10 November 2014
EMA/COMP/553659/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human monoclonal antibody of the IgG1 kappa class against human macrophage colony-stimulating factor for the treatment of tenosynovial giant cell tumour, localised and diffused type

On 15 October 2014, orphan designation (EU/3/14/1350) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for recombinant human monoclonal antibody of the IgG1 kappa class against human macrophage colony-stimulating factor for the treatment of tenosynovial giant cell tumour, localised and diffuse type.

What is tenosynovial giant cell tumour, localised and diffused type?

Tenosynovial giant cell tumour is a condition where the tissue surrounding the joints and tendons, called the synovial lining or synovium, expands abnormally forming outgrowths of the joint. It is known as 'diffuse' if the entire synovium is affected, or 'localised' if only a section of the synovium is affected. It usually affects the hand joint of young adults and is characterised by pain, swelling and stiffness of the joint.

Tenosynovial giant cell tumour is a long-term debilitating disease because it causes the destruction of joints.

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

At the time of designation, tenosynovial giant cell tumour, localised and diffuse type, affected not more than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 102,000 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 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of tenosynovial giant cell tumour and treatment consisted of surgery.

How is this medicine expected to work?

The medicine contains a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein called macrophage colony-stimulating factor (MCSF). MCSF is produced in large amounts by tenosynovial giant cell tumour cells, where it stimulates immune cells called macrophages to accumulate in the joints and cause the outgrowths. By attaching to MCSF the medicine is expected to block its activity, preventing tumour growth and helping to delay the onset of the symptoms of the disease.

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 tenosynovial giant cell tumour, localised and diffuse were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for tenosynovial giant cell tumour, localised and diffuse 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 4 September 2014 recommending the granting of this designation.

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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:

Novartis Europharm Limited
Frimley Business Park
Camberley GU16 7SR
United Kingdom
Tel. +41 61 324 11 11 (Switzerland)
E-mail: 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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human monoclonal antibody of the IgG1 kappa class against human macrophage colony-stimulating factor	Treatment of tenosynovial giant cell tumour, localised and diffused type
Bulgarian	Рекомбинантно човешко моноклонално антитяло от клас IgG1 капа срещу човешки макрофаг колония-стимулиращ фактор	Лечение на теносиновиален гигантоклетъчен тумор, локализиран и дифузен тип
Croatian	Rekombinantno ljudsko monoklonsko protutijelo je IgG1 kappa klase protiv ljudskog fakt ora stimulacije rasta makrofagnih kolonija	Liječenje gigantocelularnog tenosinovijalnog tumora, lokaliziranog i difuznog tipa
Czech	Rekombinantní lidská monoklonální protilátka třídy IgG1 kappa k lidskému kolonie makrofágů stimulujícímu faktoru	Léčba tenosynoviálního obrovskobuněčného tumoru, lokalizovaného a difuzního typu
Danish	Rekombinant humant monoklonalt antistof IgG1 kappa-klasse mod human makrofag-kolonistimulerende faktor	Behandling af tenosynovial kæmpecelletumor, lokaliseret og diffus type
Dutch	Recombinant humaan monoklonaal antilichaam van het IgG1-kappa klasse tegen humaan macrofaag-kolonie stimulerende factor	Behandeling van tenosynoviale reusceltumoren, van het gelokaliseerde en diffuse type
Estonian	Rekombinantne inimese monoklonaalne IgG1 kapa klassi antikeha inimese makrofaagi kolooniaid stimuleeriva faktori vastu	Lokaalset ja difuusset tüüpi tenosünoviaalse hiidrakulise kasvaja ravi
Finnish	Rekombinantti ihmisen monoklonaalinen vasta-aine IgG1 kappa-luokan ihmisen makrofagipesäkkeitä stimuloivaa tekijää vastaan	Jännetupen jättisolukasvaimen hoito, paikallinen ja levinnyt tyyppi

