



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Teriparatide for the treatment of hypoparathyroidism

On 27 June 2016, orphan designation (EU/3/16/1689) was granted by the European Commission to Alacrita LLP, United Kingdom, for teriparatide for the treatment of hypoparathyroidism.

What is hypoparathyroidism?

Hypoparathyroidism is a hormone disorder where the parathyroid glands in the neck produce too little parathyroid hormone, in most cases because of damage to the parathyroid glands occurring during surgery. Parathyroid hormone helps to regulate levels of calcium and phosphate in the body; too little of it may result in too little calcium and too much phosphate in the blood, which can affect bones, nerves and muscles, causing problems such as paraesthesia (tingling sensations), weakening of the bones, muscle spasms, seizures (fits) and irregular heartbeat and damage to heart muscle.

Hypoparathyroidism is a debilitating disease that is long lasting and may be life threatening due to the effects of low blood calcium which can lead to fits, irregular heartbeat and vocal cord spasms.

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

At the time of designation, hypoparathyroidism affected less than 5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 257,000 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, hypoparathyroidism was treated in the EU with calcium and vitamin D supplements; thiazide diuretics (a class of medicines that increase the production of urine) were also used because of their ability to lower the amount of calcium that is eliminated in the urine.

The sponsor has provided sufficient information to show that teriparatide (given by mouth) might be of significant benefit for patients with hypoparathyroidism because preliminary data shows that it

^{*}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).



increases calcium levels while reducing the need for calcium supplements which have been linked to kidney failure. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Teriparatide is made of part of the human parathyroid hormone and has similar effects. It is expected to replace the missing hormone in patients with hypoparathyroidism, thereby helping to correct the body's levels of calcium and phosphate.

What is the stage of development of this medicine?

The effects of teriparatide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with teriparatide in patients with hypoparathyroidism were ongoing.

At the time of submission, teriparatide was authorised in the EU for the treatment of osteoporosis.

At the time of submission, teriparatide was not authorised anywhere in the EU for hypoparathyroidism. Orphan designation had been granted in the United States 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.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Teriparatide	Treatment of hypoparathyroidism
Bulgarian	Терипаратид	Лечение на хипопаратиреоидизъм
Croatian	Teriparatid	Liječenje hipoparatiroze
Czech	Teriparatidum	Liječenje hipoparatiroidizma
Danish	Teriparatid	Behandling af hypoparathyroidisme
Dutch	Teriparatide	Behandeling van hypoparathyroidism
Estonian	Teriparatiid	Hüpoparatiroidismi ravi
Finnish	Teriparatidi	Hypoparatyreosin hoito
French	Tériparatide	Traitement de l'hypoparathyroïdie
German	Teriparatid	Behandlung von Hypoparathyroidismus
Greek	Τερίπαρτιδη	Θεραπεία υποπαραθυροειδισμού
Hungarian	Teriparatid	Hypoparathyreosis kezelése
Italian	Teriparatide	Trattamento del ipoparatiroidismo
Latvian	Teriparatīds	Hipoparatirozes ārstēšana
Lithuanian	Teriparatidas	Hipoparatiroidizmo gydymas
Maltese	Teriparatide	Kura tal-ipoparatiroidizmu
Polish	Teryparatyd	Leczenie niedoczynności przytarczyc
Portuguese	Teriparatida	Tratamento de hipoparatiroidismo
Romanian	Teriparatid	Tratamentul hipoparatiroidismului
Slovak	Teriparatid	Liečba hypoparatyreózy
Slovenian	Teriparatid	Zdravljenje hipoparatiroidizma
Spanish	Teriparatida	Tratamiento de hipoparatiroidismo
Swedish	Teriparatid	Behandling av hypoparathyroidism
Norwegian	Teriparatid	Behandling av hypoparatyreoidisme
Icelandic	Teriparatíð	Meðferð við vanstarfsemi kalkkirtla

¹ At the time of designation