



**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

**PUBLIC SUMMARY OF  
POSITIVE OPINION FOR ORPHAN DESIGNATION  
OF**

**human autologous bone-forming cells derived from bone marrow stem cells  
for treatment of non-traumatic osteonecrosis**

On 29 October 2007, orphan designation (EU/3/07/490) was granted by the European Commission to Bone Therapeutics SA, Belgium, for human autologous bone-forming cells derived from bone marrow stem cells for treatment of non-traumatic osteonecrosis.

**What is non-traumatic osteonecrosis?**

Osteonecrosis is a slow progressive disorder characterized by bone destruction most commonly in the head of the femur (the long bone in the thigh), although other bones can be involved. Non-traumatic osteonecrosis is generally associated with one or more risk factors, and in over two thirds of patients it is related to glucocorticoid therapy and/or alcohol abuse. As the disease progresses, most patients experience pain in the groin, in the thigh or even in the knee, and eventually develop osteoarthritis of the hip. The condition is chronically debilitating, as patients become progressively unable to walk due to pain and arthritis.

**What are the methods of treatment available?**

There are no authorised products for the condition in the Community. Surgical management is often required.

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

According to the information provided by the sponsor, non-traumatic osteonecrosis was considered to affect about 100,000 persons in the European Union.

**How is this medicinal product expected to act?**

Bone is a living tissue, and it is constantly destroyed (resorbed) and rebuilt (formed) in a process called bone remodelling. Bone remodelling is done by two types of cells: the osteoclasts for resorption and the osteoblasts for formation. Human bone marrow stem cells are collected from the same individual (autologous), and transformed into human autologous bone forming cells, under not fully understood mechanisms. It is hypothesized that human autologous bone forming cells will restore the bone of the patients.

**What is the stage of development of this medicinal product?**

The evaluation of the effects of human autologous bone-forming cells derived from bone marrow stem cells in experimental models was ongoing.

At the time of submission of the application for orphan designation, clinical trials in patients with non-traumatic osteonecrosis were ongoing.

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\* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Human autologous bone-forming cells derived from bone marrow stem cells were not authorised anywhere in the world for treatment of non-traumatic osteonecrosis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 12 September 2007 a positive opinion recommending the grant of the above-mentioned designation.

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Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

**For more information:**

Sponsor's contact details:

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Patients' association contact point: Not available

**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Human autologous bone-forming cells derived from bone marrow stem cells	Treatment of non-traumatic osteonecrosis
Bulgarian	Човешки автоложни костно-формиращи клетки, получени от костно-мозъчни стволови клетки	Лечение на нетравматична остеонекроза
Czech	Lidské autologní kostitvorné buňky odvozené z kmenových buněk kostní dřevě	Léčba netraumatické osteonekrózy
Danish	Humane autologe knogledannende celler fra knoglemarvsstamceller	Behandling af ikke-traumatisk osteonekrose
Dutch	Humane autologe osteoblasten, verkregen uit beenmergstamcellen	Behandeling van niet-traumatische osteonecrose
Estonian	Inimese autoloogsed luuformatsiooni rakud, mis on saadud luuüdi tüvirakkudest	Mittetraumaatilise osteonekroosi ravi
Finnish	Ihmisen luuytimen kantasoluista peräisin olevat, luuta muodostavat autologiset solut	Ei-traumaattisen osteonekroosin hoito
French	Ostéoblastes humains autologues dérivés de cellules souches de moëlle osseuse	Traitement de l'ostéonécrose atraumatique
German	Aus Knochenmarkstammzellen gewonnene humane autologe knochenbildende Zellen	Behandlung der nicht-traumatischen Osteonekrose
Greek	Ανθρώπινα αυτόλογα κύτταρα σχηματισμού οστών που προέρχονται από αρχέγονα κύτταρα του μυελού των οστών	Θεραπευτική αγωγή κατά της μη τραυματικής οστεονέκρωσης
Hungarian	Csontvelő őssejtekből előállított humán autológ csontképző sejtek	Nem traumás eredetű osteonecrosis kezelése
Italian	Cellule osteopoietiche umane autologhe derivate da cellule staminali di midollo osseo	Trattamento dell'osteonecrosi non traumatica
Latvian	Cilvēka autogēnas kaulus veidojošas šūnas, kas iegūtas no kaulu smadzeņu cilmes šūnām	Netraumatiskas osteonekrozes ārstēšana
Lithuanian	Autologinės žmogaus autologinės, kaulą formuojančios, ląstelės, kilę iš kaulų čiulpų kamieninių ląstelių	Netrauminės osteonekrozės gydymas
Maltese	Ċelluli umani awtologi li jiffurmaw l-għadam li ġejjin minn ċelluli staminali tal-mudullun	Kura ta' l-osteonekrozi mhux trawmatika
Polish	Ludzkie autologiczne komórki kościotwórcze z komórek macierzystych szpiku kostnego	Leczenie nieurazowej martwicy kości
Portuguese	Osteoblastos autologos humanos derivados das células estaminais da medula óssea	Tratamento da osteonecrose não-traumática

Romanian	Osteoblaști autologi umani derivați din celule stem din măduva osoasă	Tratamentul osteonecrozei non-traumatice
Slovak	Ľudské autológne kost'otvorné bunky získané z kmeňových buniek kostnej drene	Liečba netraumatickej osteonekrózy
Slovenian	Človeški avtologični kostni celični derivati iz izvornih celic kostnega mozga	Zdravljenje netravmatične kostne osteonekroze
Spanish	Células autólogas humanas formadoras de hueso derivadas de células madre de médula ósea	Tratamiento de la osteonecrosis no traumática
Swedish	Humana autologa benformande celler från benmärgsstadceller	Behandling av icke-traumatisk osteonekros
Norwegian	Humane autologe bendannende celler utledet fra stamceller fra benmarg	Behandling av ikke-traumatisk osteonekrose
Icelandic	Samgena beinmyndandi frumur manna afleiddar úr stofnfrumum beinmergs	Meðferð við beindrepi án áverka