10 March 2015
EMA/COMP/443659/2013 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Octreotide acetate (oral use) for treatment of acromegaly

First publication	12 September 2013
Rev.1: sponsor's change of address	10 March 2015
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/3/13/1170) was granted by the European Commission to Larode Ltd, United Kingdom, for octreotide acetate (oral use) for treatment of acromegaly.

What is acromegaly?

Acromegaly is a disease in which the pituitary gland, a small gland located at the base of the brain, produces too much growth hormone leading to excess growth of body tissues and organs. Acromegaly usually affects adults in middle age. In over 90% of patients it is caused by a benign (non-cancerous) tumour of the pituitary gland called a pituitary adenoma. The most common signs of the disease include large hands and feet, enlarged nose, lips and tongue, coarse skin with skin tags and joint aches. The disease can result in serious complications, such as severe damage to the joints and problems affecting the cardiovascular (heart and blood vessels) and respiratory (breathing) systems.

Acromegaly is a long-term debilitating disease because of the abnormal growth it causes in tissues and organs in the body.

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

At the time of designation, acromegaly affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 102,000 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).

*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).

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 treatments are available?

At the time of designation, several medicines were authorised in the EU to treat acromegaly, including somatostatin analogues (medicines that block the release of growth hormone) such as octreotide and lanreotide, and pegvisomant (a medicine that blocks the effects of growth hormone). These medicines were available as solutions for injection into a muscle or under the skin. Other treatments included surgery and, in rare cases, radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that octreotide acetate (oral use) might be of significant benefit for patients with acromegaly because the medicine is a new formulation of octreotide to be taken by mouth, which is expected to be easier to use and not to cause injection-related problems such as pain and local reactions. These assumptions 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?

Octreotide is a synthetic substance that mimics the activity of the natural hormone somatostatin. Like somatostatin, octreotide blocks the release of growth hormone, resulting in the reduction of the symptoms and complications of acromegaly.

Octreotide has been available in 'acetate salt' form for the treatment of acromegaly since the 1980s. This medicine will be formulated as an oily suspension to be taken by mouth.

What is the stage of development of this medicine?

The effects of octreotide acetate (oral use) 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 acromegaly were ongoing.

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

_____

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	Octreotide acetate (oral use)	Treatment of acromegaly
Bulgarian	Октреотид ацетат (перорално приложение)	Лечение на акромегалия
Croatian	Oktreotidacetat (oralna primjena)	Liječenje akromegalija
Czech	Oktreotid acetát (perorální podání)	Léčba akromegalie
Danish	Octreotidacetat (oral anvendelse)	Behandling af akromegali
Dutch	Octreotide acetaat (oraal gebruik)	Behandeling van acromegalie
Estonian	Oktreotiidatsetaat (suukaudne)	Akromegaalia ravi
Finnish	Oktreotidiasetaatti (suun kautta)	Akromegalian hoito
French	Acétate d'octréotide (voie orale)	Traitement de l'acromégalie
German	Octreotidacetat (zum Einnehmen)	Behandlung der Akromegalie
Greek	Οξική Οκτρεοτίδη (από στόματος χρήση)	Θεραπεία της ακρομεγαλίας
Hungarian	Octreotid acetát (orális alkalmazásra)	Acromegália kezelésére
Italian	Acetato di octreotide (uso orale)	Trattamento dell'acromegalia
Latvian	Oktreotīda acetāts (iekšķīgai lietošanai)	Akromegālijas ārstēšana
Lithuanian	Oktreotido acetatas (vartoti per burną)	Akromegalijos gydymas
Maltese	Octreotide acetate (użu orali)	Kura ta' l-akromegalija
Polish	Octan oktreotydu (podanie doustne)	Leczenie akromegalii
Portuguese	Acetato de octreotida (via oral)	Tratamento da acromegália
Romanian	Acetat de octreotid (administrare orala)	Tratamentul acromegaliei
Slovak	Oktreotid acetát (perorálne použitie)	Liečba akromegálie
Slovenian	Oktreotid acetat (peroralna uporaba)	Zdravljenje akromegalije
Spanish	Acetato de octreotida (vía oral)	Tratamiento de la acromegalia
Swedish	Oktreotidacetat (oral användning)	Behandling av akromegali
Norwegian	Oktreotidacetat (oral bruk)	Behandling af akromegali
Icelandic	Oktreótíðacetat (til inntöku)	Meðhöndlun æsavvaxtar

[1] At the time of designation