12 September 2013
EMA/COMP/439952/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human allogeneic bone marrow derived osteoblastic-like cells for the treatment of non-traumatic osteonecrosis

On 5 August 2013, orphan designation (EU/3/13/1176) was granted by the European Commission to Bone Therapeutics SA, Belgium, for human allogeneic bone marrow derived osteoblastic-like cells for the treatment of non-traumatic osteonecrosis.

What is non-traumatic osteonecrosis?

Non-traumatic osteonecrosis is a disease characterised by loss and damage of bone tissue that is not caused by physical trauma. In this disease, the bone-forming (osteoblastic) cells do not work properly, leading to the loss and damage of bone tissue. The disease mainly affects the hips, but can also affect other bones and joints such as the knees, shoulders and ankles. Symptoms include pain which becomes worse as the disease progresses and causes limitation in movement of the affected joints.

Non-traumatic osteonecrosis is a long-term debilitating condition, as patients become progressively unable to walk due to pain.

What is the estimated number of patients?

At the time of designation, non-traumatic osteonecrosis affected approximately 2.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 148,000 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).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of non-traumatic osteonecrosis. Some patients were treated with surgery.

*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 509,000,000 (Eurostat 2013).

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How is this medicine expected to work?

This medicine is an advanced medicinal product that belongs to the group called 'tissue engineered products'. These are medicines that contain cells or tissues that have been 'engineered' (modified) so they can be used to repair, regenerate or replace tissue.

The medicine is made of osteoblastic-like cells extracted from the bone marrow of a donor and grown in a laboratory. Once implanted into the patient's bone, the cells are expected to replace the damaged bone tissue thereby relieving the symptoms of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicinal product in experimental models was ongoing.

At the time of submission, no clinical trials with the medicinal product in patients with non-traumatic osteonecrosis had been started.

At the time of submission, the medicinal product was not authorised anywhere in the EU for non-traumatic osteonecrosis 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 11 July 2013 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:

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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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Human allogeneic bone marrow derived osteoblastic-like cells	Treatment of non-traumatic osteonecrosis
Bulgarian	Човешки, алогенни, получени от костен мозък остеобластоподобни клетки	Лечение на нетравматична остеонекроза
Croatian	Stanice nalik na osteoblaste dobivene iz ljudske alogene koštane srži	Liječenje netraumatske osteonekroze
Czech	Buňky podobné osteoblastům derivované z lidské allogenní kostní dřeně	Léčba netraumatické osteonekrózy
Danish	Human allogen knoglemarv-deriveret osteoblast-lignende celler	Behandling af ikke-traumatisk osteonekrose
Dutch	Humaan allogene uit beenmerg afgeleide osteoblast-like cellen	Behandeling van niet-traumatische osteonecrose
Estonian	Inimese allogeensest luuüdist lähtuvad osteoblasti sarnased rakud	Mittetraumaatilise osteonekroosi ravi
Finnish	Ihmisen allogeenisista luuydinsoluista peräisin olevat, osteoblastin kaltaiset solut	Ei-traumaattisen osteonekroosin hoito
French	Cellules ostéoblastiques similaires dérivées de la moelle osseuse allogénique humaine	Traitement de l'ostéonécrose atraumatique
German	Humane allogene aus Knochenmark abgeleitete osteoblastenartige Zellen	Behandlung der nicht-traumatischen Osteonekrose
Greek	Ανθρώπινα αλλογενή κύτταρα ομοειδή οστεοβλαστών προερχόμενα από το μυελό των οστών.	**Θεραπευτική αγωγή κατά της μη τραυματικής οστεονέκρωσης**
Hungarian	Humán allogén csontvelő eredetű osteoblats-szerű sejtek	Nem traumás eredetű osteonecrosis kezelése
Italian	Cellule allogeniche umane derivate da midollo simil-osteoblastiche	**Trattamento dell'osteonecrosi non traumatica**
Latvian	Cilvēka allogēnas no kaulu smadzenēm iegūtas osteoblasveida šunas	Netraumatiskas osteonekrozes ārstēšana
Lithuanian	Žmogaus alogeninių kaulų čiulpų derivatinės į panašios osteoblastus ląstelės	Netrauminės osteonekrozės gydymas
Maltese	Ċelluli mnisslin minn mudullun alloġeniku uman simili għal ċelluli ostejoblastici	**Kura ta' l-ostejonekrożi mhux trawmatika**
Polish	Ludzkie allogeniczne komórki osteoblasto-podobne uzyskane ze szpiku kostnego	Leczenie nieurazowej martwicy kości
Portuguese	Células da medula óssea humana alogénicas derivadas de *Osteoblastos-like*	**Tratamento da osteonecrose não-traumática**
Romanian	Celule umane alogene, asemănătoare osteoblastelor, derivate din măduva hematogenă	Tratamentul osteonecrozei non-traumatice

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Ľudské alogénne bunky podobné osteoblastom pochádzajúce z kostnej drene	Liečba netraumatickej osteonekrózy
Slovenian	Humane alogene osteoblastom podobne celice kostnega mozga	Zdravljenje netravmatične kostne osteonekroze
Spanish	Celulas de la medula osea humana alogenicas derivadas de osteobastos poco diferenciados	Tratamiento de la osteonecrosis no traumática
Swedish	Humana allogena benmärgsderiverade osteblastlika celler	Behandling av icke-traumatisk osteonekros
Norwegian	Humane allogene beinmargsderiverte osteoblast-lignende celler	Behandling av ikke-traumatisk osteonekrose
Icelandic	Ósamgena manna osteóblasta-líkar beinmergsfrumur	Meðferð við beindrepi án áverka