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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adenovirus-specific T-cells derived from allogeneic donor leukocytes, expanded ex vivo, for the treatment of adenovirus infection in allogeneic haematopoietic stem cell transplant recipients

On 16 January 2014, orphan designation (EU/3/13/1227) was granted by the European Commission to Cell Medica Ltd., United Kingdom, for adenovirus-specific T-cells derived from allogeneic donor leukocytes, expanded ex vivo, for the treatment of adenovirus infection in allogeneic haematopoietic stem cell transplant recipients.

What is adenovirus infection in allogeneic haematopoietic stem cell transplant recipients?

Adenoviruses are a type of virus that can infect human beings, but in healthy people they rarely produce serious illness. However, in patients undergoing allogeneic haematopoietic stem cell transplants (HSCT), which involves replacement of the blood-forming cells in the bone marrow and blood by cells from a healthy donor, the immune system (the body's natural defence) is suppressed by the treatment. Adenovirus infections can therefore lead to inflammation and damage in the lung, the liver, the kidney and the urinary system, the bowel and the brain or spine.

Infection with adenovirus in patients who are given HSCT can be chronically debilitating and life threatening, in particular due to the spread of severe infection.

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

At the time of designation, adenovirus infection in allogeneic HSCT recipients affected less than 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 10,000 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).

*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 512,200,000 (Eurostat 2013).

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What treatments are available?

At the time of submission of the application for orphan drug designation, no satisfactory methods were authorised for treatment of adenovirus infection in the European Union.

How is this medicine expected to work?

The medicine consists of T-cells, which are a type of white blood cell that fights infection, taken from a healthy donor. These are grown outside the body in the presence of fragments of a protein found in most kinds of adenovirus. This increases the numbers of T-cells and enables them to later recognise and specifically attack virus-infected cells. When they are given to the patient, these T-cells restore the activity that would normally be supplied by the patient's own immune system but which has been suppressed as part of the transplant.

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 this medicine in allogeneic HSCT recipients with adenovirus infection were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for adenovirus infection in allogeneic HSCT recipients 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 December 2013 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:

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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	Adenovirus-specific T-cells derived from allogeneic donor leukocytes, expanded ex vivo	Treatment of adenovirus infection in allogeneic haematopoietic stem-cell transplant recipients
Bulgarian	Специфични към аденовирус Т-клетки, получени от алогенни донорни левкоцити доразвити ex vivo	Лечение на аденовирусни инфекции при реципиенти на трансплантант от алогенни хематопоетични стволови клетки
Czech	Specifické T buňky adenoviru odvozené z alogenních dárcovských leukocytů, namnožené ex vivo	Léčba infekcí adenoviry u příjemců transplantátů alogenních hematopoetických kmenových buněk
Croatian	T-stanice specifične za adenovirus dobivene iz leukocita alogenog donora, umnožene ex vivo	Liječenje adenovirusne infekcije kod primatelja alogenog transplantata matičnih hematopoetskih stanica
Danish	Adenovirus-specifikke T-celler deriveret fra allogene donor-leukocytter , formeret ex vivo	Behandling af adenovirus-infektion hos patienter, som har gennemgået en allogen hæmopoietisk stamcelletransplantation.
Dutch	T-cellen specifiek voor adenovirus, afgeleid van leukocyten van allogene donor, ex-vivo geëxpandeerd	Behandeling van adenovirusinfectie bij patiënten die allogene, hematopoëtische stamceltransplantatie hebben ondergaan
Estonian	Spetsiifiliselt adenoviiruse vastased T-rakud, mis on saadud doonori allogeensetest ex vivo kasvatatud leukotsüütidest	Adenoviirusnakkuse ravi patsientidel, kellele on tehtud hematopoeetiliste tüvirakkude allogeenne siirdamine
Finnish	Allogeenisen luovuttajan leukosyyteistä peräisin olevat, ex vixo -viljellyt adenovirus-spesifiset T-solut	Allogeenisen hematopoeettisen kantasolusiirron vastaanottaneen henkilön adenovirusinfektion hoito
French	Lymphocytes T spécifiques de l'adénovirus dérivés de leucocytes provenant d'un donneur allogénique et expansés ex vivo	Traitement de l’infection à adénovirus chez les receveurs d'allogreffes de cellules souches hématopoïétiques
German	Adenovirus-spezifische T-Zellen, gewonnen aus allogenen, ex vivo expandierten Spenderleukozyten	Behandlung von Adenovirus-Infektionen bei Empfängern allogener hämatopoetischer Stammzelltransplantate
Greek	Ειδικά Τ-κύτταρα έναντι αδενοϊού τα οποία προέρχονται από λευκοκύτταρα αλλογενούς δότη που αναπτύχθηκαν ex vivo	Θεραπεία λοίμωξης από αδενοϊό σε δέκτες αλλογενούς μοσχεύματος αιμοποιητικών βλαστοκυττάρων

