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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pasireotide for the treatment of acromegaly

First publication	13 October 2009
Rev.1: sponsor's change of address	5 February 2015
Rev.2: information about Marketing Authorisation	2 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 8 October 2009, orphan designation (EU/3/09/670) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for pasireotide for the 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. Acromegaly usually affects adults between 30 and 50 years of 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 (lungs and airways) 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.

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

*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).

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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 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). Other treatments included surgery and, in rare cases, radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that pasireotide might be of significant benefit for patients with acromegaly because early studies indicate that it might improve the treatment of patients with this condition, and it might be used in patients who do not respond to treatment with octreotide. 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?

Pasireotide is a somatostatin analogue, a copy of the natural hormone somatostatin. Like somatostatin, pasireotide is expected to attach to somatostatin receptors, thus blocking the release of growth hormone. This may result in the reduction of the symptoms and complications of acromegaly.

What is the stage of development of this medicine?

The effects of pasireotide 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, pasireotide was not authorised anywhere in the EU for acromegaly or designated as 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 8 July 2009 recommending the granting of this designation.

Update: Pasireotide (Signifor) has been authorised in the EU since 19 November 2014 for the treatment of adult patients with acromegaly for whom surgery is not an option or has not been curative and who are inadequately controlled on treatment with another somatostatin analogue.

More information on Signifor can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

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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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E-mail: orphan.enquiries@novartis.com

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	Pasireotide	Treatment of acromegaly
Bulgarian	Пазиреотид	Лечение на акромегалия
Czech	Pasireotid	Léčba akromegalie
Danish	Pasireotid	Behandling af akromegali
Dutch	Pasireotide	Behandeling van acromegalie
Estonian	Pasireotiid	Akromegaalia ravi
Finnish	Pasireotidi	Akromegalian hoito
French	Pasiréotide	Traitement de l'acromégalie
German	Pasireotid	Behandlung der Akromegalie
Greek	Πασιροετίδη	Θεραπεία της ακρομεγαλίας
Hungarian	Pasireotid	Acromegália kezelésére
Italian	Pasireotide	Trattamento dell'acromegalia
Latvian	Pasireotīds	Akromegālijas ārstēšana
Lithuanian	Pazireotidas	Akromegalijos gydymas
Maltese	Pasireotide	Kura ta' l-akromegalija
Polish	Pasyreotyd	Leczenie akromegalii
Portuguese	Pasireotida	Tratamento da acromegália
Romanian	Pasireotidă	Tratamentul acromegaliei
Slovak	Pasireotid	Liečba akromegálie
Slovenian	Pasireotid	Zdravljenje akromegalije
Spanish	Pasireotida	Tratamiento de la acromegalia
Swedish	Pasireotid	Behandling av akromegali
Norwegian	Pasireotid	Behandling af akromegali
Icelandic	Pasireótíð	Meðhöndlun æsavvaxtar

[1] At the time of designation