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EMA/617444/2017

Public summary of opinion on orphan designation

Autologous ex vivo expanded peripheral polyclonal lymphocytes enriched in activated natural killer cells for the treatment of multiple myeloma

On 16 October 2017, orphan designation (EU/3/17/1918) was granted by the European Commission to CellProtect Nordic Pharmaceuticals AB, Sweden, for autologous ex vivo expanded peripheral polyclonal lymphocytes enriched in activated natural killer cells for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma (also called plasma cell myeloma) is a cancer of a type of white blood cell called plasma cells. Plasma cells are produced in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes uncontrolled, 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 particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

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

At the time of designation, multiple myeloma affected no more than 4.3 in 10,000 people in the European Union (EU). This was equivalent to no more than 222,000 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 authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with corticosteroids to reduce the activity of the immune system, the body's natural defences. After

*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 515,700,000 (Eurostat 2017).

chemotherapy patients received a stem-cell transplant if they were considered suitable for it. Stem-cell transplantation is a procedure where the patient's bone marrow is replaced with stem cells to form new bone marrow that produces healthy blood cells.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with multiple myeloma. Early clinical data showed that the medicine was effective in patients with multiple myeloma who were to undergo a stem-cell transplant. 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?

The medicine is made up of a type of white blood cells called natural-killer (NK) cells that belong to the immune system (the body's natural defences) and that act against infections or cancers. To produce the medicine, NK cells are taken from the patient's own blood, grown outside the body to increase their number and activated to increase their anticancer activity. When injected into the patient, the NK cells are expected to destroy cancer cells, thereby slowing down the growth of the cancer.

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 multiple myeloma were ongoing.

At the time of submission, the medicine 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 7 September 2017 recommending the granting of this designation.

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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Autologous ex-vivo-expanded peripheral polyclonal lymphocytes enriched in activated natural killer cells	Treatment of multiple myeloma
Bulgarian	Автоложни, ex-vivo култивирани периферни поликлонални лимфоцити, обогатени с активирани естествени клетки-убийци	Лечение на мултиплен миелом
Croatian	Autologni ex-vivo ekspanzirani periferni poliklonski limfociti obogaćeni u aktiviranim prirodnim stanicama ubojicama	Liječenje multiplog mijeloma
Czech	Autologní ex-vivo expandované periferní polyklonální lymfocyty obohacené v aktivovaných NK buňkách	Léčba mnohočetného myelomu
Danish	Autologe ex-vivo-eksponderede perifere polyklonale lymfocytter beriget i aktiverede dræberceller	Behandling af multipelt myelom
Dutch	Autologe ex-vivo-geëxpandeerde perifere polyclonale lymfocyten verrijkt in geactiveerde "natural killer" cellen	Behandeling van multipel myeloom
Estonian	Autologsed ex-vivo-expanded peripheral polyclonal lymphocytes enriched in activated natural killer cells Autoloogsed ex-vivo paljundatud perifeersed polükloonaalsed lümfotsüüdid, mida on rikastatud aktiveeritud naturaalsest tapjarakkudest	Multiibelse müeloomi ravi
Finnish	Autologiset ex-vivo laajennetut perifeerisen veren polyklonaaliset lymfosyytit, rikastettuina aktivoituilla luonnollisilla tappajasoluilla	Multippeli myelooman hoito
French	Lymphocytes périphériques autologues polyclonaux multipliées ex-vivo et enrichis en cellules tueuses naturelles activées	Traitement du myélome multiple
German	Autologe ex-vivo expandierte periphere polyklonale Lymphozyten mit natürlichen Killerzellen	Behandlung des multiplen Myeloms
Greek	Αυτόλογα ex-vivo πολλαπλασιασμένα περιφερικά πολυκλωνικά λεμφοκύτταρα, εμπλουτισμένα με ενεργοποιημένα φυσικά φονικά κύτταρα	Θεραπεία πολλαπλού μυελώματος
Hungarian	Aktivált naturális killer sejtekben dúsított autológ ex-vivo expandált periferiális poliklonális limfociták	Myeloma multiplex kezelése
Italian	Linfociti periferici autologhi policlonali espansi ex-vivo e arricchiti in cellule Natural Killer attivate	Trattamento del mieloma multiplo

¹ At the time of designation

Language	Active ingredient	Indication
Latvian	Autologi <i>ex vivo</i> pavairoti perifērie poliklonālie limfocīti, kas bagātināti ar aktivētām dabsikām galētājšūnām	Multiplās mielomas ārstēšana
Lithuanian	Autologiniai <i>ex-vivo</i> išskirti periferiniai polikloniniai limfocitai, praturtinti aktyvuotomis natūraliųjų kilių ląstelėmis	Dauginės mielomos gydymas
Maltese	Limfoċiti poliklonali periferali espanduti b'mod <i>ex-vivo</i> awtologi arrikkiti f'ċelloli killer naturali attivati	Kura tal-mjeloma multipla
Polish	Autologiczne <i>ex-vivo</i> namnożone peryferyczne poliklonalne limfocyty wzbogacone aktywowanymi komórkami NK	Leczenie szpiczaka mnogiego
Portuguese	Linfócitos periféricos autólogos policlonales expandidos <i>ex vivo</i> e enriquecidos em células NK (células assassinas naturais) activadas	Tratamento do mieloma múltiplo
Romanian	Limfocite periferice autologe policlonale expandate <i>ex vivo</i> si îmbogățite cu celule NK activate	Tratamentul mielomului multiplu
Slovak	Autológne <i>ex-vivo</i> expandované periférne polyklonálne lymfocyty obohatené o aktivované "natural killer cells"	Liečba mnohopočetného myelómu
Slovenian	Avtologni <i>ex-vivo</i> -ekspandirani periferni poliklonski limfociti, obogateni z aktiviranimi naravnimi celicami-ubijalkami	Zdravljenje multiplega mieloma
Spanish	Linfócitos periféricos autólogos policlonales expandidos <i>ex vivo</i> y enriquecidos en células NK (células assassinas naturales) activadas	Tratamiento del mieloma múltiple
Swedish	Autologa <i>ex-vivo</i> -exspanderade perifer polyklonala lymfocyter berikade i aktiverade killerceller	Behandling av multipelt myelom
Norwegian	Autologe perifere polyklonale lymfocytter ekspandert <i>ex vivo</i> beriket med aktive naturlige dreperceller	Behandling av myelomatose
Icelandic	Samgena <i>ex vivo</i> útvíkkaðar fjöklóna eitiðfrumur með virkjuðum náttúrulegum drápsfrumum.	Meðferð við mergfrumuæxli