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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Low molecular weight dextran sulfate as treatment for mobilisation of progenitor cells prior to stem cell transplantation

On 5 August 2011, orphan designation (EU/3/11/883) was granted by the European Commission to TikoMed AB, Sweden, for low molecular weight dextran sulfate as treatment for mobilisation of progenitor cells prior to stem cell transplantation.

What is stem cell transplantation?

Stem cells (also known as progenitor cells) are immature cells that develop into different types of cell. Patients with diseases which affect their blood cells, such as cancers of the bone marrow or malfunctions of the immune system, may require a transplantation of haematopoietic stem cells (stem cells that make blood cells) to replace the abnormal or cancerous cells. Before such a transplantation is performed, the stem cells have to be mobilised from the bone marrow into the blood stream where they can be collected.

Diseases requiring stem cell transplantation are life-threatening, and insufficient mobilisation of stem cells can cause the transplantation to be unsuccessful.

What is the estimated number of patients affected by the conditions requiring stem cell transplantation?

At the time of designation, the need for treatment for the mobilisation of progenitor cells prior to stem cell transplantation affected not more than 0.6 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 31,000 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).

*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. This represents a population of 506,300,000 (Eurostat 2011).

What treatments are available?

At the time of designation, two medicines were authorised to mobilise stem cells: plerixafor and Granulocyte-colony stimulating factor (G-CSF).

The sponsor has provided sufficient information to show that low molecular weight dextran sulfate might be of significant benefit for the mobilisation of stem cells prior to stem cell transplantation because it works in a different way to existing methods and early studies show that it might improve the outcome of patients when used alone or in combination with existing treatments. 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?

Low molecular weight dextran sulfate is thought to work by causing the release from the bone marrow into the blood of 'cytokine stromal cell-derived factor 1', a substance known to attract and guide stem cells, resulting in stem cells being mobilised from the bone marrow into the blood stream.

What is the stage of development of this medicine?

The effects of low molecular weight dextran sulfate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with low molecular weight dextran sulfate in treatment for the mobilisation of progenitor cells prior to stem cell transplantation had been started.

At the time of submission, the medicinal product was not authorised anywhere in the EU for the mobilisation of progenitor cells prior to stem cell transplantation or designated as an orphan medicinal product elsewhere for this purpose.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 May 2011 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:

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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	Low molecular weight dextran sulfate	Treatment for mobilisation of progenitor cells prior to stem cell transplantation
Bulgarian	Декстран сулфат с ниско молекулярно тегло	Лечение за мобилизация на прогениторните клетки преди трансплантация на стволови клетки
Czech	Nízkomolekulární natrium-dextran-sulfát	Stimulace mobilizace periferních kmenových buněk určených k transplantaci
Danish	Lavmolekylært dextransulfat	Behandling til mobilisering af progenitorceller før stamcelletransplantation
Dutch	Laag molecuulair gewicht dextraansulfaat	Behandeling tot mobilisatie van progenitorcellen voorafgaand aan stamceltransplantatie
Estonian	Madala molekulaarmassiga dekstraansulfaat	Ravi vereloome eellasrakkude mobiliseerimiseks enne tüvirakkude siirdamist
Finnish	Pienen molekyylipainon dekstraanisulfaatti	Esisolujen mobilisointiin ennen kantasolusiirtoa
French	Dextran sulfate de bas poids moléculaire	Traitement pour la mobilisation des cellules progénitrices avant greffe de cellules souches
German	Niedermolekulares Dextransulfat	Behandlung zur Mobilisierung von Vorläuferzellen vor Stammzelltransplantation
Greek	Θειική δεξτράνη χαμηλού μοριακού βάρους	Θεραπεία για την κινητοποίηση προγονικών κυττάρων που προηγείται της μεταμόσχευσης βλαστικών κυττάρων
Hungarian	Alacsony molekulásúlyú dextranszulfát	Őssejt transzplantációt megelőző progenitor sejt mobilizáló kezelés
Italian	Destrano solfato a basso peso molecolare	Trattamento per la mobilitazione delle cellule progenitrici precedente il trapianto di cellule staminali
Latvian	Mazas molekulmasas dekstrāna sulfāts	Ārstēšana priekšteču šūnu mobilizēšanai pirms cilmes šūnu transplantācijas
Lithuanian	Mažos molekulinės masės dekstrano sulfatas	Gydymas, siekiant mobilizuoti ląsteles – pirmtakus prieš kamieninių ląstelių transplantaciją
Maltese	Dextran sulfate ta' piż molekulari baxx	Kura għall-mobilizzazzjoni ta' ċelloli proġenituri qabel it-trapjant ta' ċelloli steminali
Polish	Siarczan dekstranu o niskim ciężarze cząsteczkowym	Leczenie w celu wcześniejszej mobilizacji komórek prekursorowych przed transplantacją komórek macierzystych
Portuguese	Sulfato de dextrano de baixo peso molecular	Tratamento prévio ao transplante de células estaminais para mobilizar células progenitoras
Romanian	Dextran sulfat cu greutate moleculară mică	Tratament pentru mobilizarea celulelor precursorare anterior transplantului de celule stem

¹ At the time of designation

Language	Active ingredient	Indication
Slovak	Dextrán sulfát s nízkou molekulárnou hmotnosťou	Liečba na mobilizáciu progenitorových buniek pred transplantáciou kmeňových buniek
Slovenian	Dekstransulfat z majhno molekularno maso	Mobilizacija prekurzorskih celic pred transplantacijo krvotvornih matičnih celic
Spanish	Sulfato de dextrano de bajo peso molecular	Tratamiento para la movilización de células progenitoras previa al trasplante de células madre
Swedish	Låg molekylärt dextransulfat	Behandling för att mobilisera progenitorceller inför stamcellstransplantation
Norwegian	Lavmolekylært dekstransulfat	Behandling for mobilisering av progenitorceller før stamcelletransplantasjon.
Icelandic	Dextransúlfat með lítinn sameindamassa	Formeðferð fyrir stofnfrumuígræðslu til að koma hreyfingu á stofnfrumur