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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 myelodysplastic syndromes

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
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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2015 on request of the Sponsor.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 8 October 2009, orphan designation (EU/3/09/664) was granted by the European Commission to Teva Pharma GmbH, Germany, for allogeneic *ex vivo* expanded umbilical cord blood cells for the treatment of myelodysplastic syndromes.

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

What are myelodysplastic syndromes?

Myelodysplastic syndromes (MDSs) are a group of disorders in which the red blood cells, white blood cells and platelets produced by the bone marrow (the spongy tissue inside the large bones) do not grow and mature normally. Patients with MDSs can develop several symptoms including tiredness or weakness due to anaemia (low red blood cell counts), infections due to low white blood cells, and bruising or abnormal bleeding due to low platelet counts.

MDSs are life-threatening diseases because they can lead to severe anaemia, infections or bleeding, and can result in leukaemia (cancer of the white blood cells).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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

At the time of designation, MDSs affected approximately 2.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 116,000 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, some medicines were authorised in the EU for the treatment of MDSs. The choice of treatment for MDSs depended on a number of factors, including the type and 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 for MDSs included chemotherapy (medicines to treat cancer) and bone marrow transplantation. This is 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 MDSs 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 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 MDSs, the stem cells they contain are expected to settle in the bone marrow and produce normal white blood cells, red blood cells and platelets, which are missing in patients with MDSs.

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 MDSs were ongoing.

At the time of submission, allogeneic *ex vivo* expanded umbilical cord blood cells were not authorised anywhere in the EU for MDSs or designated as an orphan medicinal product elsewhere for this condition.

*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).

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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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic *ex vivo* expanded umbilical cord blood cells	Treatment of myelodysplastic syndromes
Bulgarian	Алогенни *ex vivo* култивирани кръвни клетки от пъпна връв	Лечение на миелодиспластичен синдром
Croatian	Alogene *ex vivo* umnožene krvne stanice iz pupčane vrpce	Liječenje mijelodisplastičnih sindroma
Czech	Alogenní ex vivo kultivované krvetvorné buňky z pupečníkové krve	Léčba myelodysplastického syndromu
Danish	Allogene ex vivo ekspanderede navlestrengsblodlegemer	Behandling af myelodysplastiske syndromer
Dutch	Allogene *ex vivo* geëxpandeerde navelstrengbloedcellen	Behandeling van myelodysplastische syndromen
Estonian	Allogeensed *ex vivo* eraldatud nabaväädi vererakud	Müelodüsplastiliste sündroomide ravi
Finnish	Allogeeniset *ex vivo* kasvatetut napanuoran verisolut	Myelodysplastisten syndroomien hoito
French	Cellules allogéniques du sang de cordon ombilical amplifiées *ex-vivo*	Traitement des syndromes myélodysplasiques
German	Allogene *ex vivo* expandierte Nabelschnurblutzellen	Behandlung der myelodysplastischen Syndrome
Greek	Αλλογενή ex vivo καλλιεργημένα αιμοσφαίρια ομφάλιου λώρου	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	Allogén *ex vivo* megnyúlt köldökzsinór vérsejtek	Myelodysplasias syndroma kezelése
Italian	Cellule ematiche allogeniche da cordone ombelicale, espanse ex-vivo	Trattamento delle sindromi mielodisplastiche
Latvian	Allogēnas *ex vivo* ekspansētas nabassaites asins šūnas	Mielodisplastisko sindromu ārstēšana
Lithuanian	Alogeninės *ex vivo* padaugintos virkštelės kraujo kamieninės ląstelės	Mielodisplastinių sindromų gydymas
Maltese	Ċelluli tad-demm alloġeniċi ġejjin mill-kurdun umbilikolu, mwassa' ex vivo	Kura tas-sindromi mjelodisplastiċi
Polish	Alogeniczne komórki krwi pępowinowej uzyskane w wyniku ekspansji ex vivo	Leczenie zespołów mielodysplastycznych
Portuguese	Células alogénicas do sangue do cordão umbilical expandidas *ex vivo*	Tratamento dos síndromes mielodisplásicos
Romanian	Celule sanguine alogenice din cordonul ombilical amplificate *ex vivo*	Tratamentul sindromului mielodisplazic
Slovak	Alogénne *ex vivo* krvinky z pupočnej šnúry	Liečba myelodysplastického syndrómu
Slovenian	Alogenske celice, pridobljene z *ex vivo* ekspanzijo iz popkovnične krvi	Zdravljenje mielodisplastičnega sindroma

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	Células sanguíneas alogénicas de cordón umbilical expandidas *ex vivo*	Tratamiento de los síndromes mielodisplásicos
Swedish	Allogeniska *ex vivo* expanderade navelsträngsblodceller	Behandling av myelodysplastiska syndrom
Norwegian	Allogen *ex vivo* ekspanderte navlestrengsblodceller	Behandling av myelodysplastisk syndrom
Icelandic	Ósamgena *ex vivo* útvíkkuð naflastrengsblóðfrumur	Til meðferðar við mergmisþroskaheilkenni