EMA/COMP/197064/2008 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

4,7,10,13,16,19-docosahexaenoic acid for the treatment of retinitis pigmentosa

First publication	1 April 2009
Rev.1: transfer of sponsorship	20 September 2011
Rev.2: transfer of sponsorship	3 July 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.

On 3 November 2006, orphan designation (EU/3/06/412) was granted by the European Commission to Jose Manuel Cela Lopez, Spain, for 4,7,10,13,16,19-docosahexaenoic acid for the treatment of retinitis pigmentosa.

The sponsorship was transferred to Celavista Pharmaceuticals Limited, Ireland, in August 2011 and subsequently to Natac Pharma SL, Spain, in May 2013.

What is retinitis pigmentosa?

Retinitis pigmentosa is a genetic (hereditary) disorder, characterised by progressive loss of sight. In retinitis pigmentosa, some cells in the retina (the light-sensitive part of the eye), called rods and cones, are progressively damaged and eventually disappear. These cells are fundamental for eyesight. Retinitis pigmentosa is chronically debilitating due to progressive loss of vision.

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

At the time of designation retinitis pigmentosa affected less than 3.5 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of less than 175,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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 (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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

What treatments are available?

At the time of submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for the treatment of the condition. Treatment of patients with retinitis pigmentosa primarily involved genetic counselling, and general support such as information and regular medical follow up.

How is this medicine expected to work?

4,7,10,13,16,19-docosahexaenoic acid (DHA) is a so-called omega three fatty acid. These acids are an integral part of the outer lipid cell membrane of all the cells in the body (the thin double layer of fatty acids that surrounds and protects cells). In the cell membrane of the rods and cones of the eye, DHA is even more abundant than in the rest of the body. It is important for the light absorption activity of these cells. There is some evidence that suggest that patients affected by retinitis pigmentosa have decreased levels of DHA. According to the sponsor, 4,7,10,13,16,19-docosahexaenoic acid will thus reduce the effects of the disease.

What is the stage of development of this medicine?

The effects of 4,7,10,13,16,19-docosahexaenoic acid were evaluated in experimental models.

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

4,7,10,13,16,19-docosahexaenoic acid was not authorised anywhere worldwide for the treatment of retinitis pigmentosa, nor designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 October 2006 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:

Natac Pharma S.L.
C/ Faraday 7
28049 Madrid
Spain
Telephone: +34 91 827 64 70
Fax: +34 91 827 66 99

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	4,7,10,13,16,19-docosahexaenoic acid	Treatment of retinitis pigmentosa
Bulgarian	4,7,10,13,16,19 – докозахексаеноева киселина	Лечение на пигментен ретинит
Czech	Kyselina 4,7,10,13,16,19-docosahexenová	Léčba pigmentosní retitinitidy
Danish	4,7,10,13,16,19-docosahexaensyre	Behandling af retinitis pigmentosa
Dutch	4,7,10,13,16,19-docosahexaeenzuur	Behandeling van retinitis pigmentosa
Estonian	4,7,10,13,16,19-dokosaheksaeenhape	Pigmentoosse võrkkestapõletiku ravi.
Finnish	4,7,10,13,16,19-dokosaheksaeenihappo	Hoito verkkokalvorappeumaan
French	Acide 4,7,10,13,16,19-docosahexaénoïque	Traitement de la rétinite pigmentaire
German	4,7,10,13,16,19-docosahexanonsäure	Behandlung der Retinopathia Pigmentosa
Greek	4,7,10,13,16,19-δοκοσαεξαενοϊκό οξύ	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	4,7,10,13,16,19-dokozahexanoidsav	Retinitis pigmentosa kezelése
Italian	Acido 4,7,10,13,16,19-docosaesaenoico	Trattamento della retinite pigmentosa
Latvian	4,7,10,13,16,19-dokozaheksaēnskābe	Retinitis pigmentosa ārstēšana
Lithuanian	4,7,10,13,16,19-dokozahekseno rūgštis	Pigmentinio retinito gydymas
Maltese	4,7,10,13,16,19-docosahexaenoic acid	Kura tar-retinite pigmentuża
Polish	Kwas 4,7,10,13,16,19-dokozaheksaenowy	Leczenie retinopatii barwnikowej
Portuguese	Ácido 4,7,10,13,16,19-docosahexaenóico	Tratamento de retinite pigmentosa
Romanian	Acid 4,7,10,13,16,19-docosahexaenoic	Tratamentul retinitei pigmentare
Slovak	Dokos-4,7,10,13,16,19-hexaenová kyselina	Liečba retinitis pigmentosa
Slovenian	4,7,10,13,16,19-dokozaheksanojska kislina	Postopek od retinopatiya pigmentnaya
Spanish	Ácido 4,7,10,13,16,19-docosahexanoico	Tratamiento de retinosis pigmentaria
Swedish	4,7,10,13,16,19-dokosahexaensyra	Behandling av retinitis pigmentosa
Norwegian	4,7,10,13,16,19-dokosaheksaensyre	Behandling av retinitis pigmentosa
Icelandic	4,7,10,13,16,19-dócósahexaenóic sýru	Meðferð áh retinitis pigmentosa

[1] At the time of transfer of sponsorship