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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Synthetic signal peptide of human mucin-1 (amino acids 1-21) 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 15 January 2015, orphan designation (EU/3/14/1423) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd, United Kingdom, for synthetic signal peptide of human mucin-1 (amino acids 1-21) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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

At the time of designation, plasma cell myeloma affected approximately 3.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 184,000 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 corticosteroids 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 for patients with plasma cell myeloma because it works in a different way to existing treatments and early studies have shown that it might be beneficial for those patients who had cancer cells left in the body despite previous 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?

Human mucin-1 is a protein that is found on the surface of the cancerous cells in plasma cell myeloma but is rarely found on normal plasma cells. A large part of the mucin-1 protein is released into the blood. This medicine is made up of the short part of human mucin-1, which appears only on the cancer cells but not in the blood. When this medicine is given to patients, the immune system (the body's natural defences) is expected to recognise it as 'foreign'. This is expected to stimulate an immune response against human mucin-1 on the cancer cells, resulting in the immune system attacking and killing them.

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 11 December 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. This represents a population of 511,100,000 (Eurostat 2014).

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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:

Richardson Associates Regulatory Affairs Ltd
Tripps Farmhouse Lower End
Great Milton
Oxfordshire OX44 7NJ
United Kingdom
Tel. +44 (0)1844 279 821
E-mail: mark.richardson@richardsonassociatesra.com

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	Synthetic signal peptide of human mucin-1 (amino acids 1-21)	Treatment of plasma cell myeloma
Bulgarian	Синтетичен сигнален пептид на човешки муцин-1 (аминокиселини 1-21)	Лечение на плазмоцитен миелом
Croatian	Sintetički signalni peptid ljudskog mucina-1 (aminokiseline 1-21)	Liječenje multiplog mijeloma
Czech	Syntetický signální peptid lidského mucinu-1 (aminokyseliny 1-21)	Léčba myelomu
Danish	Syntetisk signalpeptid af humane mucin-1 (aminosyrerne 1-21)	Behandling af plasmacellemyelom
Dutch	Synthetisch signaalpeptide van humaan mucine-1 (aminozuren 1-21)	Behandeling van plasmacel myeloom
Estonian	Inimese mutsiin-1 sünteetiline signaalpeptiid (aminohapped 1-21)	Plasmarakulise müeloomi ravi
Finnish	Synteettinen signaalipeptidi ihmisen musiini-1 (aminohapot 1-21)	Plasmasolumyelooman hoito
French	Peptide-signal synthétique de mucine- 1 humaine (acides aminés 1-21)	Traitement du myélome des cellules plasmatiques
German	Synthetisches Signalpeptid von menschlichem Mucin-1 (Aminosäuren 1-21)	Behandlung des Plasmazell Myeloms
Greek	Συνθετικό πεπτίδιο σήματος της ανθρώπινης βλεννίνης-1 (αμινοξέα 1-21)	Θεραπεία του πλασματοκυτταρικού μυελώματος
Hungarian	Humán mucin-1 (1-21 aminosavak) szintetikus szignál peptidje	Plasma sejtes myeloma kezelése
Italian	Peptide segnale sintetico della mucina-1 (aminoacidi 1-21) umana	Trattamento del mieloma plasmacellulare
Latvian	Cilvēka mucīna-1 sintētiskais signālpeptīds (aminoskābes 1-21)	Plazmas šūnu mielomas ārstēšana
Lithuanian	Sintetinis signalinis žmogaus mucino-1 (aminorūgščių 1-21) peptidas	Plazminių ląstelių mielomos gydymas
Maltese	Peptide tas-sinjal sintetiku ta' mucin-1 uman (aċidi amminiċi 1-21)	Kura tal-mjeloma taċ-ċelluli tal-plasma
Polish	Syntetyczny peptyd sygnałowy ludzkiej mucyny-1 (aminokwasy 1-21)	Leczenie szpiczaka mnogiego
Portuguese	Péptido sinal sintético da mucina-1 humana (aminoácidos 1-21)	Tratamento do mieloma de células plasmáticas
Romanian	Peptidă semnal sintetică a mucin-1 umana (aminoacizii 1-21)	Tratamentul mielomului plasmocitar
Slovak	Syntetický signálny peptid ľudskéhoMucínu-1 (aminokyseliny 1-21)	Liečba myelómu z plazmatických buniek

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Sintetični signalni peptid človeškega mucina-1 (aminokisline 1-21)	Zdravljenje plazmocitoma
Spanish	Péptido señal sintético de (aminoácidos 1-21) humanos mucina-1	Tratamiento del mieloma de células plasmáticas
Swedish	Syntetisk signalpeptid av human mucin-1 (aminosyror 1-21)	Behandling av plasmacellsmyelom
Norwegian	Syntetisk signalpeptid av human mucin-1 (aminosyre 1-21)	Behandling av plasmacellemyelom
Icelandic	Samtengt merkipeptíð manna múcín-1 (amínósýrur 1-21)	Meðferð plasmafrumu mýelóms