



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Autologous adult live cultured osteoblasts for the treatment of non-traumatic osteonecrosis

On 26 February 2019, orphan designation (EU/3/19/2139) was granted by the European Commission to Clinical Network Services (UK) Limited, United Kingdom, for autologous adult live cultured osteoblasts 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 but by the osteoblasts (bone-forming cells) not working properly. The disease mainly affects the hips, but can also affect other bones and joints such as the knees, jaws, shoulders and ankles. Symptoms include pain which becomes worse as the disease progresses and causes limitation in movements.

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

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

At the time of designation, non-traumatic osteonecrosis affected approximately 3.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 176,000 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. Medicines that widen blood vessels can reduce the pain in the early stage of the disease. 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 28), Norway, Iceland and Liechtenstein. This represents a population of 518,400,000 (Eurostat 2019).



How is this medicine expected to work?

The medicine is made of cells that are extracted from the patient's bone marrow and grown in a laboratory to form osteoblasts. Once implanted back 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?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was authorised in India for the treatment of avascular necrosis (death of bone tissue due to interruption of blood supply) of the hip, under the name Ossgrow.

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 24 January 2019 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:

Contact details of the current sponsor for this orphan designation can be found on [the EMA website](#).

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	Autologous adult live cultured osteoblasts	Treatment of non-traumatic osteonecrosis
Bulgarian	Автоложни остеобласти от жива култура от възрастни донори	Лечение на нетравматична остеонекроза
Croatian	Autologni odrasli osteoblasti iz živih kultura	Liječenje netraumatske osteonekroze
Czech	Kultivované adultní autologní osteoblasty	Léčba netraumatické osteonekrózy
Danish	Autologe voksne levende-dyrkede osteoblaster	Behandling af ikke-traumatisk osteonekrose
Dutch	Autologe adulte levend-gecultiveerde osteoblasten	Behandeling van niet-traumatische osteonecrose
Estonian	Autoloogsed täiskasvanud kultiveeritud osteoblastid	Mittetraumaatilise osteonekroosi ravi
Finnish	Autologiset aikuisen ihmisen elävänä viljeltyt osteoblastit	Ei-traumaattisen osteonekroosin hoito
French	Ostéoblastes adultes autologues cultivés in vivo	Traitement de l'ostéonécrose atraumatique
German	Autologe adulte kultivierte Osteoblasten	Behandlung der nicht-traumatischen Osteonekrose
Greek	Αυτολογες ζωντανές-καλλιεργημένες οστεοβλάστες ενηλίκων	Θεραπευτική αγωγή κατά της μη τραυματικής οστεονέκρωσης
Hungarian	Autológ felnőtt élő-tenyésztett oszteoblaszt	Nem traumás eredetű osteonecrosis kezelése
Italian	Osteoblasti autologhi adulti in coltura vivente	Trattamento dell'osteonecrosi non traumatica
Latvian	Autologi, dzīvi-kultivēti pieaugušo osteoblasti	Netraumatiskas osteonekrozes ārstēšana
Lithuanian	Autologiniai subrendę gyvai kultivuoti osteoblastai	Netrauminės osteonekrozės gydymas
Maltese	Osteoblasti awtologi adulti kkulturati u ħajjin	Kura ta' l-ostejonekrozi mhux trawmatika
Polish	Autologiczne osteoblasty dorosłego, z żywej kultury	Leczenie nieurazowej martwicy kości
Portuguese	Osteoblastos adultos autólogos vivos cultivados	Tratamento da osteonecrose não-traumática
Romanian	Osteoblaști adulți autologi cultivați in vivo	Tratamentul osteonecrozei non-traumatice
Slovak	Autológne dospelé živo-kultivované osteoblasty	Liečba netraumatickej osteonekrózy

¹ At the time of designation

Slovenian	Avtologna živa kultura osteoplastov odraslih	Zdravljenje netravmatične kostne osteonekroze
Spanish	Osteoblastocitos adultos autologos cultivados en vivo.	Tratamiento de la osteonecrosis no traumática
Swedish	Levande odlade autologa adulta osteoplastser	Behandling av icke-traumatisk osteonekros
Norwegian	Levende dyrket autologe voksne osteoblaster	Behandling av ikke-traumatisk osteonekrose
Icelandic	Samgena fullþroska beinkímfrumur úr lifandi ræktun	Meðferð við beindrepi án áverka