



13 April 2015
EMA/COMP/364682/2009 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic *ex vivo* expanded umbilical cord blood cells for the treatment of Hodgkin's lymphoma

First publication	7 September 2009
Rev.1: sponsor's change of address	9 November 2011
Rev.2: transfer of sponsorship	12 September 2013
Rev.3: withdrawal from the Community Register	13 April 2015
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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2015 on request of the Sponsor.

On 24 July 2009, orphan designation (EU/3/09/649) was granted by the European Commission to Teva Pharma GmbH, Germany, for allogeneic *ex vivo* expanded umbilical cord blood cells for the treatment of Hodgkin's lymphoma.

The sponsorship was transferred to Regulatory Resources Group Ltd, United Kingdom, in July 2013.

What is Hodgkin's lymphoma?

Hodgkin's lymphoma is a type of cancer of the lymphatic system, a network of vessels that transport lymph from tissues through the lymph nodes and into the bloodstream.

The cause of Hodgkin's lymphoma is unknown. Because the lymphatic system is found throughout the body, the cancer can begin in almost any part of the body. In Hodgkin's lymphoma, the lymphatic cancer cells multiply too quickly and live for too long, so there are too many of them in a lymph node. Sometimes these cells spread through the lymphatic system to other lymph nodes and they may also enter the bloodstream which carries them to various organs, forming new tumours.



Many people with Hodgkin's lymphoma can be cured if the disease is found and treated early. However, despite the available treatments, Hodgkin's lymphoma remains a serious and life-threatening disease in some patients.

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

At the time of designation, Hodgkin's lymphoma affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of 50,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several medicines were authorised for the treatment of Hodgkin's lymphoma in the EU. The main treatments of Hodgkin's lymphoma included chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). Bone marrow transplantation was also used when patients had not responded to treatment or when the disease had come back after treatment. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with bone marrow from a matched donor.

The sponsor has provided sufficient information to show that allogeneic *ex vivo* expanded umbilical cord blood cells might be of significant benefit for patients with Hodgkin's lymphoma because they may be an alternative to donated bone marrow for use in transplantation. Advantages of this medicine may include an easier way of obtaining the stem cells for use in transplantation; the availability of the treatment particularly to those patients lacking a matching donor; and a reduced delay in finding a matching donor as the medicine is ready to use. These assumptions 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 umbilical cord is the tube that connects an unborn child to its mother until birth. It contains 'stem cells', cells that are usually made in the bone marrow and can develop into different types of cells.

Allogeneic *ex vivo* expanded umbilical cord blood cells come from umbilical cord donated after birth. Because umbilical cord blood cells are only found in small quantities, the cells are cultivated using a technique called *ex vivo* expansion to increase their number.

When allogeneic *ex vivo* expanded umbilical cord blood cells are transplanted into patients with Hodgkin's lymphoma, the stem cells they contain are expected to settle in the lymphatic system and produce normal lymphatic cells, which will replace the abnormal cells.

What is the stage of development of this medicine?

The effects of allogeneic *ex vivo* expanded umbilical cord blood cells have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial in patients with different types of cancer including Hodgkin's lymphoma had finished, and a further trial was ongoing.

* 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 504,800,000 (Eurostat 2009).

At the time of submission, this medicine was not authorised anywhere in the EU for Hodgkin's lymphoma. Orphan designation of this medicine had been granted in the United States of America for Hodgkin's lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 June 2009 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:

Regulatory Resources Group Ltd
Innovation House
Albany Park
Camberley
Surrey GU16 7PL
United Kingdom
Tel.: +44 1276 671166
Fax: +44 1276 670960
E-mail: info@rrgconsultancy.com

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	Allogeneic ex vivo expanded umbilical cord blood cells	Treatment of Hodgkin lymphoma
Bulgarian	Алогенни ex vivo култивирани кръвни клетки от пъпна връв	Лечение на лимфом на Хочкин
Czech	Alogenní ex vivo kultivované krvetvorné buňky z pupečnickové krve	Léčba Hodgkinova lymfomu
Danish	Allogene ex vivo ekspanderede navlestrengsblodlegemer	Behandling af Hodgkin lymfom
Dutch	Allogene ex vivo geëxpandeerde navelstrengbloedcellen	Behandeling van Hodgkin lymfoom
Estonian	Allogeensed ex vivo eraldatud nabaväadi vererakud	Hodgkini lümfoomi ravi
Finnish	Allogeeniset ex vivo kasvatetut napanuoran verisolut	Hodgkinin lymfooman hoito
French	Cellules allogéniques du sang de cordon ombilical amplifiées ex-vivo	Traitement du lymphome de Hodgkin
German	Allogene ex vivo expandierte Nabelschnurblutzellen	Behandlung des Hodgkin-Lymphoms
Greek	Αλλογενή ex vivo καλλιεργημένα αιμοσφαίρια ομφάλιου λώρου	Θεραπεία του λεμφώματος Hodgkin
Hungarian	Allogén ex vivo megnyúlt köldökzsinór vérsejtek	Hodgkin lymphoma kezelése
Italian	Cellule ematiche allogeniche da cordone ombelicale, espanse ex-vivo	Trattamento del linfoma di Hodgkin
Latvian	Allogēnas ex vivo ekspansētas nabassaites asins šūnas	Hodžkina limfomas ārstēšana
Lithuanian	Alogeninės ex vivo padaugintos virkštelės kraujo kamieninės ląstelės	Hodžkino limfomos gydymas
Maltese	Ċelluli tad-demm alloġeniċi ġejjin mill-kurdun umbilikolu, mwassa' ex vivo	Kura tal-linfoma ta' Hodgkin
Polish	Alogeniczne komórki krwi pępowinowej uzyskane w wyniku ekspansji ex vivo	Leczenie chłoniaka Hodgkina (ziarnicy złośliwej)
Portuguese	Células alogénicas do sangue do cordão umbilical expandidas ex vivo	Tratamento do linfoma de Hodgkin
Romanian	Celule sanguine alogenice din cordonul ombilical amplificate ex vivo	Tratamentul limfomului Hodgkin
Slovak	Alogénne ex vivo krvinky z pupečnej šnúry	Liečba lymfómu Hodgkinovho typu
Slovenian	Alogenske celice, pridobljene z ex vivo ekspanzijo iz popkovnične krvi	Zdravljenje Hodgkinovega limfoma
Spanish	Células sanguíneas alogénicas de cordón umbilical expandidas ex vivo	Tratamiento del linfoma de Hodgkin

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Swedish	Allogeniska ex vivo expanderade navelsträngsblodceller	Behandling av Hodgkin lymfom
Norwegian	Allogen <i>ex vivo</i> ekspanderte navlestrengsblodceller	Behandling av Hodgkin-lymfom
Icelandic	Ósamgena <i>ex vivo</i> útvíkkuð naflastrengsblóðfrumur	Meðferð við Hodgkins sjúkdómi