουμάμπη	Θεραπεία του πλασματοκυτταρικού μυελώματος
Hungarian	Daratumumab	Plasma sejtes myeloma kezelése
Italian	Daratumumab	Trattamento del Mieloma Plasmacellulare
Latvian	Daratumumabs	Plazmas šūnu mielomas ārstēšana
Lithuanian	Daratumumabas	Plazminių ląstelių mielomos gydymas
Maltese	Daratumumab	Kura tal-mjeloma taċ-ċelluli tal-plasma
Polish	Daratumumab	Leczenie szpiczaka mnogiego
Portuguese	Daratumumab	Tratamento do mieloma de células plasmáticas
Romanian	Daratumumab	Tratamentul mielomului plasmocitar
Slovak	Daratumumab	Liečba myelómu z plazmatických buniek
Slovenian	Daratumumab	Zdravljenje plazmocitoma
Spanish	Daratumumab	Tratamiento del mieloma de células plasmáticas
Swedish	Daratumumab	Behandling av plasmacellsmyelom
Norwegian	Daratumumab	Behandling av plasmacellemyelom
Icelandic	Daratúmúmab	Meðferð ´plamergkrabameinismafrumu

[1] At the time of designation