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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised monoclonal antibody against CD38 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 29 April 2014, orphan designation (EU/3/14/1268) was granted by the European Commission to Sanofi-Aventis Groupe, France, for humanised monoclonal antibody against CD38 for the treatment of plasma cell myeloma.

What is plasma cell myeloma?

Plasma cell myeloma (also called multiple myeloma) is a cancer of a type of white blood cell 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 the 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 particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

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

At the time of designation, plasma cell myeloma affected approximately 1.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 92,000 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).

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 due to bone damage and prevent further damage. Interferon alfa was sometimes used in combination with chemotherapy.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit to patients with plasma cell myeloma because it works in a different way to existing treatments and early studies showed some improvements in patients whose cancer had come back or did not respond to standard treatment. 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?

This medicine is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific target. The target for this medicine is a protein called CD38, which is present on the surface of many white blood cells but occurs in higher amounts in some blood cancers, including plasma cell myeloma. By attaching to CD38 on the cancerous plasma cells, the medicine is expected to activate certain components of the immune system so that they kill the cancerous plasma cells. It is also expected to interfere with cell survival thus causing the death of the cancer cells. This is expected to slow down the development of plasma cell myeloma.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for plasma cell 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 12 March 2014 recommending the granting of this designation.

*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 512,900,000 (Eurostat 2014).

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	Humanised monoclonal antibody against CD38	Treatment of plasma cell myeloma
Bulgarian	Хуманизирано моноклонално антитяло срещу CD38	Лечение на плазмоцитен миелом
Croatian	Humanizirano monoklonsko protutijelo protiv CD38	Liječenje multiplog mijeloma
Czech	Humánní monoklonální protilátka proti CD38	Léčba myelomu
Danish	Humaniseret monoklonalt antistof mod CD38	Behandling af plasmacellsmyelom
Dutch	Gehumaniseerd monoklonaal antilichaam tegen CD38	Behandeling van plasmacel myeloom
Estonian	Humaniseeritud CD38 vastane monoklonaalne antikeha	Plasmarakulise müeloomi ravi
Finnish	Humanisoitu monoklonaalinen CD38:n vasta-aine	Plasmasolumyelooman hoito
French	Anticorps monoclonal humanisé anti-CD38	Traitement du myélome des cellules plasmatiques
German	Humanisierter monoklonaler Antikörper gegen CD38	Behandlung des Plasmazell Myeloms
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι του CD38	Θεραπεία του πλασματοκυτταρικού μυελώματος
Hungarian	CD38 ellenes humanizált monoklonális antitest	Plasma sejtes myeloma kezelése
Italian	Anticorpo monoclonale umanizzato anti-CD38	Trattamento del Mieloma Plasmacellulare
Latvian	Humanizēta monoklonāla antiviela pret CD38	Plazmas šūnu mielomas ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas prieš CD38	Plazminių ląstelių mielomos gydymas
Maltese	Antikorp monoklonali umanizzat kontra CD38	Kura tal-mjeloma taċ-ċelluli tal-plasma
Polish	Humanizowane przeciwciało monoklonalne przeciw CD38	Leczenie szpiczaka mnogiego
Portuguese	Anticorpo monoclonal humanizado anti CD38	Tratamento do mieloma de células plasmáticas
Romanian	Anticorp monoclonal umanizat anti-CD38	Tratamentul mielomului plasmocitar
Slovak	Humanizovaná monoklonálna protilátka proti CD38	Liečba myelómu z plazmatických buniek
Slovenian	humanizirano monoklonsko protitelo proti CD38	Zdravljenje plazmocitoma

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Anticuerpo monoclonal humano anti-CD38	Tratamiento del mieloma de células plasmáticas
Swedish	Humaniserad monoklonal antikropp mot CD38	Behandling av plasmacellsmyelom
Norwegian	Humanisert monoklonalt antistoff mot CD38	Behandling av plasmacellemyelom
Icelandic	Mannaaðlagað einstofna mótefni gegn CD38	Meðferð ´plamergkrabameinismafrumu