



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fumagillin for the treatment of diarrhoea associated with intestinal microsporidial infection

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Rev.1: administrative update	22 January 2003
Rev.2: sponsor's name change	29 May 2007
Rev.3: sponsor's name and address change	3 April 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 4 February 2002, orphan designation (EU/3/01/081) was granted by the European Commission to Sanofi-Synthélabo, France, for fumagillin for the treatment of diarrhoea associated with intestinal microsporidial infection.

In May 2006, Sanofi-Synthélabo changed name to Sanofi Aventis and in October 2012 Sanofi Aventis changed name to Sanofi-Aventis Groupe.

What is intestinal microsporidial infection?

Microsporidia are intestinal parasites that can cause severe chronic diarrhoea and malabsorption especially in patients with immunodeficiency such as patients with AIDS and in patients undergoing transplantation. The infection is life threatening.

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

At the time of designation, diarrhoea associated with intestinal microsporidial infection affected approximately 1.3 to 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 49,000 to 91,000 people*, and is below the ceiling for orphan designation, which is 5 people

*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 380,600,000 (Eurostat 2002).



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?

No satisfactory treatment existed that had been authorised at the time of designation although different antiparasitic drugs have shown symptomatic improvement but without eradicating the microsporidia from the intestinal tract.

How is this medicine expected to work?

The mechanism of action is not fully known but fumagillin is thought to inhibit the RNA synthesis and growth of microsporidial parasites.

What is the stage of development of this medicine?

The effects of fumagillin have been evaluated in experimental models, and clinical trials for the treatment of diarrhoea associated with intestinal microsporidial infection have been performed. Fumagillin is currently being developed for treatment of diarrhoea associated with intestinal microsporidial infection in severely immunosuppressed AIDS patients.

The product was not authorised nor designated as orphan medicinal product in any country at the time of submission of the application.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 November 2001 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:

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www.sanofi-aventis.com/contact/contact.asp

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	Fumagillin	Treatment of diarrhoea associated with intestinal microsporidial infection
Danish	Fumagillin	Behandling af diaré associeret med intestinal mikrosporidial infektion
Dutch	Fumagilline	Behandeling van diarree bij intestinale microsporidia infectie
Finnish	Fumagilliini	Ripuli, johon liittyy intestinaalinen mikrosporidia-infektio
French	Fumagilline	Traitement de la diarrhée associée à une infection intestinale à microsporidies
German	Fumagillin	Zur Behandlung der mit intestinaler Mikrosporidiose assoziierten Diarrhöe
Greek	Fumagillin	Θεραπεία διάρροιας σχετιζόμενης με εντερική μικροσποριδιακή λοίμωξη
Italian	Fumagillina	Trattamento della diarrea associata ad infezione intestinale da microsporidiosi
Portuguese	Fumagilina	Tratamento da diarreia associada a infecção microsporidial intestinal
Spanish	Fumagilina	Tratamiento de diarrea asociada a la infección microsporidial intestinal
Swedish	Fumagillin	Behandling av diarré associerad med intestinal mikrosporidial infektion

¹ At the time of designation