



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

25 February 2015  
EMA/COMP/822954/2009 Rev.2  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class for the treatment of chronic non-infectious uveitis

|                                                                                                                                                                                                                                                                                                                         |                  |
|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|------------------|
| First publication                                                                                                                                                                                                                                                                                                       | 3 March 2010     |
| Rev.1: sponsor's name change                                                                                                                                                                                                                                                                                            | 5 February 2015  |
| Rev.2: withdrawal from the Community Register                                                                                                                                                                                                                                                                           | 25 February 2015 |
| <b>Disclaimer</b><br>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. |                  |

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2015 on request of the Sponsor.***

On 2 February 2010, orphan designation (EU/3/09/724) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class for the treatment of chronic non-infectious uveitis.

### What is chronic non-infectious uveitis?

Uveitis is inflammation of the uvea, the middle layer of the eye, which includes the iris. Uveitis can affect one or both eyes and may cause discomfort, pain, and blurring of the vision. In chronic (long-term) non-infectious uveitis the inflammation is usually caused by the body's immune system (the body's natural defences) attacking normal tissue. It is not caused by an infection.

Chronic non-infectious uveitis is a long-term debilitating disease because it may lead to blindness.



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

At the time of designation, chronic non-infectious uveitis affected less than 4.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 243,000 people\*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

## **What treatments are available?**

At the time of designation, several medicines were authorised in Member States of the EU for the treatment of chronic non-infectious uveitis. Most treatments aimed to reduce the inflammation by lowering the activity of the immune system.

The sponsor has provided sufficient information to show that recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class might be of significant benefit for patients with chronic non-infectious uveitis because early studies indicate that it might improve the treatment of patients with this condition. 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?**

Recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to interleukin-17A (IL-17A). IL-17A is a chemical messenger that is thought to be produced in high levels in patients with chronic non-infectious uveitis, causing the inflammation. By attaching to IL-17A, the medicine blocks its activity, helping to relieve the symptoms of the disease.

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

The effects of recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class have been evaluated in experimental models.

At the time of submission of the application for orphan designation, one 16-patient study in patients with chronic non-infectious uveitis had been completed.

At the time of submission, human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class was not authorised anywhere in the EU for chronic non-infectious uveitis 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 3 December 2009 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 Community) or insufficient returns on investment.

---

\*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. At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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:

Novartis Europharm Limited  
Frimley Business Park  
Camberley GU16 7SR  
United Kingdom  
Tel. +41 61 324 11 11 (Switzerland)  
E-mail: [orphan.enquiries@novartis.com](mailto:orphan.enquiries@novartis.com)

