



EMA/COMP/241184/2009 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Alicaforsen for the treatment of pouchitis

First publication	5 June 2009
Rev.1: sponsor's name and address change	16 September 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 15 May 2009, orphan designation (EU/3/09/641) was granted by the European Commission to Atlantic Healthcare Limited, United Kingdom, for alicaforsen for the treatment of pouchitis.

In July 2013, Atlantic Healthcare Limited changed name to Atlantic Healthcare plc.

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 part of 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 and dehydration.

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 was equivalent to a total of around 111,000 people^{*}, and is below the ceiling for

^{*}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. At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).



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.

The sponsor has provided sufficient information to show that alicaforsen might be of significant benefit for patients with pouchitis because it might be more effective than existing treatments. 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?

Alicaforsen is an 'antisense oligonucleotide', a very short fragment of DNA. It is designed to attach to the genetic material of cell responsible for producing a protein called ICAM-1, blocking its production. ICAM-1 is found on the surface of leucocytes (white blood cells) and on the inner surface of blood vessels, and is involved in the inflammation process seen in pouchitis. By blocking the production of ICAM-1, alicaforsen is expected to reduce the inflammation and relieve the symptoms of the condition.

What is the stage of development of this medicine?

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

At the time of submission, alicaforsen was not authorised anywhere in the EU for pouchitis.

Orphan designation of alicaforsen 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 2 April 2009 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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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	Alicaforsen	Treatment of pouchitis
Bulgarian	Аликафорсен	Лечение на паучитис
Czech	Alikaforsen	Léčba pouchitidy
Danish	Alicaforsen	Behandling af pouchitis
Dutch	Alicaforsen	Behandeling van pouchitis
Estonian	Alikaforseen	Reservuaariileidi ravi
Finnish	Alikaforseeni	Pussin tulehduksen hoito
French	Alicaforsen	Traitement de la pouchite
German	Alicaforsen	Behandlung der Pouchitis
Greek	Αλικαφορσένη	Θεραπεία ληκυθιτιδας
Hungarian	Alicaforsen	Pouchitis kezelésére
Italian	Alicaforsen	Trattamento della pouchite
Latvian	Alikaforsēns	Iekaisuma ārstēšanai ileoanālās anastomozes un ileostomas vietā pēc proktokolonektomijas
Lithuanian	Alikaforsenas	Paučito gydymas
Maltese	Alicaforsen	Kura tal-pouchitis
Polish	Alikaforsen	Leczenie zapalenia zbiornika jelitowego
Portuguese	Alicaforseno	Tratamento da bolsite
Romanian	Alicaforsen	Tratamentul inflamației rezorvului ileal
Slovak	Alikaforsen	Liečba zápalu ileálneho vačku
Slovenian	Alikaforsen	Zdravljenje pouchitisa
Spanish	Alicaforsen	Tratamiento de la reservoritis
Swedish	Alicaforsen	Behandling av pouchit
Norwegian	Alikaforsen	Behandling av pouchitt
Icelandic	Alicaforsen	Meðferð við sýkingu í endaparmspoka

¹ At the time of designation