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Public summary of opinion on orphan designation

Recombinant human interleukin-7 fused to a hybrid crystallisable fragment region of a human antibody for treatment of idiopathic CD4 lymphocytopenia

On 22 May 2017, orphan designation (EU/3/17/1875) was granted by the European Commission to NeoImmuneTech, Inc, Poland, for recombinant human interleukin-7 fused to a hybrid crystallisable fragment region of a human antibody for treatment of idiopathic CD4 lymphocytopenia.

What is idiopathic CD4 lymphocytopenia?

Idiopathic CD4 lymphocytopenia is a condition in which patients have very low levels of a type of white blood cell called CD4 T cell for no apparent reason. To diagnose this condition, doctors have to rule out other causes of low CD4 T cell levels, including HIV infection and treatments that weaken the immune system.

Because their immune system is weakened, many patients may have recurring, sometimes severe, infections. Patients also commonly have auto-immune diseases, where the immune system attacks the patients' own tissues, but the relationship between the two conditions is unclear. Patients may also have cancers linked to viral infections such as EBV-associated lymphomas and Kaposi sarcoma.

Idiopathic CD4 lymphocytopenia is life threatening and long-term debilitating due to the recurring infections.

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

At the time of designation, idiopathic CD4 lymphocytopenia affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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. This represents a population of 515,700,000 (Eurostat 2017).

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 treatments are available?

At the time of the orphan designation, there were no satisfactory treatments for idiopathic CD4 lymphocytopenia authorised in the EU. CD4 T cell levels were carefully monitored and infections detected and treated promptly. Patients also received treatments to prevent infections.

How is this medicine expected to work?

The medicine is made of interleukin-7, a natural substance in the blood which is involved in the development of CD4 T cells. It is expected to increase the patient's CD4 T cell levels and by doing so help fight and prevent infection.

The interleukin-7 in this medicine has been made by a method known as 'recombinant DNA technology': it is made by cells into which a gene (DNA) has been introduced that enables them to produce interleukin-7. In this medicine the interleukin-7 is attached to part of a protein, which is expected to make it stay in the body for longer.

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, no clinical trials with the medicine in patients with idiopathic CD4 lymphocytopenia had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for idiopathic CD4 lymphocytopenia 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 April 2017 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human interleukin-7 fused to a hybrid crystallisable fragment region of a human antibody	Treatment of idiopathic CD4 lymphocytopenia
Bulgarian	Рекомбинантен човешки интерлевкин-7 кондензиран с хибридна кристализираща фрагментна област на човешко антитяло	Лечение на идиопатична CD4 лимфоцитопения
Croatian	Rekombinantni humani interleukin 7 spojen s hibridnim fragmentskim područjem humanog antitijela koje se može kristalizirati	Liječenje idiopatske CD4 limfocitopenije
Czech	Rekombinantní lidský interleukin-7 fúzovaný do hybridní oblasti krystalizovaného fragmentu lidských protilátek	Léčba idiopatická CD4 lymfocytopenie
Danish	Rekombineret humant interleukin-7 sammensluttet til en hybrid Fc-region af et humant antistof	Behandling af idiopatisk CD4 lymfocytopeni
Dutch	Recombinant humaan interleukine-7 gefuseerd aan een hybride Fc-deel van het humaan antilichaam	Behandeling van idiopathische CD4 lymfocytopenie
Estonian	Rekombinantne inimese interleukiin-7, mis on sulandatud inimese antikeha hübriidse kristalliseeruva fragmendipiirkonna külge	Idiopaatiline CD4 lümfotsütopeeniat ravi
Finnish	Ihmisen rekombinantti interleukiini-7 fuusioituna ihmisen vasta-aineen hybridiin Fc-osaan	Idiopaattisen CD4 lymfosytopenian hoito
French	Interleukine -7 humaine recombinée et fusionnée à un fragment de région hybride cristallisable d'anticorps humain	Traitement de la lymphocytopénie CD4 idiopathique
German	Rekombinantes menschliches Interleukin-7, fusioniert an eine hybride kristallisierbare Fragmentregion eines menschlichen Antikörpers	Behandlung von idiopathischer CD4-Lymphozytopenie
Greek	Ανασυνδυασμένη ανθρώπινη ιντερλευκίνη-7 ενωμένη με ένα υβριδικό κρυσταλλοποιήσιμο τμήμα ανθρώπινου αντισώματος	Θεραπεία της ιδιοπαθούς CD4 λεμφοκυτταροπενίας
Hungarian	Emberi rekombinált interleukin-7, összekapcsolva egy kristályosítható hibrid emberi antitest régió részletével	Idiopátiás CD4 lymphocytopenia kezelése
Italian	Intrleuchina-7 umana ricombinante fusa ad un frammento cristallizabile ibrido della regione di un anticorpo umano	Trattamento della linfopenia idiopatica CD4
Latvian	Rekombinants cilvēka interleikīns 7, kas sapludināts ar cilvēka antivielas hibrīdu kristalizējamu fragmentu	Idiopātiskas CD4 limfocitopēnijas ārstēšana
Lithuanian	Rekombinantinis žmogaus interleukinas-7 sulietas su hibridinio kristalizuojančiojo žmogaus antikūno fragmento sritimi	Idiopatinės CD4 limfocitopenijos gydymas

[1] At the time of designation

Language	Active ingredient	Indication
Maltese	Interlewkin-7 uman rikombinanti magħqud ma' reġjun ta' framment ibridu kristallizzabbli ibrid ta' antikorp uman	Kura tal-limfoċitopenja CD4 idjopatika
Polish	Ludzka rekombinowana interleukina 7 przyłączona do hybrydowego krystalizującego fragmentu regionu ludzkiego przeciwciała	Leczenie idiopatycznej limfocytopenii CD4
Portuguese	Interleucina-7 recombinante humana fusionada a um fragmento cristalizável da região híbrida de um anticorpo humano	Tratamento da linfocitopenia CD4 idiopática
Romanian	Interleukină-7 recombinantă umană fuzionată cu o regiune de fragment cristalizabil hibrid al unui anticorp uman.	Tratamentul limfocitopeniei CD4 idiopatice
Slovak	Rekombinantný ľudský interleukín-7 fúzovaný do hybridnej oblasti kryštalizovaného fragmentu ľudskej protilátky	Liečba idiopatickej CD4 lymfocytopénie
Slovenian	Rekombinantni humani interlevkin-7, vezan na hibridni fragment regije humanega protitelesa, ki kristalizira.	Zdravljenje idiopatske CD4 limfocitopenije
Spanish	Interleucina-7 humana recombinante fusionada a la región cristalizable del anticuerpo humano	Tratamiento para la linfocitopenia de CD4 idiopática
Swedish	Rekombinant humant interleukin-7 fuserat till enkristalliserbar hybridfragmentregion i en mänsklig antikropp	Behandling av idiopatisk CD4 lymfocytopeni
Norwegian	Rekombinant humant interleukin-7 fusjonert med en hybrid krystalliserbar region av et humant antistoff	Behandling av idiopatisk CD4 lymfocytopeni
Icelandic	Raðbrigða manna interleukín-7 blandað saman við kristallaðan blendingshluta mótefnis úr mönnum	Meðferð við sjálfvakinn CD4 eitilfrumnafæð