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EMA/COMP/151270/2016  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes for the treatment of post-transplant lymphoproliferative disorder

On 21 March 2016, orphan designation (EU/3/16/1627) was granted by the European Commission to Wainwright Associates Ltd, United Kingdom, for allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes for the treatment of post-transplant lymphoproliferative disorder.

#### What is post-transplant lymphoproliferative disorder?

A post-transplant lymphoproliferative disorder is a blood cancer (lymphoma) that occurs after transplantation. Following a transplant, patients receive medicines that weaken their immune system (the body's natural defences) to prevent rejection of the transplant. However, a weakened immune system also makes patients vulnerable to infection with viruses such as the Epstein-Barr virus. The Epstein-Barr virus infects white blood cells called B cells and after transplantation it may cause changes to the infected blood cells leading to cancer.

Post-transplant lymphoproliferative disorder is a life-threatening condition and is debilitating due to weight loss, fever and organ dysfunction.

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

At the time of designation, post-transplant lymphoproliferative disorder affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 82,000 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).

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\*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 513,700,000 (Eurostat 2016).

## **What treatments are available?**

At the time of designation, no satisfactory method were authorised in the European Union for the treatment of post-transplant lymphoproliferative disorder. Patients with the condition were treated with medicines authorised for non-Hodgkin's Lymphoma.

## **How is this medicine expected to work?**

This medicine is made of cells of the immune system called T cells that have been taken from a donor. The T cells are first mixed with B cells from the same donor that have been infected with the Epstein-Barr virus so that the T cells learn to recognise infected B cells as 'foreign'. The T cells are then grown to increase their numbers. When the medicine is then given to the patient, the T cells are expected to attack and kill the patient's own infected B cells, helping to control cancers associated with the virus.

## **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 post-transplant lymphoproliferative disorder were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for post-transplant lymphoproliferative disorder 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 18 February 2016 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes	Treatment of post-transplant lymphoproliferative disorder
Bulgarian	Алогенни цитотоксични Т-лимфоцити, специфични за вируса на Епщайн-Бар	Лечение на посттрансплантационно лимфопролиферативно заболяване
Croatian	Alogenični citotoksični limfociti T specifični za Epstein-Barrov virus	Liječenje post-transplancijskog limfoproliferativnog poremećaja
Czech	Alogenní cytotoxické T-lymfocyty specifické pro virus Epstein-Barrové	Léčba posttransplančních lymfoproliferací
Danish	Allogene Epstein-Barr-virusspecifikke cytotoxiske T-lymfocytter	Behandling af transplantationsrelateret lymfomsygdom
Dutch	Allogene, Epstein-Barr-virus-specifieke cytotoxische T-lymfocyten	Behandeling van post-transplant lymphoproliferatieve aandoening
Estonian	Allogeensed Epstein-Barri viiruse suhtes spetsiifilised tsütotoksilised T-lümfotsüüdid	Siirdamisjärgsete lümfoproliferatiivsete haiguste ravi
Finnish	Allogeeniset, Epstein-Barrin virukselle spesifiset sytotoksiset T-lymfosyytit	Elinsiirron jälkeisen lymfoproliferatiivisen sairauden hoito
French	Lymphocytes T cytotoxiques spécifiques au virus Epstein-Barr, d'origine allogénique	Traitement des désordres lymphoprolifératifs post-transplantation
German	Allogene, für das Epstein-Barr-Virus spezifische zytotoxische T-Lymphozyten	Behandlung des Lymphoproliferativen Syndroms nach Transplantation
Greek	Αλλογενή κυτταροτοξικά Τ λεμφοκύτταρα ειδικά έναντι του ιού Epstein-Barr	Θεραπεία της λεμφοϋπερπλαστικής διαταραχής μετά από μεταμόσχευση
Hungarian	Allogén Epstein-Barr vírus specifikus citotoxikus T-lymphocyták	Poszt-transzplantációs limfoproliferatív megbetegedés kezelése
Italian	Linfociti T citotossici allogenici specifici per il virus di Epstein-Barr	Trattamento del disordine linfoproliferativo post-trapianto
Latvian	Allogēni pret Epšteina-Barra vīrusu specifiski citotoksiskie T limfocīti	Pēctransplantācijas limfoproliferatīvo traucējumu ārstēšana
Lithuanian	Alogeniniai Epšteino-Baro virusui specifiniai citotoksiniai T limfocitai	Potransplantacinės limfoproliferacinės ligos gydymas
Maltese	Limfoċiti T ċitotossiċi alloġeniċi speċifiċi għall-virus ta' Epstein-Barr	Kura tal-marda limfoproliferattiva ta' wara t-trapjant
Polish	Allogeniczne limfocyty T cytotoksyczne specyficzne względem wirusa Epstein-Barr	Leczenie przeszczepowej choroby limfoproliferacyjnej
Portuguese	Linfócitos T citotóxicos alogénicos específicos para o vírus de Epstein-Barr	Tratamento da doença linfoproliferativa pós-transplante
Romanian	Limfocite T citotoxice alogene, specifice virusului Epstein-Barr	Tratamentul tulburării limfoproliferative post-transplant
Slovak	Alogénne cytotoxické T-lymfocyty špecifické pre vírus Epstein-Barrovej	Liečba posttransplantačnej lymfoproliferatívnej poruchy

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Slovenian	Alogenski citotoksični limfociti T, specifični za Epstein-Barrov virus	Zdravljenje potransplantacijske limfoproliferativne motnje
Spanish	Linfocitos T citotóxicos alogénicos específicos contra el virus de Epstein-Barr	Tratamiento del trastorno post-transplante limfoproliferativo
Swedish	Allogena Epstein-Barrvirusspecifika cytotoxiska T-lymfocyter	Behandling av post-transplantation lymfoproliferativ sjukdom
Norwegian	Allogene Epstein-Barr-viruspesifikke cytotoxiske T-lymfocytter	Behandling av post-transplantasjon lymfoproliferativ sykdom
Icelandic	Ósamgena Epstein-Barr veirusértækar frumudrepandi T-eitilfrumur	Meðferð á eitulfrumuoffjölgunar kvilla eftir líffæraígræðslu