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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Octreotide hydrochloride for the treatment of acromegaly

First publication	30 June 2009
Rev.1: administrative update	15 September 2009
Rev.2: transfer of sponsorship	1 October 2014
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 12 June 2009, orphan designation (EU/3/09/645) was granted by the European Commission to Camurus AB, Sweden, for octreotide hydrochloride for the treatment of acromegaly.

The sponsorship was transferred to Novartis Europharm Limited, United Kingdom, in April 2014.

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. Acromegaly usually affects adults in middle age. In over 90% of patients, it is caused by a non-cancerous tumour of the pituitary gland called a pituitary adenoma. One of the most common symptoms of the disease is the abnormal growth of the hands and feet. 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 systems.

Acromegaly is a long-term debilitating disease that can be life threatening because of its cardiovascular and respiratory complications, and the increased risk of developing cancer.

[1] Correction of active substance name throughout following corrigendum, 12 September 2024.

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

At the time of designation, acromegaly affected approximately 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 61,000 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 submission of the application for orphan drug 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 chloride (lipid depot solution) might be of significant benefit for patients with acromegaly because the medicine will be available as a ready-to-use prefilled syringe that will enable the patients to inject themselves. It will also be injected using a thinner needle than with other available medicines. These two factors might improve the patients' quality of life. 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?

Octreotide has been available as the 'acetate salt' for the treatment of acromegaly since the 1980s. Octreotide is an analogue (a copy) of the natural hormone somatostatin. Like somatostatin, octreotide blocks the release of growth hormone. This results in the reduction of the symptoms and complications of acromegaly. The acetate salt is available as two forms, one that releases octreotide immediately and is injected once a day under the skin, and another that releases it more slowly and is injected once a month into a muscle.

In octreotide hydrochloride, octreotide is to be available as the 'chloride salt' in a new formulation that is expected to release octreotide slowly and consistently. The new formulation is to be injected under the skin through a thin needle.

What is the stage of development of this medicine?

The effects of octreotide hydrochloride have been evaluated in experimental models.

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

At the time of submission, octreotide hydrochloride was not authorised anywhere in the EU for acromegaly or designated as orphan medicinal product elsewhere for this condition.

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 May 2009 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:

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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[2], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Octreotide hydrochloride	Treatment of acromegaly
Bulgarian	Октреотид хидрохлорид	Лечение на акромегалия
Croatian	Oktreotid hidroklorid	Liječenje akromegalije
Czech	Oktreotid hydrochlorid	Léčba akromegalie
Danish	Octreotidhydrochlorid	Behandling af akromegali
Dutch	Octreotide hydrochloride	Behandeling van acromegalie
Estonian	Oktreotiidvesinikkloriid	Akromegaalia ravi
Finnish	Oktreotidihydrokloridi	Akromegalian hoito
French	Chlorhydrate d'octréotide	Traitement de l’acromégalie
German	Octreotidhydrochlorid	Behandlung der Akromegalie
Greek	Υδροχλωρικό οκτρεοτίδη	Θεραπεία της ακρομεγαλίας
Hungarian	Oktreotid-hidroklorid	Acromegália kezelésére
Italian	Octreotide cloridrato	Trattamento dell'acromegalia
Latvian	Oktreotīda hidrohlorīds	Akromegālijas ārstēšana
Lithuanian	Oktreotido hidrochloridas	Akromegalijos gydymas
Maltese	Octreotide hydrochloride	Kura ta’ l-akromegalija
Polish	Chlorowodorek oktreotydu	Leczenie akromegalii
Portuguese	Cloridrato de octreotida	Tratamento da acromegália
Romanian	Clorhidrat de octreotid	Tratamentul acromegaliei
Slovak	Oktreotid hydrochlorid	Liečba akromegálie
Slovenian	Oktreotid hidroklorid	Zdravljenje akromegalije
Spanish	Hidrocloruro de octreotida	Tratamiento de la acromegalia
Swedish	Oktreotidhydroklorid	Behandling av akromegali

[2] At the time of transfer of sponsorship