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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human monoclonal antibody against human interleukin 13 for the treatment of eosinophilic oesophagitis

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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 13 November 2013, orphan designation (EU/3/13/1205) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for human monoclonal antibody against human interleukin 13 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), which may lead to food getting stuck in the oesophagus, heartburn and acid regurgitation (acid flowing up into the mouth).

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 256,000 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).

*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.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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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.

How is this medicine expected to work?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to interleukin 13 (IL-13). IL-13 is produced at high levels by immune cells in patients with eosinophilic oesophagitis and is involved in attracting eosinophils and inducing inflammation. By attaching to IL-13, the medicine blocks its activity, reducing the number of eosinophils in the oesophagus and relieving inflammation and the symptoms of the disease.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, one clinical trial with this medicine in patients with eosinophilic oesophagitis had been completed and further studies were planned.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2013 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Human monoclonal antibody against human interleukin 13	Treatment of eosinophilic oesophagitis
Bulgarian	Човешко моноклонално антитяло срещу човешки интерлевкин 13	Лечение на еозинофилен езофагит
Czech	Humánní monoklonální protilátka proti lidskému interleukinu 13	Léčba eosinofilní esofagitidy
Croatian	Ljudsko monoklonsko protutijelo protiv ljudskog interleukina 13	Liječenje eozinofilnog ezofagitisa
Danish	Humant monoklonalt antistof mod humant interleukin 13	Behandling af eosinofil øsofagit
Dutch	Humaan monoklonaal antilichaam gericht tegen humaan interleukine 13	Behandeling van eosinofiele oesofagitis
Estonian	Interleukiin 13 vastane inimese monoklonaalne antikeha	Eosinofiilse ösofagiidi ravi
Finnish	Ihmisen monoklonaalinen interleukiini 13:n vasta-aine	Eosinofiilisen esofagiitin hoito
French	Anticorps monoclonal humain dirigé contre l'interleukine 13 humaine	Traitement de l'œsophagite à éosinophiles
German	Humaner monoklonaler Antikörper gegen humanes Interleukin 13	Behandlung einer eosinophilen Ösophagitis
Greek	Ανθρώπινο μονοκλονικό αντίσωμα έναντι της ανθρώπινης ιντερλευκίνης 13	Θεραπεία της ηωσινοφιλικής οισοφαγίτιδας
Hungarian	Humán interleukin 13 ellenes human monoklonális antitest	Eosinophil eosophagitis kezelése
Italian	Anticorpo monoclonale umano contro l'interleuchina 13 umana	Trattamento dell'esofagite eosinofila.
Latvian	Cilvēka monoklonāla antiviela pret cilvēka interleikīnu 13	Eozinofīlā ezofagīta ārstēšana
Lithuanian	Žmogaus monokloninis antikūnas prieš žmogaus interleukiną 13	Eozinofilinio ezofagito gydymas
Maltese	Antikorp monoklonali uman kontra l-interleukin 13 uman	Kura tal-esofaġite eosinofilika
Polish	Ludzkie przeciwciało monoklonalne przeciw ludzkiej interleukinie 13	Leczenie eozynofilowego zapalenia przełyku
Portuguese	Anticorpo monoclonal humano anti interleucina 13 humana	Tratamento da esofagite eosinofílica
Romanian	Anticorp uman monoclonal anti-interleukină umană 13	Tratamentul esofagitei eozinofilice
Slovak	Ľudská monoklonálna protilátka proti ľudskému interleukínu 13	Liečba eozinofilnej ezofagitídy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humano monoklonsko protitelo proti humanemu interlevkinu 13	Za zdravljenje eozinofilnega ezofagitisa
Spanish	Anticuerpo monoclonal humano anti interleucina 13 humana	Para el tratamiento de la esofagitis eosinofílica
Swedish	Human monoklonal antikropp mot humant interleukin 13	Behandling av eosinofil esofagit
Norwegian	Humant monoklonalt antistoff mot human interleukin 13	Behandling av eosinofil øsofagitt
Icelandic	Einstofna mannamótefni gegn manna interleukíni 13	Meðferðar við eósínófíl vélindisbólgu