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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human monoclonal IgM antibody targeting glucose regulated protein 78 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 7 October 2013, orphan designation (EU/3/13/1190) was granted by the European Commission to Patrys GmbH, Germany, for recombinant human monoclonal IgM antibody targeting glucose regulated protein 78 for the treatment of plasma cell myeloma.

What is plasma cell myeloma?

Plasma cell myeloma (previously 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 because it disrupts the normal functioning of the bone marrow, leads to bone lesions 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 and weakened bones. Interferon alfa was sometimes used in combination with chemotherapy.

The sponsor has provided sufficient information to show that the medicine 'recombinant human monoclonal IgM antibody targeting glucose regulated protein 78' might be of significant benefit for patients with plasma cell myeloma because it works in a different way to existing treatments and early studies have shown that it might improve the outcome of patients with this condition. 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 the glucose-regulated protein 78, which is present on the surface of cancer cells but not on the surface of normal cells. Once attached to the protein, the medicine activates a cascade of events that are toxic to the cancer cell, ultimately leading to its death.

What is the stage of development of this medicine?

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

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

At the time of submission, this 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 4 September 2013 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 512,200,000 (Eurostat 2013).

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	Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78	Treatment of plasma cell myeloma
Bulgarian	Рекомбинантно човешко моноклонално антитяло IgM насочено към глюкоза регулиран протеин 78	Лечение на плазмоцитен миелом
Czech	Rekombinantní lidská monoklonální IgM protilátka cílená proti glukózou regulovaného proteinu 78	Léčba myelomu
Croatian	Rekombinantno ljudsko monoklonsko IgM protutijelo protiv proteina reguliranog glukozom 78	Liječenje multiplog mijeloma
Danish	Rekombinant humant monoklonalt IgM-antistof rettet mod glucose reguleret protein 78	Behandling af plasmacellsmyelom
Dutch	Recombinant humaan monoklonaal IgM antilichaam gericht tegen glucose gereguleerd proteïne 78	Behandeling van plasmacel myeloom
Estonian	Rekombinantne inimese monoklonaalne IgM antikeha suunatud glükoosi reguleeritud valgu 78 vastu	Plasmarakulise müeloomi ravi
Finnish	Rekombinantti ihmisen monoklonaalinen IgM-vasta-aine glukoosisäätöiselle proteiini 78:lle	Plasmasolumyelooman hoito
French	Anticorps IgM recombinant monoclonal humain ciblant la protéine 78 régulée par le glucose	Traitement du myélome des cellules plasmatiques
German	Rekombinanter humaner monoklonaler IgM Antikörper gegen Glukose reguliertes Protein 78	Behandlung des Plasmazell Myeloms
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα IgM στόχευσης γλυκόζης ρυθμιζόμενης πρωτεΐνης 78	Θεραπεία του πλασματοκυτταρικού μυελώματος
Hungarian	Glükóz-regulált fehérje 78 (GRP78)-t célzó rekombináns humán monoklonális IgM ellenanyag	Plasma sejtes myeloma kezelése
Italian	Anticorpo monoclonale ricombinante umano IgM contro proteina regolata dal glucosio 78	Trattamento del Mieloma Plasmacellulare
Latvian	Rekombinantās cilvēka monoklonāla IgM antivielas pret glikozes regulēto olbaltumvielu 78	Plazmas šūnu mielomas ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Rekombinantinis žmogaus monokloninis IgM antikūnas nukreiptas į gliukozę reguliuojantį baltymą 78	Plazminių ląstelių mielomos gydymas
Maltese	Antikorp IgM monoklonali uman rikombinanti immirat għall-proteina 78 irregolata mill-glukożju	Kura tal-mjeloma taċ-ċelluli tal-plasma
Polish	Rekombinowane ludzkie przeciwciało monoklonalne IgM przeciw regulowanemu glukozą białku 78	Leczenie szpiczaka mnogiego
Portuguese	Anticorpo monoclonal humano recombinante da classe IgM anti proteína 78 glucose-regulada	Tratamento do mieloma de células plasmáticas
Romanian	Anticorp IgM monoclonal uman recombinant împotriva proteinei 78 reglata de glucoza	Tratamentul mielomului plasmocitar
Slovak	Rekombinantná ľudská monoklonálna IgM protilátka namie rená proti glukózou regulovanému proteín u 78	Liečba myelómu z plazmatických buniek
Slovenian	Rekombinantno humano monoklonsko IgM protitelo proti proteinu 78 , reguliranemu z glukozo	Zdravljenje plazmocitoma
Spanish	Anticuerpo monoclonal humano recombinante de la clase IgM anti proteína 78 glucosa regulada	Tratamiento del mieloma de células plasmáticas
Swedish	Rekombinant human monoklonal IgM antikropp riktad mot glukosregulerat protein 78	Behandling av plasmacellsmyelom
Norwegian	Rekombinant humant monoklonalt IgM antistoff rettet mot glukoseregulert protein 78	Behandling av plasmacellemyelom
Icelandic	Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78Raðbrigða, manna einstofna IgM mótefni sem beinist að glúkósa-stýrðu próteini 78	Meðferð ´plamergkrabameinismafrumu