30 September 2015
EMA/COMP/495514/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human allogeneic bone-marrow-derived osteoblastic cells for the treatment of osteogenesis imperfecta

On 10 August 2015, orphan designation (EU/3/15/1533) was granted by the European Commission to Bone Therapeutics SA, Belgium, for human allogeneic bone-marrow-derived osteoblastic cells for the treatment of osteogenesis imperfecta.

What is osteogenesis imperfecta?

Osteogenesis imperfecta is a group of genetic disorders that mainly affect the bones. People with the condition have a low bone mass, which causes bones to break easily, often from mild trauma. Other symptoms may include deformity, short stature, large head size (macrocephaly), hearing loss, problems with teeth development, as well as brain and lung complications.

Osteogenesis imperfecta is a seriously debilitating disease due to fragile bones, multiple fractures and deformities, which may cause pain and restriction of daily activities.

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

At the time of designation, osteogenesis imperfecta affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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 treating osteogenesis imperfecta. Patients were given supportive treatments such as physiotherapy, bracing and surgery to manage the symptoms of the disease.

*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 512,900,000 (Eurostat 2015).

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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 up of healthy bone-forming cells (osteoblasts) produced from cells known as stem cells taken from the bone marrow of a healthy donor. 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?

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

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with osteogenesis imperfecta had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for osteogenesis imperfecta. Orphan designation of the medicine had been granted in the European Union and in the United States for the treatment of osteonecrosis (death of bone tissue).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 July 2015 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 cells	Treatment of osteogenesis imperfecta
Bulgarian	Човешки алогенни остеобласти от костен мозък	Лечение на остеогенезис имперфекта
Croatian	Ljudske alogene stanice osteoblasta podrijetlom iz koštane srži	Liječenje osteogenesis imperfecte
Czech	Lidské allogenní osteoblasty derivované z kostní dřeně	Léčba osteogenesis imperfecta
Danish	Humane allogene knoglemarv afledte osteoblastiske celler	Behandling af osteogenesis imperfecta
Dutch	Humaan allogene beenmerg-afgeleide osteoblastische cellen	Behandeling van osteogenesis imperfecta
Estonian	Inimese allogeensed luuüdist lähtuvad osteoblastilised rakud	*Osteogenesis imperfecta* ravi
Finnish	Ihmisen allogeeniset luuytimestä peräisin olevat osteoblastisolut	Synnynnäisen luutumisvajauksen hoito
French	Cellules ostéoblastiques humaines allogéniques dérivées de moelle osseuse	Traitement de l'ostéogenèse imparfaite
German	Allogene humane osteoblastische Zellen gewonnen aus dem Knochenmark	Behandlung von Osteogenesis Imperfekta
Greek	Ανθρώπινα αλλογενή οστεοβλαστικά κύτταρα προερχόμενα από μυελό των οστών	Θεραπεία ατελούς οστεογένεσης
Hungarian	Humán allogén csontvelőből eredő osteoblast sejtek	Osteogenesis imperfecta kezelése
Italian	Cellule osteoblastiche umane allogeniche derivate da midollo osseo	Trattamento dell'osteogenesi imperfetta
Latvian	No kaula smadzenēm iegūtas allogēnas cilvēka osteoblastu šūnas	*Osteogenesis imperfect* ārstēšana
Lithuanian	Žmogaus alogeninės osteoblastinės ląstelės išskirtos iš kaulų čiulpų	Nebaigtinės osteogenezės gydymas
Maltese	Ċelluli ostejoblastici umani alloġeneiċi mnisslin minn mudullun	Kura tal-osteoġenesi imperfecta
Polish	Ludzkie allogeniczne komórki osteoblastyczne wywodzące się ze szpiku kostnego	Leczenie wrodzonej łamliwości kości
Portuguese	Células osteoblasticas humanas alogénicas derivadas da medula óssea	Tratamento da osteogénese imperfeita
Romanian	Celule osteoblastice umane alogenice derivate din măduva osoasă	Tratamentul osteogeneză imperfectă
Slovak	Humánne alogénne osteoblastické bunky získané z kostnej drene	Liečba osteogenesis imperfecta
Slovenian	Osteoblasti, pridobljeni iz človeških	Zdravljenje osteogenesis imperfecta

[1] At the time of designation

Language	Active ingredient	Indication
	alogenskih celic kostnega mozga	
Spanish	Celulas derivadas de la medula osea humana alogenicas	Tratamiento de Osteogénesis imperfecta
Swedish	Mänskliga osteoblastceller från allogen benmärg	Behandling av osteogenesis imperfecta
Norwegian	Humane allogene osteoblaster isolert fra beinmarg	Behandling av arvelig beinskjørhet
Icelandic	Ósamgena manna beinmergs osteoblasta frumur	Meðferð við osteogenesis imperfecta