



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

29 June 2011
EMA/COMP/247584/2011
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Metronidazole for the treatment of pouchitis

On 21 June 2011, orphan designation (EU/3/11/875) was granted by the European Commission to FORMAC Pharmaceuticals NV, Belgium, for metronidazole for the treatment of pouchitis.

What is pouchitis?

Pouchitis is inflammation of the lining of the 'ileal pouch'. This is a pouch that has been surgically created from the ileum (part of the small intestine) to store faeces in patients who have had their large intestine removed. The large intestine is removed to treat diseases such as ulcerative colitis (inflammation of the large intestine causing ulceration and bleeding) or familial adenomatous polyposis (formation of numerous growths mainly in the large intestine). Pouchitis causes symptoms such as diarrhoea, increased defecation, incontinence (a lack of control over defecation), bleeding, cramps, fever and dehydration.

Pouchitis is a long-term debilitating disease that has a large impact on the quality of life of patients particularly because of incontinence, bleeding and cramps.

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

At the time of designation, pouchitis affected approximately 2.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 111,000 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).

What treatments are available?

At the time of designation, pouchitis was mainly treated with antibiotics to kill bacteria that could be causing the inflammation. In cases where the condition was not responding to antibiotics, surgery to remove the pouch was performed.

*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 506,300,000 (Eurostat 2011).



The sponsor has provided sufficient information to show that metronidazole might be of significant benefit for patients with pouchitis because the medicine is a new formulation of a known antibiotic, which is expected to cause fewer side effects. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Metronidazole is a well-known antibiotic that has been available since the 1960s and is frequently used to treat pouchitis. It belongs to the group of antibiotics called 'nitroimidazoles'. They work by entering bacteria where they are converted into toxic substances that attach to and block their DNA, thereby killing the bacteria.

This medicine will be available as a modified-release tablet containing a lower dose of metronidazole than the standard tablet. The modified-release tablet is expected to release metronidazole mainly in the pouch rather than in the stomach or small intestine, thereby avoiding high levels in the rest of the body and causing fewer side effects.

What is the stage of development of this medicine?

As metronidazole is a well-known antibiotic, the sponsor has provided data in experimental models from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, a clinical trial with metronidazole in patients with pouchitis had finished.

At the time of submission, this formulation of metronidazole was not authorised anywhere in the EU for pouchitis. Orphan designation of metronidazole had been granted in the United States of America for pouchitis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 March 2011 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:

FORMAC Pharmaceuticals NV
Gaston Geenslaan 1
B-3001 Leuven
Belgium
Telephone: +32 16 75 13 70
Telefax: +32 16 75 13 71

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	Metronidazole	Treatment of pouchitis
Bulgarian	Метронидазол	Лечение на паучитис
Czech	Metronidazol	Léčba pouchitidy
Danish	Metronidazol	Behandling af pouchitis
Dutch	Metronidazole	Behandeling van pouchitis
Estonian	Metronidasool	Reservuaariileidi ravi
Finnish	Metronidatsoli	Pussin tulehduksen hoito
French	Métronidazole	Traitement de la pouchite
German	Metronidazol	Behandlung der Pouchitis
Greek	Μετρονιδαζόλη	Θεραπεία ληκυθιτιδας
Hungarian	Metronidazol	Pouchitis kezeléské
Italian	Metronidazolo	Trattamento della pouchite
Latvian	Metronidazols	Ileoanāls anastamozes iekaisuma ārstēšana
Lithuanian	Metronidazolas	Paučito gydymas
Maltese	Metronidazole	Kura tal-pouchitis
Polish	Metronidazol	Leczenie zapalenia zbiornika jelitowego
Portuguese	Metronidazol	Tratamento da bolsite
Romanian	Metronidazol	Tratamentul inflamației rezervorului ileal
Slovak	Metronidazol	Liečba zápalu ileálneho vačku
Slovenian	Metronidazol	Zdravljenje pouchitisa
Spanish	Metronidazol	Tratamiento de la reservoritis
Swedish	Metronidazol	Behandling av pouchit
Norwegian	Metronidazol	Behandling av pouchitt
Icelandic	Metróníðazól	Meðferð við sýkingu í endaparmspoka

¹ At the time of designation