[1] At the time of designation

Language	Active ingredient	Indication
French	Anticorps monoclonal recombinant humain de la classe IgG1 kappa contre macrophages facteur de stimulation de colonie humaine	Traitement des tumeurs ténosynoviales à cellules géantes, de type localisées et diffuses
German	Rekombinanter humaner monoklonaler Antikörper der IgG1-kappa-Klasse gegen menschlichen Makrophagen-Kolonie-stimulierenden Faktor	Behandlung des tenosynovialen Riesenzelltumors vom lokalisierten und diffusen Typ
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα της κατηγορίας IgG1 κάππα έναντι ανθρώπινου παράγοντα διέγερσης αποικιών μακροφάγων	Θεραπεία του τενοντο-αρθρικού γιγαντοκυτταρικού όγκου, εντοπισμένου και διάχυτου τύπου
Hungarian	Humán makrofág kolónia-stimuláló faktor elleni IgG1 kappa osztályú rekombináns human monoklonális antitest	Lokalizált és diffúz típusú tenosynovialis óriássejtes tumor kezelésére
Italian	Anticorpo ricombinante monoclonale umano i della classe IgG1 kappa contro il fattore stimolante le colonie di macrofagi umani	Trattamento di sinovite pigmentata villonodulare e tumore tenosinoviale a cellule giganti di tipo localizzato e diffuso
Latvian	Rekombinanta IgG1 kappa klases cilvēka monoklonāla antiviela pret cilvēka makrofāgu koloniju stimulējošo faktoru	Norobežota vai difūza tipa tenosinoviālo gigantisko šūnu audzēja ārstēšana
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas iš IgG1 kapa klasės prieš žmogaus makrofagų kolonijas stimuliuojantį faktorių	Tenosinovialinių stambiųjų ląstelių lokalaus ir išplitusio tipo naviko gydymas
Maltese	Antikorp monoklonali uman rikombinanti tal-klassi IgG1 kappa kontra fattur li jistimula kolonji makrofagu uman	Kura ta' tumur tenosinovjali ta' ċelluli ġganti, tat-tip lokalizzat u mxerred
Polish	Rekombinowane ludzkie przeciwciało monoklonalne klasy IgG1 kappa przeciw ludzkiemu czynnikowi stymulującemu wzrost kolonii makrofagów	Leczenie guza olbrzymiokomórkowego pochewki ścięgnistej, miejscowego i rozsianego typu

Language	Active ingredient	Indication
Portuguese	Anticorpo monoclonal humano recombinante da classe IgG1 kappa (κ) anti fator estimulante de colónias de macrófagos humanos	Tratamento do tumor tenosinovial de células gigantes, localizado e difuso.
Romanian	Anticorp monoclonal uman recombinant din clasa IgG1 kappa împotriva factorului de stimulare al coloniilor de macrofage uman \	Tratamentul tumorilor tenosinoviale cu celule gigant, localizate şi difuze
Slovak	Rekombinantná ľudská monoklonálna protilátka triedy IgG1 kappa proti ľudskému faktor u stimulujúcemu kolónie makrofágov	Liečba tendosynoviálneho obrovskobunkového nádora, lokalizovaného a difúzneho typu
Slovenian	Rekombinantno humano monoklonsko protitelo razreda IgG1 kapa proti makrofagov faktor človeškega kolonije stimulirajoči	Zdravljenje tenosinuvialnega gigantocelularnega lokaliziranega ali difuznega tumorja
Spanish	Anticuerpo monoclonal humano recombinante de la clase IgG1 kappa contra el factor estimulante de colonias de macrófagos humano	Tratamiento de tumores tenosinoviales de células gigantes, formas difusa y localizada
Swedish	Rekombinant human monoklonal antikropp enligt IgG1 kappa klassen mot humant makrofag-kolonistimulerande faktor	Behandling av tenosynovial jättecellstumör, lokal och diffuse typ
Norwegian	Rekombinant humant monoklonalt antistoff av IgG1 kappa klassen mot human makrofag-kolonistimulerende faktorfactor	Behandling av tenosynovial kjempecelletumor, lokalisert og diffus type
Icelandic	Raðbrigða mannat einstofna mótefni af IgG1 kappa flokki gegn manna stórátfruma colony-stimulating factor	Meðferð við litaðri totu-hnökrahálahimnubólgu