3 October 2013
EMA/COMP/1443/2003 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human immunoglobulin for the treatment of polymyositis

First publication	26 April 2004
Rev.1: sponsor's change of address	6 April 2011
Rev.2: withdrawal from the Community Register	3 October 2013
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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2013 on request of the Sponsor.

On 24 October 2003, orphan designation (EU/3/03/170) was granted by the European Commission to Orfagen, France, for human immunoglobulin for the treatment of polymyositis.

What is polymyositis?

Polymyositis is a disease involving the muscles. The typical sign of polymyositis is progressive weakness of the muscles of the upper part of the legs or arms. In the course of the disease the muscles of the oesophagus, the respiratory system and the heart can also be affected so that the patients have difficulties in eating and breathing.

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

At the time of designation, polymyositis affected less than 1.25 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 48,000 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 382,800,000 (Eurostat 2003.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

What treatments are available?

Several products including glucocorticoids and azathioprine were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation. Human immunoglobulin is expected to be of potential significant benefit in particular in patients not responding to or not tolerating the standard treatments. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Although the exact cause of the disease remains unknown, it seems that the immune system of the patient is directly involved. The patient's immune system seems, in fact, to react against some natural body elements. The human immunoglobulin mechanism of action is complex. It seems to interfere with the immune system and decrease the immune reactions against body elements.

What is the stage of development of this medicine?

Human immunoglobulin is used in the treatment of polymyositis since several years. Case reports and results from small clinical trials on the use of human immunoglobulin in polymyositis have been published in the literature.

At the time of submission of the application for orphan designation, no clinical trials in patients with the condition were ongoing.

The proposed medicinal product is not authorised in any country in the designated indication, but it is authorised in the Community for the treatment of immunoglobuline deficiency and some autoimmune diseases. It was designated for treatment of dermatomyositis and polymyositis in the United States in October 1993.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 September 2003 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:

Orfagen
CRDPF Langlade
3 Avenue Hubert Curien - BP 13562
31035 Toulouse Cedex 1
France
Telephone: +33 5 34 50 64 58
Telefax: +33 5 34 50 34 57
E-mail: info@orfagen.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	Human immunoglobulin	Treatment of polymyositis
Danish	Humant immunglobulin	Behandling af polymyositis
Dutch	Humane immunoglobulinen	Behandeling van polymyositis
Finnish	Ihmisen immunoglobuliini	Polymyosiitin hoito
French	Immunoglobuline humaine	Traitement des polymyosites
German	Immunglobulin vom Menschen	Behandlung von Polymyositis
Greek	Ανθρώπινες ανοσοσφαιρίνες	Θεραπεία της πολυμυοσίτιδος
Italian	Immunoglobulina umana	Trattamento delle polimiositi
Portuguese	Imunoglobulina humana	Tratamento da polimiosite
Spanish	Inmunoglobulina humana	Tratamiento de la polimiositis
Swedish	Humant immunoglobulin	Behandling av polymyosit

[1] At the time of designation