



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8 for the treatment of mastocytosis

On 16 October 2017, orphan designation (EU/3/17/1929) was granted by the European Commission to Envestia Limited, United Kingdom, for recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8 (also known as AK002) for the treatment of mastocytosis.

What is mastocytosis?

Mastocytosis is a group of disorders in which there are too many mast cells, a type of white blood cell, in various organs in the body. These cells release large amounts of histamine and other chemicals into the blood, causing symptoms such as a skin rash, itchy skin and hot flushes.

In children, the disorder usually only affects the skin ('cutaneous mastocytosis') and causes a red and itchy rash, which may disappear on its own. In some patients, mainly adults, the disorder progresses into 'systemic mastocytosis', in which the mast cells infiltrate organs, such as the intestine, the liver, the spleen and the bone marrow. This causes various symptoms such as palpitations and fainting, bone pain, tiredness, weight loss, diarrhoea, nausea (feeling sick), vomiting and stomach ache.

Mastocytosis is a condition that is debilitating in the long term and may be life threatening in those patients who develop the systemic form of the disorder.

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

At the time of designation, mastocytosis affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 155,000 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. This represents a population of 515,700,000 (Eurostat 2017).



What treatments are available?

At the time of designation, treatments available in the EU aimed at relieving some of the symptoms of mastocytosis, including antihistamines such as cimetidine or famotidine to block the action of histamine produced by the mast cells.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with mastocytosis, with studies showing that the medicine reduces levels of mast cells and of eosinophils, another type of white blood cell found at high levels in mastocytosis.

This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

This medicine is a monoclonal antibody, a type of protein, that is designed to attach to a target on mast cells known as sialic acid-binding Ig-like lectin 8 (siglec 8). By attaching to siglec 8, the medicine blocks the growth of mast cells and their release of histamine and other chemicals. This is expected to relieve symptoms of the condition.

What is the stage of development of this medicine?

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

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

At the time of submission, the medicine was not authorised anywhere in the EU for mastocytosis 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 7 September 2017 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	Recombinant monoclonal antibody to sialic acid-binding Ig-like lectin 8	Treatment of mastocytosis
Bulgarian	Рекомбинантно моноклонално антитяло срещу Ig-подобен лектин 8, свързващ сialова киселина	Лечение на мастоцитоза
Croatian	Rekombinantno monoklonsko protutijelo na lektin 8 sličan Ig-u koji se veže na sijaličnu kiselinu	Liječenje mastocitoze
Czech	Rekombinantní monoklonální protilátka ke kyselině sialové vázající Ig-podobný lektin 8	Léčba mastocytózy
Danish	Rekombinant monoklonal antistof rettet mod sialicsyrebindende Ig-lignende lektin 8	Behandling af mastocytose
Dutch	Recombinant monoklonaal antilichaam tegen siaalzuurbindend Ig-achtig lectine 8	Behandeling van mastocytose
Estonian	Rekombinantne monoklonaalne antikeha siaalhappega siduva Ig-sarnase lektiini 8 vastu	Mastotsütoosi ravi
Finnish	Rekombinantti monoklonaalinen vasta-aine siaalihappoon sitoutuvalle Ig:n kaltaiselle lektiini 8:lle	Mastosytoosin hoito
French	Anticorps monoclonal recombinant anti-lectine 8 de type Ig liant l'acide sialique	Traitement de la mastocytose
German	Rekombinanter monoklonaler Antikörper gegen antibody to sialic acid-binding Ig-like lectin (SIGLEC) 8	Behandlung der Mastozytose
Greek	Ανασυνδυασμένο μονοκλωνικό αντίσωμα εναντι της λεκτίνης-8 τύπου ανοσοσφαιρίνης που δεσμεύει το σιαλικό οξύ	Θεραπεία της μαστοκυττάρωσης
Hungarian	Szialisavkötő Ig-szerű lektin 8 elleni rekombináns monoklonális antitest	Mastocytosis kezelése
Italian	Anticorpo monoclonale ricombinante verso la lectina 8 Ig-simile legante l'acido sialico	Trattamento della mastocitosi
Latvian	Rekombinanta monoklonāla antivielā pret Ig līdzīgu siāliskābi saistošu lektīnu 8	Mastocitozes ārstēšana
Lithuanian	Rekombinantinis monokloninis antikūnas prieš į Ig panašų, sialo rūgštį surišantį lektiną 8	Mastocitozės gydymas
Maltese	Antikorp monoklonali rikombinanti għal lektina 8 simili għal Ig li tehel mal-aċidu sijaliku	Kura tal-mastocitosi
Polish	Rekombinowane monoklonalne przeciwciało skierowane przeciw immunoglobulinopodobnej wiążącej kwas sialowy lektynie 8	Leczenie mastocytozy
Portuguese	Anticorpo monoclonal recombinante para a ligação ao ácido siálico da lectina 8 semelhante a Ig	Tratamento da mastocitose
Romanian	Anticorp monoclonal recombinant anti-lectina 8 Ig-like ce leagă acidul sialic	Tratamentul mastocitozei

¹ At the time of designation

Language	Active ingredient	Indication
Slovak	Rekombinantná monoklonálna protilátka voči kyseline sialovej viažuca Ig podobný lektín 8	Liečba mastocytózy
Slovenian	Rekombinantno monoklonsko protitelo proti Ig podobnemu lektinu 8, ki veže sialno kislino	Zdravljenje dermatofibrosarkoma protuberans
Spanish	Anticuerpo monoclonal recombinante contra la lectina 8 de tipo Ig de unión al ácido siálico	Tratamiento de la mastocitosis
Swedish	Rekombinant monoklonal antikropp mot sialinsyrabindande Ig-liknande lektin 8	Behandling av mastocytos
Norwegian	Rekombinant monoklonalt antistoff mot sialinsyrebindende Ig-lignende lektin 8	Behandling av mastocytose
Icelandic	Raðbrigða einstofna mótefni gegn sialínsýrubindandi Ig-líku lektíni 8	Meðferð mastócýtosis