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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains for the treatment of growth hormone deficiency

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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 5 August 2013, orphan designation (EU/3/13/1179) was granted by the European Commission to Larode Ltd, United Kingdom, for recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains for the treatment of growth hormone deficiency.

What is growth hormone deficiency?

Growth hormone deficiency is a condition where the patient lacks a sufficient amount of growth hormone, which is normally secreted by the pituitary gland (at the base of the brain). Growth hormone promotes growth during childhood and adolescence, and also acts on the way the body handles proteins, fat and carbohydrates. The condition can be caused by a genetic mutation or other factors such as trauma and inflammation, or it may have no known cause. It can affect people of any age. In childhood, the main signs include failure to grow normally and impaired development of bones and skeletal muscle. In adulthood, the condition can affect the functioning of the heart, and cause reduced ability to perform exercise and psychological symptoms such as anxiety and depression.

Growth hormone deficiency is a long-term debilitating condition which includes decreased bone mass, bone fractures and psychological symptoms. The disease can be life-threatening due to the risk of problems with the heart and blood circulation.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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

At the time of designation, growth hormone deficiency affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of 205,000 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, several medicines containing recombinant human growth hormone were already authorised in the EU to treat growth hormone deficiency. These were given to patients by daily injection.

The sponsor has provided sufficient information to show that recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains might be of significant benefit for patients with growth hormone deficiency because early studies show that it may act for longer in the body, which would allow it to be injected less frequently than existing medicines, thereby making it easier for patients to take their treatment as intended. 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?

The medicine 'recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains' is expected to act in the same way as existing treatments for growth hormone deficiency, by replacing the missing growth hormone in the patient's body.

The medicine is made of growth hormone attached to two chains of amino acids. It is produced by a method known as 'recombinant DNA technology': it is made by bacteria into which a gene (DNA) has been introduced that makes them able to produce the growth hormone along with the two chains of amino acids. The two chains of amino acids are expected to prolong the time needed for the medicine to be broken down in the body.

What is the stage of development of this medicine?

The effects of recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains 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 growth hormone deficiency were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for growth hormone deficiency 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 11 July 2013 recommending the granting of this designation.

*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 512,200,000 (Eurostat 2013).

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:

Larode Ltd
Rhode Farm
Priory Lane
Selborne
Hampshire GU34 3BU
United Kingdom
Tel. +44 (0)1420 511 012
E-mail: info@larode.org

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 growth hormone modified by fusion with two hydrophilic polypeptide chains	Treatment of growth hormone deficiency
Bulgarian	Рекомбинантен човешки растежен хормон, модифициран чрез сливане с две хидрофилни, полипептидни вериги.	Лечение на дефицит на растежния хормон
Croatian	Rekombinantni ljudski hormon rasta modificiran fuzijom s dva hidrofilna polipeptidna lanca	Liječenje manjka hormona rasta
Czech	Rekombinantní lidský růstový faktor modifikovaný fúzí s dvěma hydrofilními polypeptidovými řetězci	Léčba deficitu růstového hormonu
Danish	Recombinant humant væksthormon modificeret ved fusion af to hydrofile polypeptid kæder	Behandling af væksthormonmangel
Dutch	Recombinant humaan groeihormoon gemodifiërd door fusie met twee hydrofiele polypeptideketens	Behandeling van groeihormoondeficiëntie
Estonian	Rekombinantne inimese kasvuhormoon, mis on modifitseeritud kahe hüdrofiilse polüpeptiidahela liitmise teel	Kasvuhormooni puudulikkuse ravi
Finnish	Rekombinanttitekniikalla tehty ihmisen kasvuhormoni, johon on liitetty kaksi hydrofiilistä polypeptidiketjua	Kasvuhormonin puutoksen hoito
French	Hormone recombinante de croissance humaine modifiée par fusion de 2 chaînes polypetidiques hydrophiles	Le traitement de la déficience en hormone de croissance
German	Rekombinantes humanes Wachstumshormon modifiziert durch Fusion mit 2 hydrophilen Polypeptidketten	Behandlung eines Wachstumshormonmangels
Greek	Ανασυνδυασμένη ανθρώπινη αυξητική ορμόνη τροποποιημένη με σύντηξη δύο υδρόφιλων πολυπεπτιδικών αλυσίδων.	Θεραπεία της ανεπάρκειας της αυξητικής ορμόνης
Hungarian	Fuzióval módosított két hidrofil polipeptid láncot tartalmazó rekombináns human növekedési hormon	Növekedési hormon hiány kezelése
Italian	Ormone della crescita umano ricombinante modificato dalla fusione con due catene polipeptidiche idrofiliche	Trattamento del deficit di ormone della crescita
Latvian	Rekombinēts cilvēka augšanas hormons, kurš modificēts ar divu hidrofīlu polipeptīdu ķēžu sajaukumu	Augšanas hormona deficīta ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Rekombinantinis žmogaus augimo hormonas, modifikuotas, susijungiant dviem hidrofilinėms polipeptidinėms grandinėms	Augimo hormono stokos gydymas
Maltese	Ormon tat-tkabbir uman rikombinanti modifikat b'fużjoni ma' żewg katini polipeptidi idrofiliċi	Kura ta' nuqqas tal-ormon tat-tkabbir
Polish	Rekombinowany ludzki hormon wzrostu zmodyfikowany fuzyjnie dwoma hydrofilowymi łańcuchami polipeptydowymi	Leczenie niedoboru hormonu wzrostu
Portuguese	Hormona de crescimento humana recombinante modificada por fusão com duas cadeias polipeptídicas hidrofílicas	Tratamento do défice de hormona de crescimento
Romanian	Hormon de creştere uman recombinant modificat prin fuziunea cu două lanţuri polipeptidice hidrofile	Tratamentul deficienţei de hormon de creştere
Slovak	Ľudský rekombinantný rastový hormón modifikovaný fúziou s dvoma hydrofilnými polypeptidovými reťazcami	Liečba nedostatku rastového hormónu
Slovenian	Rekombinantni humani rastni hormone modificiran s fuzijo dveh hidrofilnih polipeptidnih verig	Zdravljenje pomanjkanja rastnega hormona
Spanish	Hormona de crecimiento humana recombinante modificada por fusion de dos cadenas polipetidicas hidrofilicas.	Tratamiento de la deficiencia de la hormona del crecimiento
Swedish	Rekombinant humant tillväxthormon modifierat genom fusion med två hydrofila polypeptidkedjor	Behandling av tillväxthormonbrist
Norwegian	Rekombinant humant veksthormon modifisert ved fusjon med to hydrofile polypeptidkjeder	Behandling av veksthormonmangel
Icelandic	Raðbrigða manna vaxtarhormón umbreytt með samruna við tvær vatnssæknar fjölpeptíða keðjur	Meðferð við vaxtarhormónskorti