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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pasireotide for the treatment of Cushing's disease

First publication	13 October 2009
Rev.1: information about Marketing Authorisation	10 September 2013
Rev.2: sponsor's change of address	5 February 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/3/09/671) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for pasireotide for the treatment of Cushing's disease.

What is Cushing's disease?

Cushing's disease is a disease characterised by an excess of the hormone cortisol in the blood. It is caused by a tumour of the pituitary gland (a gland located at the base of the brain) that produces large amounts of adrenocorticotrophic hormone (ACTH), which in turn stimulates the production of excess cortisol. Patients with Cushing's disease have 'central' weight gain (affecting the face and torso but not the limbs), growth of fat above the collar bone and the back of the neck, a roundish face, easy bruising, excessive growth of coarse hair on the face, weakening of the muscles and bones, depression and high blood pressure.

Cushing's disease is a severe disease that is long lasting and may be life threatening because of its complications, including diabetes, high blood pressure and mental problems.

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

At the time of designation, Cushing's disease affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 20,000 people*, and is below the ceiling

*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).



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, the main treatment for Cushing's disease involved surgery to remove the tumour responsible for causing the high cortisol levels, sometimes followed by radiotherapy (treatment with radiation). Several medicines were used in the EU to reduce the production of cortisol or prevent it from working.

The sponsor has provided sufficient information to show that pasireotide might be of significant benefit for patients with Cushing's disease because it might improve the treatment of patients with this condition and may represent an alternative to surgery when surgery is not an option or has failed. 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. These receptors are found in high amount in tumour cells, including tumours of the pituitary gland. By attaching to somatostatin receptors, pasireotide is expected to block the release of ACTH. This may result in the reduction of cortisol levels, helping to relieve the symptoms of Cushing's disease.

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 Cushing's disease were ongoing.

At the time of submission, pasireotide was not authorised anywhere in the EU for Cushing's disease 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 24 April 2012 for treatment of adult patients with Cushing's disease for whom surgery is not an option or for whom surgery has failed.

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

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pasireotide	Treatment of Cushing's disease
Bulgarian	Пазиреотид	Лечение на болест на Cushing
Czech	Pasireotid	Léčba Cushingovy choroby
Danish	Pasireotid	Behandling af Cushings sygdom
Dutch	Pasireotide	Behandeling van de ziekte van Cushing
Estonian	Pasireotiid	Cushingi tõve ravi
Finnish	Pasireotidi	Cushingin taudin hoito
French	Pasiréotide	Traitement de la maladie de Cushing
German	Pasireotid	Behandlung des Morbus Cushing
Greek	Πασιροετιδῆ	Θεραπεία της ασθένειας Cushing's
Hungarian	Pasireotid	Cushing-kór kezelése
Italian	Pasireotide	Trattamento della malattia di Cushing
Latvian	Pasireotīds	Kušinga slimības ārstēšanai
Lithuanian	Pazireotidas	Kušingo ligos gydymas
Maltese	Pasireotide	Kura tal-marda ta' Cushing
Polish	Pasyreotydy	Leczenie choroby Cushinga
Portuguese	Pasireotida	Tratamento da doença de Cushing
Romanian	Pasireotidă	Tratamentul bolii Cushing
Slovak	Pasireotid	Liečba Cushingovej choroby
Slovenian	pasireotid	zdravljenje Cushingove bolezni
Spanish	Pasireotida	Tratamiento de la enfermedad de Cushing
Swedish	Pasireotid	Behandling av Cushings sjukdom
Norwegian	Pasireotid	Behandling av Cushings sykdom
Icelandic	Pasireotíð	Til meðferðar á Cushingssjúkdómi

¹ At the time of designation