EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegvisomant for the treatment of acromegaly

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in November 2012 at the end of the period of market exclusivity.

On 14 February 2001, orphan designation EU/3/01/023 was granted by the European Commission to Pharmacia & Upjohn, United Kingdom, for pegvisomant for the treatment of acromegaly. The sponsorhip was transferred to Pharmacia Enterprises SA, Luxembourg, in October 2001 and subsequently to Pfizer Limited, United Kingdom, in November 2003.

What is acromegaly?

The pituitary is a small gland at the base of the brain that produces several important hormones to control body functions. This includes growth hormone, which regulates growth and some other metabolic processes of the body. When the pituitary gland produces too much growth hormone then this may cause a disease called acromegaly. In over 90% of acromegaly patients, too much growth hormone is produced due to a benign tumour of the pituitary gland. This benign tumour is called an adenoma. The name acromegaly comes from the Greek words for “extremities” and “enlargement” and reflects one of its most common symptoms, the abnormal growth of hands and feet. It most commonly affects middle-aged adults and can result in serious illness. Acromegaly is a chronically debilitating condition that can be life-threatening.

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

At the time of designation, acromegaly affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 23,000 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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

What treatments are available?

Once recognized, acromegaly is treatable in most patients, but because of its slow onset, often without marked symptoms, it frequently is not diagnosed correctly. Current treatment options for acromegaly are removal of the adenoma, using surgery, or radiotherapy and certain drugs that will reduce the production of growth hormone and the tumour size. There are several medicinal products authorised in the Community for the treatment of acromegaly. Pegvisomant could be of interest for the treatment of acromegaly, due to its new mechanism of action, which might result in a potentially significant benefit. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain orphan status

How is this medicine expected to work?

Growth hormone needs to interact with certain proteins of the cells called growth hormone receptors in order to regulate growth and some other metabolic processes of the body. Pegvisomant is a protein that has a structure which is similar to growth hormone. It can bind to the receptors (without affecting the growth or metabolic processes of the body) and prevent growth hormone from binding to its receptors. This is expected to block the effect of too much growth hormone in the body, and this might be used to treat acromegaly.

What is the stage of development of this medicine?

The effects of pegvisomant were evaluated in experimental models.

At the time of submission of the application for orphan designation, a number of clinical trials in patients with acromegaly had been completed.

Pegvisomant was not marketed anywhere worldwide for acromegaly. Orphan designation of pegvisomant was granted in the United States and Japan for acromegaly at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 December 2000 recommending the granting of this designation.

Update: Pegvisomant (Somavert) has been authorised in the EU since 13 November 2002 for treatment of patients with acromegaly who have had an inadequate response to surgery and/or radiation therapy and in whom an appropriate medical treatment with somatostatin analogues did not normalize IGF-I concentrations or was not tolerated.

More information on Somavert 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:

Pfizer Limited
Ramsgate Road
Sandwich
Kent CT13 9NJ
United Kingdom
Telephone: +44 1304 616161
Telefax: +44 1304 652144

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	Pegvisomant	Treatment of acromegaly
Danish	Pegvisomant	Behandling af acromegali
Dutch	Pegvisomant	Behandeling van acromegalie
Finnish	pegvisomantti	Akromegalian hoito
French	Pegvisomant	Traitement de l'acromégalie
German	Pegvisomant	Behandlung der Akromegalie
Greek	Pegvisomant	Θεραπεία της ακρομεγαλίας
Italian	Pegvisomant	Trattamento dell'acromegalia
Portuguese	Pegvisomant	Tratamento da acromegália
Spanish	Pegvisomant	Tratamiento de la acromegalia
Swedish	pegvisomant	Behandling av akromegali

[1] At the time of designation