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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin for the treatment of osteogenesis imperfecta

On 27 June 2016, orphan designation (EU/3/16/1686) was granted by the European Commission to Mereo Biopharma Group Limited, United Kingdom, for recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin for the treatment of osteogenesis imperfecta.

What is osteogenesis imperfecta?

Osteogenesis imperfecta is a group of inherited disorders that mainly affect the bones. People with the condition have fragile bones which break easily. Other sign and 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 (broken bones) and deformities, which may cause pain and restrict 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 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. Bisphosphonates were used to reduce the breakdown of the bone.

[*]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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

This medicine is a monoclonal antibody, a type of protein that has been designed to attach to and block a substance called sclerostin, which is produced by mature bone cells. The main function of sclerostin is to prevent excessive bone formation. By blocking sclerostin, this medicine is expected to increase bone formation, thereby strengthening bones and making them less prone to fracture.

The medicine is made by a method known as 'recombinant DNA technology': it is made by cells into which a gene (DNA) has been introduced that makes them able to produce the medicine.

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, one clinical trial with the medicine in patients with osteogenesis imperfecta had been completed and further trials were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for osteogenesis imperfecta 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 19 May 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin	Treatment of osteogenesis imperfecta
Bulgarian	Рекомбинантно хуманизирано моноклонално IgG2 ламбда антитяло срещу човешки склеростин	Лечение на остеогенезис имперфекта
Croatian	Rekombinantno humanizirano monoklonsko IgG2 lambda antitijelo protiv humanog sklerostina	Liječenje osteogenesis imperfecte
Czech	Rekombinantní humanizovaná monoklonální protilátka IgG2 lambda proti lidskému sklerostinu	Léčba osteogenesis imperfecta
Danish	Rekombinant humaniseret monoklonalt IgG2 lambda antistof mod human sclerostin	Behandling af osteogenesis imperfecta
Dutch	Recombinant gehumaniseerd monoklonaal IgG2 lambda-antilichaam tegen humaan sclerostine	Behandeling van osteogenesis imperfecta
Estonian	Inimese sklerostiini vastane rekombinantne inimese monoklonaalne IgG2 lambda antikeha	*Osteogenesis imperfecta* ravi
Finnish	Rekombinantti humaani monoklonaalinen IgG2 lambda -vasta-aine ihmisen sklerostiinia kohtaan	Synnynnäisen luutumisvajauksen hoito
French	Anticorps monoclonal recombinant humanisé de classe IgG2 lambda dirigé contre la sclérostine humaine	Traitement de l'ostéogenèse imparfaite
German	Rekombinanter humanisierter monoklonaler IgG2-Lambda-Antikörper gegen humanes Sclerostin	Behandlung von Osteogenesis Imperfecta
Greek	Ανασυνδυασμένο ανθρωποποιημένο μονοκλωνικό IgG2λ αντίσωμα έναντι της ανθρώπινης σκληροστίνης	Θεραπεία ατελούς οστεογένεσης
Hungarian	Humán sclerostin elleni, IgG2 lambda típusú rekombináns humanizált monoklonális antitest	Osteogenesis imperfecta kezelése
Italian	Anticorpo monoclonale umanizzato ricombinante IgG2 lambda contro la sclerostina umana	Trattamento dell'osteogenesi imperfetta
Latvian	Rekombinanta humanizēta monoklonālā IgG2 lambda antiviela pret cilvēka sklerostīnu	*Osteogenesis imperfecta* ārstēšana
Lithuanian	Rekombinantinis humanizuotas monokloninis IgG2 lambda antikūnas prieš žmogaus sklerostiną	Nebaigtinės osteogenezės gydymas
Maltese	Antikorp IgG2 lambda monoklonali umanizzat rikombinanti kontra l-isklerostina umana	Kura tal-osteoġenesi imperfecta
Polish	Rekombinowane humanizowane przeciwciało monoklonalne klasy IgG2 lambda przeciw ludzkiej sklerostynie	Leczenie wrodzonej łamliwości kości
Portuguese	Anticorpo IgG2 lambda monoclonal humanizado recombinante contra a esclerostina humana	Tratamento da osteogénese imperfeita
Romanian	Anticorp monoclonal umanizat recombinant IgG2 lambda împotriva sclerostinei umane	Tratamentul osteogenezei imperfecte
Slovak	rekombinantná humanizovaná monoklonálna protilátka IgG2 lambda proti ľudskému sklerostínu	Liečba osteogenesis imperfecta

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Rekombinantno humano monoklonsko protitelo IgG2 lambda proti humanemu sklerostinu	Zdravljenje osteogenesis imperfecta
Spanish	Anticuerpo monoclonal humanizado recombinante de tipo IgG2 lambda contra la esclerostina humana	Tratamiento de la Osteogénesis imperfecta
Swedish	Rekombinant, humaniserad monoklonal antikropp av IgG2 lambda mot humant sklerostin	Behandling av osteogenesis imperfecta
Norwegian	Rekombinant humanisert monoklonalt IgG2 lambda antistoff mot humant sclerostin	Behandling av osteogenesis imperfecta
Icelandic	Raðbrigða, mannaðlagað, einstofnaIgG2-lambda mótefni gegn manna skleróstíni .	Meðferð við osteogenesis imperfecta