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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised monoclonal antibody targeting interleukin-15 for the treatment of eosinophilic oesophagitis

On 27 June 2016, orphan designation (EU/3/16/1681) was granted by the European Commission to Dr Alain Vicari, France, for humanised monoclonal antibody targeting interleukin-15 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 chest pain.

Eosinophilic oesophagitis is a long-term debilitating disease that leads to oesophageal stenosis (narrowing of the oesophagus), and scarring and fragility of the 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*, 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 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 performed in some patients but 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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

How is this medicine expected to work?

This medicine is a monoclonal antibody (a type of protein) that has been designed to attach to interleukin 15 (IL-15). IL-15 is produced at high levels in the oesophagus of patients with eosinophilic oesophagitis and is involved in attracting eosinophils and inducing inflammation. By attaching to IL-15, 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?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with eosinophilic oesophagitis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for eosinophilic oesophagitis 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 19 May 2016 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 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Humanised monoclonal antibody targeting interleukin-15	Treatment of eosinophilic oesophagitis
Bulgarian	Хуманизирано моноклонално антитяло срещу интерлевкин-15	Лечение на еозинофилен езофагит
Croatian	Humanizirano monoklonsko protutijelo protiv interleukina-15	Liječenje eozinofilnog ezofagitisa
Czech	Humanizovaná monoklonální protilátka proti interleukinu-15	Léčba eozinofilní ezofagitidy
Danish	Humaniseret monoklonalt antistof mod interleukin-15	Behandling af eosinofil øsofagit
Dutch	Gehumaniseerd monoklonaal antilichaam gericht tegen interleukine-15	Behandeling van eosinofiele oesofagitis
Estonian	Interleukiin-15 vastane humaniseeritud monoklonaalne antikeha	Eosinofiilse ösofagiidi ravi
Finnish	Humanisoitu monoklonaalinen interleukiini-15 vasta-aine	Eosinofiilisen esofagiitin hoito
French	Anticorps monoclonal humanisé dirigé contre l'interleukine-15	Traitement de l'œsophagite à éosinophiles
German	Humanisierter monoklonaler Antikörper gegen Interleukin-15	Behandlung einer eosinophilen Ösophagitis
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι της ιντερλευκίνης-15	Θεραπεία της ηωσινοφιλικής οισοφαγίτιδας
Hungarian	Interleukin-15 ellenes humanizált monoklonális antitest	Eosinophil eosophagitis kezelése
Italian	Anticorpo monoclonale umanizzato contro l'interleuchina-15	Trattamento dell'esofagite eosinofila.
Latvian	Humanizēta monoklonāla antivielā pret interleikīnu-15	Eozinofilā ezofagīta ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas prieš interleukiną 15	Eozinofilinio ezofagito gydymas
Maltese	Antikorp monoklonali umanizzat kontra l-interleukin-15	Kura tal-esofagite eosinofilika
Polish	Humanizowane przeciwciało monoklonalne przeciw interleukinie-15	Leczenie eozynofilowego zapalenia przełyku
Portuguese	Anticorpo monoclonal humanizado direcionado para a interleucina-15	Tratamento da esofagite eosinofílica
Romanian	Anticorp monoclonal umanizat anti-interleukină-15	Tratamentul esofagitei eozinofilice
Slovak	Humanizovaná monoklonálna protilátka proti interleukínu-15	Liečba eozinofilnej ezofagitídy

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Humanizirano monoklonsko protitelo proti interleukinu-15	Za zdravljenje eozinofilnega ezofagitisa
Spanish	Anticuerpo monoclonal humanizado anti interleucina-15	Para el tratamiento de la esofagitis eosinofílica
Swedish	Humaniserad monoklonal antikropp mot interleukin-15	Behandling av eosinofil esofagit
Norwegian	Humanisert monoklonalt antistoff mot interleukin-15	Behandling av eosinofil øsofagitt
Icelandic	Mannaaðlagað einstofna mótefni gegn interleukíni-15	Meðferðar við eósínófíl vélindisbólgu