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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Allogeneic *ex vivo* expanded umbilical cord blood cells for the treatment of chronic myeloid leukaemia

First publication	13 October 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
<b>Disclaimer</b> 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***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2015 on request of the Sponsor.***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 8 October 2009, orphan designation (EU/3/09/665) was granted by the European Commission to Teva Pharma GmbH, Germany, for allogeneic *ex vivo* expanded umbilical cord blood cells for the treatment of chronic myeloid leukaemia.

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

### What is chronic myeloid leukaemia?

Chronic myeloid leukaemia (CML) is a cancer of the white blood cells (cells that fight against infections). In patients with CML, the bone marrow (the spongy tissue inside the large bones) produces large numbers of abnormal, immature white blood cells called 'blasts', so there are too many of them circulating in the blood. These blast cells are not fully developed and do not work properly. Over a period of time, they replace the normal white cells, red blood cells and platelets in the bone marrow.

CML is most common in adults and older people, but children may also be affected. The disease usually develops very slowly, which is why it is called 'chronic'. However, when it progresses, CML is a severe and life-threatening disease that leads to poor overall survival.



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

At the time of designation, CML affected approximately 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 61,000 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?**

Treatment for CML is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatments for CML were chemotherapy (medicines to kill cancer cells) and bone marrow transplantation (a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy 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 CML 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 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 cell.

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 CML, the stem cells they contain are expected to settle in the bone marrow and produce normal white blood cells, which will replace the abnormal cells in CML.

## **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, clinical trials in patients with CML were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for CML 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 8 July 2009 recommending the granting of this designation.

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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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic <i>ex vivo</i> expanded umbilical cord blood cells	Treatment of chronic myeloid leukaemia
Bulgarian	Алогенни <i>ex vivo</i> култивирани кръвни клетки от пъпна връв	Лечение на хронична миелоидна левкемия
Croatian	Alogene <i>ex vivo</i> umnožene krvne stanice iz pupčane vrpce	Liječenje kronične mijeloične leukemije
Czech	Alogenní <i>ex vivo</i> kultivované krvetvorné buňky z pupečníkové krve	Léčba chronické myeloidní leukémie
Danish	Allogene <i>ex vivo</i> ekspanderede navlestrengsblodlegemer	Behandling af kronisk myeloid leukæmi
Dutch	Allogene <i>ex vivo</i> geëxpandeerde navelstrengbloedcellen	Behandeling van chronische myeloïde leukemie
Estonian	Allogeensed <i>ex vivo</i> eraldatud nabaväädi vererakud	Kroonilise müeloidse leukeemia ravi
Finnish	Allogeeniset <i>ex vivo</i> kasvatetut napanuoran verisolut	Kroonisen myelooisen leukemian hoito
French	Cellules allogéniques du sang de cordon ombilical amplifiées <i>ex-vivo</i>	Traitement de la leucémie myéloïde chronique
German	Allogene <i>ex vivo</i> expandierte Nabelschnurblutzellen	Behandlung der chronischen myeloischen Leukämie
Greek	Αλλογενή <i>ex vivo</i> καλλιεργημένα αιμοσφαίρια ομφάλιου λώρου	Θεραπεία της χρόνιας μυελοειδούς λευχαιμίας
Hungarian	Allogén <i>ex vivo</i> megnyúlt köldökzsinór vérsejtek	Krónikus myeloid leukémia kezelése
Italian	Cellule ematiche allogeniche da cordone ombelicale, espanse <i>ex-vivo</i>	Trattamento della leucemia mieloide cronica
Latvian	Allogēnas <i>ex vivo</i> ekspansētas nabassaites asins šūnas	Hroniskas mieloleikozes ārstēšana
Lithuanian	Aloeninės <i>ex vivo</i> padaugintos virkštelės kraujo kamieninės ląstelės	Lėtinės mielocitinės leukemijos gydymas
Maltese	Ċelluli tad-demm alloġeniċi ġejjin mill-kurdun umbilikolu, mwassa' <i>ex vivo</i>	Kura tal-lewkimja mjelojda kronika
Polish	Alogeniczne komórki krwi pępowinowej uzyskane w wyniku ekspansji <i>ex vivo</i>	Leczenie przewlekłej białaczki szpikowej
Portuguese	Células alogénicas do sangue do cordão umbilical expandidas <i>ex vivo</i>	Tratamento da leucemia mielóide crónica
Romanian	Celule sanguine alogenice din cordonul ombilical amplificate <i>ex vivo</i>	Tratamentul leucemiei mieloidne cronice
Slovak	Alogénne <i>ex vivo</i> krvinky z pupočnej šnúry	Liečba chronickej myeloidnej leukémie
Slovenian	Alogenske celice, pridobljene z <i>ex vivo</i> ekspanzijo iz popkovnične krvi	Zdravljenje kronične mieloične levkemije

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

Spanish	Células sanguíneas alogénicas de cordón umbilical expandidas <i>ex vivo</i>	Tratamiento de la leucemia mieloide crónica
Swedish	Allogeniska <i>ex vivo</i> expanderade navelsträngsblodceller	Behandling av kronisk myeloid leukemi
Norwegian	Allogen <i>ex vivo</i> ekspanderte navlestrengsblodceller	Behandling av kronisk myelogen leukemi
Icelandic	Ósamgena <i>ex vivo</i> útvíkkuð naflastrengsblóðfrumur	Meðferð við langvinnu kyrningahvítblæði