



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Somatropin for AIDS wasting

On 8 August 2000, orphan designation (EU/3/00/001) was granted by the European Commission to Ares-Serono (Europe) Ltd., United Kingdom, for somatropin for AIDS wasting.

The sponsorship was transferred to Serono Europe Limited, United Kingdom, in May 2002. The name of the sponsor changed to Merck Serono Europe Limited in November 2009.

#### **What is AIDS wasting?**

Wasting is defined as the unintended loss of bodyweight and it is one of the symptoms of AIDS (acquired immune deficiency syndrome). There are usually several causes for this symptom such as loss of appetite, diarrhoea, decreased absorption of nutrients from food and immune factors (a decreased activity of the body's natural defence system which is also the main symptom of AIDS). Irrespective of the cause, wasting leads to widespread metabolic and immunologic dysfunction. AIDS wasting is chronically debilitating.

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

At the time of designation AIDS wasting affected less than 2 in 10,000 people in the European Union (EU)\*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 76,000 people.

#### **What treatments are available?**

At the time of submission of application for orphan drug designation, there were no medicinal products authorised for the treatment of AIDS wasting. Some patients were treated with appetite-stimulating agents.

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\*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union. This represents a population of 377,000,000 (Eurostat 2001).

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## How is this medicine expected to work?

Somatropin consists of growth hormone, which is a natural substance found in the body. It is thought that one of the causes of wasting in AIDS patients is a decrease in growth hormone production or a decrease in sensitivity towards this hormone in the body. Growth hormone has many functions in various tissues but it primarily stimulates the growth of muscle tissue (lean body mass) and the break down of fatty tissues.

## What is the stage of development of this medicine?

The effects of somatropin were evaluated in experimental models.

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

Somatropin was authorised in the United States and Japan for the treatment of AIDS wasting, at the time of submission. Orphan designation of the medicinal product was granted in the United States for this condition.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 11 July 2000 a positive opinion recommending the grant of the above-mentioned designation.

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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 European Union) 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.