

8 March 2017  
EMA/4662/2017  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Fluticasone propionate for the treatment of eosinophilic oesophagitis

On 12 January 2017, orphan designation (EU/3/16/1815) was granted by the European Commission to Adare Pharmaceuticals srl, Italy, for fluticasone propionate for the treatment of eosinophilic oesophagitis.

#### What is eosinophilic oesophagitis?

Eosinophilic oesophagitis is a disease characterised by inflammation of the oesophagus (the tube that leads from the mouth to the stomach) caused by the build-up of a type of white blood cell called eosinophils. The main symptoms of the disease are dysphagia (difficulty swallowing), which may lead to food getting stuck in the oesophagus, heartburn and chest pain.

Eosinophilic oesophagitis is a long-term debilitating disease that leads to oesophageal stenosis (narrowing of the oesophagus), and a scarred and fragile oesophageal wall.

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

At the time of designation, eosinophilic oesophagitis affected approximately 3.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 195,000 people<sup>\*</sup>, 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?

No satisfactory methods were authorised for treating eosinophilic oesophagitis at the time of the application. As food allergy was thought to be a possible cause of the disease, certain food was excluded from the diet. Corticosteroids were given to reduce inflammation and improve symptoms. Oesophageal dilation (widening) was used in some patients but there was a risk of perforation (tearing) of the fragile oesophageal wall in patients with eosinophilic oesophagitis.

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<sup>\*</sup>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 513,700,000 (Eurostat 2016).

## How is this medicine expected to work?

Fluticasone propionate is a corticosteroid that reduces inflammation. The medicine will be available in a formulation to be taken by mouth in order to deliver fluticasone propionate to the oesophagus and the upper part of the gut. This is expected to reduce inflammation and relieve the symptoms of eosinophilic oesophagitis.

## What is the stage of development of this medicine?

The effects of fluticasone propionate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with eosinophilic oesophagitis were ongoing.

At the time of submission, fluticasone propionate was not authorised anywhere in the EU for eosinophilic oesophagitis. Orphan designation of the medicine had been granted in the United States for eosinophilic oesophagitis in adults and children.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2016 recommending the granting of this designation.

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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 EMA website, on the medicine's [rare disease designations page](#).

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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Fluticasone propionate	Treatment of eosinophilic oesophagitis
Bulgarian	Флутиказон пропионат	Лечение на еозинофилен езофагит
Croatian	Flutikazon propionat	Liječenje eozinofilnog ezofagitisa
Czech	Flutikazon-propionát	Léčba eozinofilní ezofagitidy
Danish	Fluticasonpropionat	Behandling af eosinofil øsofagit
Dutch	Fluticasonpropionaat	Behandeling van eosinofiele oesofagitis
Estonian	Flutikasoonpropionaat	Eosinofiilse ösofagiidi ravi
Finnish	Flutikasonipropionaatti	Eosinofiilisen esofagiitin hoito
French	Propionate de fluticasone	Traitement de l'œsophagite à éosinophiles
German	Fluticasonpropionat	Behandlung einer eosinophilen Ösophagitis
Greek	Προπιονική φλουτικάζονη	Θεραπεία της ηωσινοφιλικής οισοφαγίτιδας
Hungarian	Flutikazon-propionát	Eosinophil oesophagitis kezelése
Italian	Fluticasone propionato	Trattamento dell'esofagite eosinofila.
Latvian	Flutikazona propionāts	Eozinofilā ezofagīta ārstēšana
Lithuanian	Flutikazono propionatas	Eozinofilinio ezofagito gydymas
Maltese	Fluticasone propionate	Kura tal-esofagite eosinofilika
Polish	Propionian flutykazonu	Leczenie eozynofilowego zapalenia przełyku
Portuguese	Propionato de fluticasona	Tratamento da esofagite eosinofílica
Romanian	Propionat de fluticazonă	Tratamentul esofagitei eozinofilice
Slovak	Flutikazón propionát	Liečba eozinofilnej ezofagitídy
Slovenian	Flutikazonpropionat	Za zdravljenje eozinofilnega ezofagitisa
Spanish	Propionato de fluticasona	Para el tratamiento de la esofagitis eosinofílica
Swedish	Flutikasonpropionat	Behandling av eosinofil esofagit
Norwegian	Flutikasonpropionat	Behandling av eosinofil øsofagitt
Icelandic	Flútikasónprópiónat	Meðferðar við eósínófil vélindisbólgu

<sup>1</sup> At the time of designation