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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human parathyroid hormone for the treatment of hypoparathyroidism

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 16 January 2014, orphan designation (EU/3/13/1210) was granted by the European Commission to NPS Pharma UK Ltd, United Kingdom, for recombinant human parathyroid hormone 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 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 produce effects on bones, nerves and muscles, including paraesthesia (tingling sensations), weakening of the bones, muscle spasms, seizures (fits) and abnormal heart beats 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.

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 256,000 people*, and is below the ceiling for

*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.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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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 recombinant human parathyroid hormone might be of significant benefit for patients with hypoparathyroidism because it replaces the hormone that is missing, thus easing the symptoms of the disease. 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?

Recombinant human parathyroid hormone is identical to the hormone naturally produced by the body. This medicine replaces the missing hormone and is expected to restore the body's levels of calcium and phosphate.

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 human parathyroid hormone.

What is the stage of development of this medicine?

The effects of recombinant human parathyroid hormone have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicinal product in patients with hypoparathyroidism had finished.

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

At the time of submission, this medicine was not authorised anywhere in the EU for hypoparathyroidism. Orphan designation of recombinant human parathyroid hormone had been granted in the United States for the treatment of hypoparathyroidism.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 November 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	Recombinant human parathyroid hormone	Treatment of hypoparathyroidism
Bulgarian	Рекомбинантен човешки паратиреоиден хормон	Лечение на хипопаратиреоидизъм
Czech	Rekombinantní lidský parathormon	Liječenje hipoparatireoidizam
Croatian	Rekombinantni ljudski paratireoidni hormon	Liječenje hipoparatireoze
Danish	Rekombinant humant parathyreoidea hormon	Behandling af hypoparathyreoidisme
Dutch	Recombinant humaan parathyroïd hormoon	Behandeling van hypoparathyroidism
Estonian	Rekombinantne inimese parathormoon	Hüpoparatüreoidismi ravi
Finnish	Ihmisen rekombinantti paratyroidihormoni	Hypoparatyreoosin hoito
French	Hormone parathyroïdienne recombinante humaine	Traitement de l'hypoparathyroïdie
German	Rekombinantes humanes Parathormon	Behandlung von Hypoparathyreoidismus
Greek	Ανασυνδυασμένη ανθρώπινη παραθορμόνη	Θεραπεία υποπαραθυρεοειδισμού
Hungarian	Rekombináns humán parathyreoid hormon	Hypoparathyreosis kezelése
Italian	Ormone paratiroideo umano ricombinante	Trattamento del ipoparatiroidismo
Latvian	Rekombinants cilvēka parathormons	Hipoparatireozes ārstēšana
Lithuanian	Rekombinantinis žmogaus parathormonas	Hipoparatiroidizmo gydymas
Maltese	Ormon tal-paratirojde uman rikombinanti	Kura tal-ipoparatirojdiżmu
Polish	Rekombinowany ludzki parathormon	Leczenie niedoczynności przytarczyc
Portuguese	Hormona paratiróide humana recombinante	Tratamento de hipoparatiroidismo
Romanian	Hormon paratiroidian uman recombinant	Tratamentul hipoparatiroidismului
Slovak	Rekombinantný ľudský parathormón	Liečba hypoparatyreózy
Slovenian	Rekombinantni človeški obščitnični hormon	Zdravljenje hipoparatiroidizma
Spanish	Hormona paratiroide recombinante humana	Tratamiento de hipoparatiroidismo
Swedish	Rekombinant humant parathyroideahormon	Behandling av hypoparathyroidism
Norwegian	Rekombinant humant paratyreoideahormon	Behandling av hypoparatyreoidisme
Icelandic	Raðbrigða manna kalkkirtlahormón	Meðferð við vanstarfsemi kalkkirtla

[1] At the time of designation