

13 April 2015
EMA/COMP/15606/2009 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 acute myeloid leukaemia

First publication	26 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
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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2015 on request of the Sponsor.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 27 February 2009, orphan designation (EU/3/09/619) was granted by the European Commission to Teva Pharma GmbH, Germany, for allogeneic ex vivo expanded umbilical cord blood cells for the treatment of acute myeloid leukaemia.

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

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight against infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones in the body) produces large numbers of abnormal, immature white blood cells called 'blasts'. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a life-threatening disease because these immature cells take the place of the normal white blood cells, reducing the patient's ability to fight infections.

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

At the time of designation AML affected less than 2 in 10,000 people in the European Union (EU). This was below the threshold for orphan designation, which is 5 in 10,000, and is equivalent to a total of less than 101,000 people*. 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 AML 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. The main treatments of AML are chemotherapy (medicines to treat cancer) and bone marrow transplantation (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 potential significant benefit for the treatment of AML because they may be an alternative to donated bone marrow for use in transplantation. This assumption 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 cords 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 AML, the stem cell they contain are expected to 'home' to the bone marrow and produce normal white blood cells, which will replace the immature blast 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, clinical trials in patients with AML were ongoing.

At the time of submission, allogeneic *ex vivo* expanded umbilical cord blood cells had not been authorised anywhere in the world for treatment of AML. Orphan designation of the medicine had been granted in the United States of America for AML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 January 2009 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 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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Allogeneic <i>ex vivo</i> expanded umbilical cord blood cells	Treatment of acute myeloid leukaemia
Bulgarian	Алогенни <i>ex vivo</i> култивирани кръвни клетки от пъпна връв	Лечение на остра миелоидна левкемия
Croatian	Alogene <i>ex vivo</i> umnožene krvne stanice iz pupčane vrpce	Liječenje akutne mijeloične leukemije
Czech	Alogenní <i>ex vivo</i> kultivované krvetvorné buňky z pupečnickové krve	Léčba akutní myeloidní leukémie
Danish	Allogene <i>ex vivo</i> ekspanderede navlestrengsblodlegemer	Behandling af akut myeloid leukæmi
Dutch	Allogene <i>ex vivo</i> geëxpandeerde navelstrengbloedcellen	Behandeling van acute myeloïde leukemie
Estonian	Allogeensed <i>ex vivo</i> eraldatud nabaväädi vererakud	Akuutse müeloidse leukeemia ravi
Finnish	Allogeeniset <i>ex vivo</i> kasvatetut napanuoran verisolut	Akuutin myelooisen leukemian hoito
French	Cellules allogéniques du sang de cordon ombilical amplifiées <i>ex-vivo</i>	Traitement de la leucémie aiguë myéloïde
German	Allogene <i>ex vivo</i> expandierte Nabelschnurblutzellen	Behandlung der akuten myeloischen Leukämie
Greek	Αλλογενή <i>ex vivo</i> καλλιεργημένα αιμοσφαίρια ομφάλιου λώρου	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Allogén <i>ex vivo</i> megnyúlt köldökszínór vérsejtek	Akut myeloid leukaemia kezelése
Italian	Cellule ematiche allogeniche da cordone ombelicale, espanse <i>ex-vivo</i>	Trattamento della leucemia mieloide acuta
Latvian	Allogēnas <i>ex vivo</i> ekspansētas nabassaites asins šūnas	Akūtas mieloleikozes ārstēšana
Lithuanian	Alogeninės <i>ex vivo</i> padaugintos virkštelės kraujo kamieninės ląstelės	Ūmios mieloleukozės gydymas
Maltese	Ċelluli tad-demmi alloġeniċi ġejjin mill-kurdun umbilikolu, mwassa' <i>ex vivo</i>	Kura tal-lewkimja mjelojda akuta
Polish	Alogeniczne komórki krwi pępowinowej uzyskane w wyniku ekspansji <i>ex vivo</i>	Leczenie ostrej białaczki szpikowej
Portuguese	Células alogénicas do sangue do cordão umbilical expandidas <i>ex vivo</i>	Tratamento da leucémia mielóide aguda
Romanian	Celule sanguine alogenice din cordonul ombilical amplificate <i>ex vivo</i>	Tratamentul leucemiei mieloid acute
Slovak	Alogénne <i>ex vivo</i> krvinky z pupočnej šnúry	Liečba akútnej myeloidkej leukémie
Slovenian	Alogenske celice, pridobljene z <i>ex vivo</i> ekspanzijo iz popkovnične krvi	Zdravljenje akutne mieloične levkemije

¹ At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Spanish	Células sanguíneas alogénicas de cordón umbilical expandidas ex vivo	Tratamiento de la leucemia mieloide aguda
Swedish	Allogeniska ex vivo expanderade navelsträngsblodceller	Behandling av akut myeloisk leukemi
Norwegian	Allogen ex vivo ekspanderte navlestrengsblodceller	Behandling av akutt myelogen leukemi
Icelandic	Ósamgena ex vivo útvíkkuð naflastrengsblóðfrumur	Meðferð við bráðu kyrningahvítblæði