[1] At the time of designation

Language	Active ingredient	Indication
Hungarian	Adenovírus-specifikus T-sejtek, amelyek allogén donor fehérvérsejtekből származnak, és ex vivo terjeszkednek.	Az adenovírus okozta fertőzés kezelése allogén hemopoetikus őssejt transzplantáción átesett betegeknél.
Italian	Cellule T specifiche per Adenovirus ottenute da leucociti provenienti da donatore allogenico, espanse ex vivo	Trattamento dell'infezione da adenovirus in pazienti sottoposti a trapianto allogenico di cellule staminali ematopoietiche
Latvian	Adenovīrusam specifiskas T-šūnas, kas iegūtas no alogēnu donoru leikocītiem, pavairotas ex vivo	Adenovīrusa infekcijas ārstēšana alogēnu hematopoētisko cilmes šūnu transplantātu saņēmējiem
Lithuanian	Adenovirusui specifinės T-ląstelės, išskirtos iš alogeninių donoro leukocitų, pagausintų ex vivo	Adenoviruso sukeltos infekcijos gydymas, recipientams po hematopoetinių kamieninių ląstelių persodinimo
Maltese	Ċelloli T speċifiċi għal adenovirus imnisslin minn lewkoċiti alloġeniċi minn donatur, li ġew mwassa' ex vivo	Kura ta' infezzjoni minn adenovirus f'persuni li kellhom trapjant ta' ċelluli staminali ematopojetiċi alloġeniċi
Polish	Komórki T swoiste dla adenowirusa otrzymane z allogenicznych leukocytów dawcy namnożone ex vivo	Leczenie zakażeń adenowirusem u biorców allogenicznych przeszczepów szpiku.
Portuguese	Células T específicas do adenovírus derivadas de leucócitos de dadores alogénicos, expandidas ex vivo	Tratamento de infecção por adenovírus em doentes transplantados com células estaminais hematopoiéticas alogénicas
Romanian	Limfocite T specifice pentru adenovirus derivate din leucocite de la donatori alogeni, multiplicate ex vivo	Tratamentul infecţiei cu adenovirus la pacienţii cu transplant de celule stem hematopoietice alogene
Slovak	T-bunky špecifické pre adenovírusy odvodené od alogénnych darcovských leukocytov, expandované ex vivo	Liečba infekcie spôsobenej adenovírusmi u osôb po alogénnej transplantácii hematopoetických kmeňových buniek
Slovenian	Za adenovirus specifične celice T, pridobljene iz darovalčevih levkocitov in razmnožene ex vivo	Zdravljenje adenovirusne okužbe pri bolnikih s presajenimi hematopoetičnimi matičnimi celicami darovalca
Spanish	Linfocitos T específicos de adenovirus derivados de leucocitos de donantes alogénicos, expandidos ex vivo	Tratamiento de la infección por adenovirus en pacientes beneficiarios de un trasplante alogénico de hemocitoblastos
Swedish	Adenovirusspecifika T-celler utvunna från allogena donatorleukocyter, expanderade ex vivo	Behandling av adenovirusinfektion hos mottagare av allogen hematopoietisk stamscellstransplantation
Norwegian	Adenovirusspesifikke T-celler derivert av allogene donorleukocytter, ekspandert ex vivo	Behandling av adenovirusinfeksjon hos mottakere av allogen hematopoetisk stamcelletransplantasjon

Language	Active ingredient	Indication
Icelandic	Adenóveiru sértækar T-frumur, fengnarfrá gjafaósamgena hvítra blóðkorna og ræktaðar ex vivo	Meðferð við adenoveiru-sýkingu hjá sjúklingum sem hafa undirgengist ígræðslu á blóðmyndandi ósamgena stofnfrumum