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, Norwegian and Icelandic

| Language   | Active ingredient                                                                                           | Indication                                                          |
|------------|-------------------------------------------------------------------------------------------------------------|---------------------------------------------------------------------|
| English    | Recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class                     | Treatment of chronic non-infectious uveitis                         |
| Bulgarian  | Рекомбинантно човешко моноклонално анти тяло от клас IgG1/k срещу човешки интерлевкин (IL)-17A              | Лечение на хроничен неинфекциозен увеит                             |
| Czech      | Rekombinantní humánní monoklonální protilátka proti humánnímu Interleukinu-(IL)-17A třídy IgG1/k            | Léčba chronické neinfekční uveitidy                                 |
| Danish     | Rekombinant humant monoklonalt antistof mod human interleukin (IL)-17A af IgG1/k klassen                    | Behandling af kronisk ikke-infektøs uveitis                         |
| Dutch      | Recombinant humaan monocloonaal anti-antilichaam tegen humaan Interleukine-17A van de IgG1/K klasse         | Behandeling van chronische niet-infectieuze uveitis                 |
| Estonian   | Inimese IgG1/k klassi interleukiini (IL)-17A vastane rekombinantne inimese monoklonaalne antikeha           | Kroonilise mitteinfektsioosse uveidi ravi.                          |
| Finnish    | Rekombinoitu ihmisen monoklonaalinen vasta-aine ihmisen IgG1/k -luokan interleukiini (IL)-17A:ta vastaan    | Kroonisen, ei-infektioperäisen uveitin hoito                        |
| French     | Anticorps monoclonal humain recombinant anti-interleukine humaine (IL)-17A de classe IgG1/k                 | Traitement de l'uvéite chronique non infectieuse                    |
| German     | Rekombinanter humaner monoklonaler Antikörper gegen humanes Interleukin (IL) -17A der Klasse IgG1/k         | Behandlung der chronischen nicht-infektiösen Uveitis                |
| Greek      | Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα έναντι της ανθρώπινης Ιντερλευκίνης (IL)-17A της τάξης IgG1/k | Θεραπεία της χρόνιας μη μολυσματικής ραγοειδίτιδας.                 |
| Hungarian  | Rekombináns, human, monoklonális, IgG1/k osztályú humán interleukin (IL)-17A ellenes antitest               | Krónikus, nem fertőzőes eredetű uveitis kezelése                    |
| Italian    | Anticorpo monoclonale ricombinante umano per l'interleuchina umana (IL)-17A, di classe IgG1/k               | Trattamento dell'uveite cronica non infettiva                       |
| Latvian    | Rekombinantā monokloniāla humanizēta antivielā pret cilvēka IgG1/k klases interleikīnu (IL)-17A             | Hroniskā neinfekciozā uveīta ārstēšana                              |
| Lithuanian | Rekombinantinis žmogaus monokloninis IgG1/k klasės antikūnas prieš žmogaus interleukiną (IL)-17A            | Lėtinio neinfekcinio uveito gydymas                                 |
| Maltese    | Anti-korp monoklonali rikombinanti uman għal interleukin (IL)-17A uman tal-klassi IgG1/k                    | Kura ta' l-ujejite kronika u mhux infettiva                         |
| Polish     | Ludzkie rekombinowane przeciwciało monoklonalne klasy IgG1/k przeciwko ludzkiej interleukinie (IL) – 17A    | Leczenie przewlekłego niezakaźnego zapalenia błony naczyniowej oka. |
| Portuguese | Anticorpo monoclonal humano recombinante da classe IgG1/k anti interleucina (IL)-17A                        | Tratamento da uveíte crónica não infecciosa                         |

| Language  | Active ingredient                                                                                | Indication                                                   |
|-----------|--------------------------------------------------------------------------------------------------|--------------------------------------------------------------|
| Romanian  | Anticorp monoclonal uman recombinant anti-interleukină-17A umană (IL) din clasa IgG1/k           | Tratamentul uveitei non-infecțioase cronice                  |
| Slovak    | Rekombinantná ľudská monoklonálna protilátka proti ľudskému interleukínu (IL)-17A triedy IgG1/k  | Liečba chronickej neinfekčnej uveitídy                       |
| Slovenian | rekombinantno humano monoklonsko protitelo proti humanemu interlevkinu (IL)-17A razreda IgG1/k   | Zdravljenje kroničnega neinfekcijskega uveitisa              |
| Spanish   | Anticuerpo monoclonal humano recombinante contra la interleucina (IL)-17A humana de clase IgG1/k | El tratamiento de la uveitis crónica no infecciosa           |
| Swedish   | Rekombinant human monoklonal antikropp mot human Interleukin (IL)-17A av IgG1/k klassen          | Behandling av kronisk icke infektiös uveit                   |
| Norwegian | Rekombinant humant monoklonalt antistoff mot human Interleukin (IL)-17A av IgG1/k klassen        | Behandling av kronisk ikke-infeksiøs uveitt,                 |
| Icelandic | Einstofna, manna raðbrigða mótefni gegn manna Interleukín (IL)-17A af flokki IgG1/k              | Til meðferðar á augnæðahjúpsbólgu sem ekki stafar af sýkingu |