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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Budesonide for the treatment of eosinophilic oesophagitis

On 5 August 2013, orphan designation (EU/3/13/1181) was granted by the European Commission to Dr Falk Pharma GmbH, Germany, for budesonide 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 excess of a type of white blood cell called eosinophils. The main symptoms of the disease are dysphagia (difficulty swallowing) and obstruction of the oesophagus.

Eosinophilic oesophagitis is a long-term debilitating disease that leads to oesophageal stenosis (narrowing of the oesophagus), which can only be treated with invasive procedures.

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

At the time of designation, eosinophilic oesophagitis affected less than 5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 255,000 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, no satisfactory methods were authorised in the EU for the treatment of eosinophilic oesophagitis. As allergy was thought to be a possible cause of the disease, allergens were excluded from the diet. Oesophageal dilation (widening) was performed in some patients, although it carries the risk of complication such as perforation of the oesophagus.

*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 509,000,000 (Eurostat 2013).

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How is this medicine expected to work?

Budesonide is a well-known corticosteroid mainly used by inhalation for the treatment of asthma. Corticosteroids help reduce inflammation by attaching to receptors of immune cells and reducing the release of substances that are involved in the inflammation process.

This medicine will be available as effervescent tablets to be dissolved in the mouth for use in adults, and a viscous (thick) suspension to be taken by mouth in children. These formulations are expected to release budesonide mainly in the oesophagus, reducing the inflammation and relieving the symptoms of eosinophilic oesophagitis.

What is the stage of development of this medicine?

The effects of budesonide have been evaluated in experimental models.

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

At the time of submission, budesonide was not authorised anywhere in the EU for eosinophilic oesophagitis. Orphan designation of budesonide had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2013 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Budesonide	Treatment of eosinophilic oesophagitis
Bulgarian	Будезонид	Лечение на еозинофилен езофагит.
Croatian	Budezonid	Liječenje eozinofilnog ezofagitisa
Czech	Budesonid	Léčba eosinofilní esofagitidy
Danish	Budesonid	Behandling af eosinofil øsofagit
Dutch	Budesonide	Behandeling van eosinofiele oesofagitis
Estonian	Budesoniid	Eosinofiilse ösofagiidi ravi
Finnish	Budesonidi	Eosinofiilisen esofagiitin hoito
French	Budésonide	Traitement de l'œsophagite à éosinophiles
German	Budesonid	Behandlung einer eosinophilen Ösophagitis
Greek	βουδεσονίδη	Θεραπεία της ηωσινοφιλικής οισοφαγίτιδας
Hungarian	Budezonid	Eosinophil kezelése
Italian	Budesonide	Trattamento dell'esofagite eosinofila.
Latvian	Budezonīds	Eozinofīlā ezofagīta ārstēšana
Lithuanian	Budezonidas	Eozinofilinio ezofagito gydymas
Maltese	Budesonide	Kura tal-esofaġite eosinofilika
Polish	Budezonid	Leczenie eozynofilowego zapalenia przełyku
Portuguese	Budesonida	Tratamento da esofagite eosinofílica
Romanian	Budesonidă	Tratamentul esofagitei eozinofilice
Slovak	Budezonid	Liečba eozinofilnej ezofagitídy
Slovenian	Budezonid	Za zdravljenje eozinofilnega ezofagitisa
Spanish	Budesónida	Para el tratamiento de la esofagitis eosinofílica
Swedish	Budesonid	Behandling av eosinofil esofagit
Norwegian	Budesonid	Behandling av eosinofil øsofagitt
Icelandic	Búdesóníð	Meðferðar við eósínófíl vélindisbólgu

[1] At the time of designation