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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Donor lymphocyte preparation depleted of functional alloreactive T cells for the prevention of Graft-versus-Host disease

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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 5 September 2008, orphan designation (EU/3/08/561) was granted by the European Commission to Kiadis Pharma Netherlands B.V, Netherlands, for donor lymphocyte preparation depleted of functional alloreactive T-cells for the prevention of Graft-versus-Host disease.

What is Graft-versus-Host disease?

Graft-versus-host disease (GvHD) is a complication of bone marrow transplant. The bone marrow is the spongy tissue inside the large bones in the body that makes blood cells and platelets (components that help the blood to clot). Bone marrow transplants are used for diseases such as leukaemia or multiple myeloma (cancer of the white blood cells).

In GvHD, the cells in the transplanted bone marrow react against the patients' organs, such as the stomach, gut, skin and liver, leading to organ damage. GvHD may happen in the short term, or later after transplantation, in which case a wider range of organs can be involved. GvHD is a serious, life-threatening disease.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of GvHD was estimated to be approximately 0.2 people in 10,000 in the European Union (EU)· This was equivalent to a total of around 10,000

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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 methods of prevention are available?

Several products were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation.

Current methods for preventing GvHD aim to reduce the activity of the immune system (the body's natural defences). These methods work by targeting cells that divide often, such as the immune system cells.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that donor lymphocyte preparation depleted of functional alloreactive T-cells might be of potential significant benefit for the prevention of GvHD, mainly because it has a new mechanism of action. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

The product is a preparation of a type of immune system cell called T-cells. It involves the use of a chemical compound that reacts to light, and destroys the T-cells in the cell preparation called donor lymphocyte preparation that could attack the patient's organs.

This cell preparation is given to patients whose immune system is weak after chemotherapy. It is thought to enable the immune system to be rebuilt with healthy T-cells.

What is the stage of development of this medicine?

The effects of donor lymphocyte preparation depleted of functional alloreactive T-cells have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients who have undergone a bone marrow transplant were ongoing.

Donor lymphocyte preparation depleted of functional alloreactive T-cells was not authorised anywhere worldwide for prevention of GvHD at the time of submission. Orphan designation of donor lymphocyte preparation depleted of functional alloreactive T-cells had been granted in the United States of America for immune reconstitution and prevention of GvHD following allogeneic haematopoietic stem cell transplantation.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 June 2008 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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	Donor lymphocyte preparation depleted of functional alloreactive T-cells	Prevention of Graft-versus-Host disease
Bulgarian	Лимфоцитен донорен препарат, свободен от функционално алореактивни Т- клетки	Профилактика на болест на присадката срещу приемателя
Czech	Dárcovské lymfocyty s deplecí fuknčních alloreaktivních T lymfocytů	Prevence onemocnění štěpu proti hostiteli
Danish	Donor lymfocyt præparation hvorfra funktionelle alloreaktive T-celler er fjernet	Forebyggelse af graft versus host reaktion
Dutch	Donor lymphocytenpreparaat, ontdaan van functionele alloreactieve T-cellen	Preventie van "graft versus host" ziekte
Estonian	Doonori lümfotsüütide preparaat, millest on eemaldatud funktsionaalsed alloreaktiivsed T-rakud	*Graft versus host* haiguse preventsioon
Finnish	Luovuttajan lymfosyyttipreparaatti, josta on poistettu alloreaktiiviset T-solut	Käänteishyljintäreaktion esto
French	Préparation de lymphocytes de donneur dépourvue de cellules T alloréactives fonctionnelles	Prévention de la réaction du greffon contre l'hôte
German	Spenderlymphozytenpräparat, aus dem funktionelle alloreaktive T-Zellen entfernt wurden	Prävention der Graft-versus-Host-Reaktion
Greek	Παρασκεύασμα λυμφοκυττάρων δότη στερούμενο λειτουργικών αλλοδραστικών κυττάρων Τ	Πρόληψη της αντίδρασης του μοσχεύματος
Hungarian	Funkcionális alloreaktív T sejt-mentes donor lymphocíta-készítmény	Graft-versus-host betegség megelőzése
Italian	Preparazione di linfociti di donatore dopo eliminazione delle cellule T alloreattive	Prevenzione della reazione del trapianto contro l'ospite
Latvian	Donora limfocītu preparāts atdalīts no funkcionālām alloreaktīvām T-šunām	Saimnieka-transplantāta slimības novēršana
Lithuanian	Donoro limfocitų preparatas išvalytas nuo funkcinių aloreaktyvių T-ląstelių	Transplantato atmetimo ligos prevencija
Maltese	Preparazzjoni ta' limfoċiti minn donatur li tneħħitilha ċelloli funzjonali u alloreattivi tat-tip T	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Preparat limfocytów dawcy pozbawiony alloreaktywnych limfocytów T	Zapobieganie chorobie przeszczep przeciw gospodarzowi
Portuguese	Linfócitos de dador sem celulas T funcionais alloreactivas	Prevenção da doença do enxerto contra o hospedeiro

[1] At the time of designation

Language	Active Ingredient	Indication
Romanian	Preparat limfocitar provenit de la donator după îndepărtatrea celulelor T aloreactive funcţionale	Prevenirea bolii grefă contra gazdă
Slovak	Lymfocytový preparát od darcu zbavený funkčných alloreaktívnych T-buniek	Prevencia reakcie štepu proti hostiteľovi
Slovenian	Donorski limfociti brez funcionalnih aloreaktivnih T-celic	Preprečevanje zavrnitvene reakcije pri presaditvi
Spanish	Preparación de linfocitos de donante con reducción de células T funcionales aloreactivas	Prevención de la enfermedad de injerto contra huésped
Swedish	Donor lymphocyt preparat, uttömd av funktionella alloreaktiva T-celler	Förebyggande av graft-värd host reaktion
Norwegian	Donor lymfocytt preparat der funksjonelle alloreaktive T-celler er fjernet	Forebygging av graft-versus-host - reaksjon
Icelandic	Gjöf lymfócýta sem hafa verið svipt starfhæfum allóvirkum T-frumum	Forvörn gegn hýsilssótt