



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 February 2015
EMA/COMP/736522/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous collagen type II-specific regulatory T cells for the treatment of non-infectious uveitis

On 16 December 2014, orphan designation (EU/3/14/1405) was granted by the European Commission to TxCell, France, for autologous collagen type II-specific regulatory T cells for the treatment of non-infectious uveitis.

What is non-infectious uveitis?

Uveitis is inflammation of the uvea, the middle layer of the eye, just beneath the white part of the eye. The inflammation can affect one or both eyes, and may cause discomfort, pain, and blurring of vision. Non-infectious uveitis is usually caused by the body's immune system (the body's natural defences) attacking normal tissue and not by an infection.

Non-infectious uveitis is a long-term debilitating disease because it may lead to partial or complete loss of vision (blindness).

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

At the time of designation, non-infectious uveitis affected approximately 4.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 245,000 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, several medicines were authorised in Member States of the EU for the treatment of non-infectious uveitis. The first treatment option was corticosteroids, which were used to reduce the inflammation by lowering the activity of the immune system. Other immunosuppressant agents such as ciclosporin were also authorised for use in non-infectious uveitis.

*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 511,100,000 (Eurostat 2014).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with non-infectious uveitis because studies in experimental models showed that the product may have improved efficacy compared with available treatments, and its use may result in a reduction in the use of corticosteroids. These assumptions 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 an advanced therapy medicine that belongs to the group called 'somatic cell therapy products'. These are medicines that contain cells or tissues that have been manipulated to change their biological characteristics so that they can be used to cure, diagnose or prevent a disease.

To prepare this medicine, certain immune cells called regulatory T cells are extracted from the patient's blood and those regulatory T cells that attach specifically to collagen type II, a protein present only in the eye and the joints, are harvested to increase their numbers. They are then given back to the patient, where they preferentially migrate to the inflamed tissue in the eye and are 'activated' by collagen type II, triggering a cascade of activities that lead to a reduction in inflammation and hence to easing of the patient's symptoms.

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, no clinical trials with the medicine in patients with non-infectious uveitis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for 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 13 November 2014 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:

TxCell
Allée de la Nertière - Les Cardoulines
F-06560 Valbonne - Sophia Antipolis
France
Tel. +33 497 21 83 01
Fax +33 493 64 15 80
E-mail: contact@txcell.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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Autologous collagen type II-specific regulatory T cells	Treatment of non-infectious uveitis
Bulgarian	Автоложни регулаторни Т клетки, специфични за колаген тип II	Лечение на хроничен неинфекциозен увеит
Croatian	Autologne regulacijske T-stanice specifične za kolagen tipa II	Liječenje neinfektivnog uveitisa
Czech	Autologní regulační T buňky specifické pro kolagen typu II	Léčba neinfekční uveitidy
Danish	Autologe, kollagen-type II-specifikke, regulatoriske T-celler	Behandling af non-infektøs uveitis
Dutch	Autologe collageen type II-specifieke regulatoire T-cellen	Behandeling van niet-infectieuze uveïtis
Estonian	Autoloogsed kollageen tüüp II spetsiifilised reguleeritud T-rakud	Mitte-infektsioosse uveiidi ravi
Finnish	Autologiset kollageeni II-tyyppispesifiset regulatoriset T-solut	Ei-infektioperäisen uveitin hoito
French	Cellules T régulatrices, autologues, spécifiques du collagène de type II	Traitement de l'uvéïte non infectieuse
German	Autologe Kollagen typ II-spezifische regulatorische T-Zellen	Behandlung der nicht-infektiösen Uveitis
Greek	Αυτόλογα ρυθμιστικά Τ-κύτταρα ειδικά για κολλαγόνο τύπου II	Θεραπεία της μη μολυσματικής ραγοειδίτιδας.
Hungarian	II-es típusú kollagénre specifikus autológ regulátor T-sejtek	Nem fertőzőeses eredetű uveitis kezelésé
Italian	Cellule T regolatrici autologhe specifiche di collagene di tipo II	Trattamento dell'uveite non infettiva
Latvian	Autologās II tipa kolagēna -specifiskās regulatorās T šūnas	Ne-infekciozā uveīta ārstēšana
Lithuanian	Autologiniam II tipo kolagenui specifinės reguliacinės T ląstelės	Neinfekcinio uveito gydymas
Maltese	Ċelluli T regolatorji awtologi speċifiċi għall-kollaġen tat-tip II	Kura ta' uveite mhux infettiva
Polish	Autologiczne limfocyty T regulatorowe swoiste wobec kolagenu typu II	Leczenie nieinfekcyjnego zapalenia błony naczyniowej oka
Portuguese	Células T reguladoras autólogas específicas do colagénio tipo II	Tratamento da uveíte não infecciosa
Romanian	Celule T reglatoare specifice pentru colagenul autolog de tip II	Tratamentul uveitei non-infectioase
Slovak	Autológne regulačné T bunky špecifické pre kolagén typu II	Liečba neinfekčnej uveitídy

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Avtologne regulatorne celice T, specifične za kolagen tipa II	Zdravljenje neinfekcijskega uveitis
Spanish	Células T reguladoras autólogas específicas de colágeno tipo II	Tratamiento de la uveítis no infecciosa
Swedish	Autologa regulatoriska T-celler som är specifika för kollagentyp II	Behandling av icke-infektiös uveit
Norwegian	Autologe kollagen type II-spesifikke regulatoriske T-celler	Behandling av ikke-infeksiøs uveitt
Icelandic	Samgena T-stýrfrumur sértækar fyrir kollagen af gerð II	Meðferð á æðahjúpsbólgu án sýkingar