

25 February 2019
EMA/818573/2018

Public summary of opinion on orphan designation

Acetylcysteine for the treatment of pseudomyxoma peritonei

On 14 December 2018, orphan designation (EU/3/18/2107) was granted by the European Commission to MUCPharm Pty Ltd, United Kingdom, for acetylcysteine for the treatment of pseudomyxoma peritonei.

What is pseudomyxoma peritonei?

Pseudomyxoma peritonei is a disease where tumours that produce mucus (a thick slimy fluid) develop and spread throughout the abdomen (belly). If left untreated, the tumours and the mucus they produce fill the abdominal cavity causing pain and swelling and even blockage of the bowel.

Pseudomyxoma peritonei is a long-term debilitating and life-threatening disease which can cause pain, pressure on internal organs, malnutrition, bowel obstruction and internal bleeding.

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

At the time of designation, pseudomyxoma peritonei affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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, the main treatments for the condition were surgery and chemotherapy (medicines to treat cancer) given directly into the peritoneum (the lining around the abdomen).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pseudomyxoma peritonei. Preliminary data show that treatment with acetylcysteine in combination with another medicine called bromelain can dissolve the mucus and lead to tumour reduction in patients who are not suitable for surgery and chemotherapy or in whom these methods have not worked.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

How is this medicine expected to work?

In patients with pseudomyxoma peritonei, acetylcysteine is thought to work by dissolving the mucus so that it can be more easily cleared out.

Acetylcysteine is expected to be injected into the tumours together with bromelain, which also breaks down mucus.

What is the stage of development of this medicine?

The effects of acetylcysteine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with acetylcysteine in combination with bromelain in patients with pseudomyxoma peritonei were ongoing.

At the time of submission, acetylcysteine was authorised in the EU for paracetamol overdose and to loosen mucus in patients with cystic fibrosis or chronic obstructive pulmonary disease.

At the time of submission, acetylcysteine was not authorised anywhere in the EU for pseudomyxoma peritonei or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 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:

Contact details of the current sponsor for this orphan designation can be found on [the EMA website](#).

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	Acetylcysteine	Treatment of pseudomyxoma peritonei
Bulgarian	Ацетилцистеин	Лечение на псевдомиксом на перитонеума
Croatian	Acetilcistein	Liječenje pseudomyxoma peritonei
Czech	Acetylcystein	Léčba pseudomyxomu peritonea
Danish	Acetylcysteine	Behandling af pseudomyxoma peritonei
Dutch	Acetylcysteïne	Behandeling van pseudomyxoma peritonei
Estonian	Atsetüültsüsteiin	Kõhukelme pseudomüksoomi ravi
Finnish	Asetyylikysteini	Peritoneumin pseudomyksooman hoito
French	Acétylcystéine	Traitement de pseudomyxoma peritonei
German	Acetylcystein	Behandlung von pseudomyxoma peritonei
Greek	Ακετυλοκυστεΐνη	Θεραπεία του ψευδομυξώματος του περιτοναίου
Hungarian	Acetilcisztein	Pseudomyxoma peritonei kezelése
Italian	Acetilcisteina	Trattamento di pseudomyxoma peritonei
Latvian	Acetilcisteīns	<i>Pseudomyxoma peritonei ārstēšana</i>
Lithuanian	Acetilcisteinas	Peritoninės pseudomiksomos gydymas
Maltese	Acetylcysteine	Trattament ta pseudomyxoma peritonei
Polish	Acetylocysteina	Leczenie śluzaka rzekomego otrzewnej
Portuguese	Acetilcisteína	Tratamento do pseudomixoma peritoneal
Romanian	Acetilcisteină	Tratamentul pseudomixomului peritoneal
Slovak	Acetylcysteín	Liečba pseudomyxoma peritonei
Slovenian	Acetilcistein	Zdravljenje peritonealnega psevdomiksoma
Spanish	Acetilcisteina	Tratamiento de pseudomixoma peritoneal
Swedish	Acetylcystein	Behandling av pseudomyxoma peritonei
Norwegian	Acetylcystein	Behandling av pseudomyxoma peritonei
Icelandic	Acetyl cystein	Meðferð pseudomyxoma peritonei

¹ At the time of designation