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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cabiralizumab for the treatment of tenosynovial giant cell tumour, localised and diffuse type

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On 12 December 2016, orphan designation (EU/3/16/1799) was granted by the European Commission to Albany Regulatory Consulting Ltd, United Kingdom, for cabiralizumab for the treatment of tenosynovial giant cell tumour, localised and diffuse type.

What is tenosynovial giant cell tumour, localised and diffuse 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 'localised' if only one site of the body is affected, or 'diffuse' when several sites are affected. It usually affects the hand joints 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 less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 154,000 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 tenosynovial giant cell tumour and treatment consisted of surgery and medicines to reduce inflammation.

*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).

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

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How is this medicine expected to work?

Patients with tenosynovial giant cell tumours produce too much of a protein called colony-stimulating factor 1 (CSF1). This causes a build-up of immune cells called macrophages in joints, leading to the outgrowths.

Cabiralizumab attaches to and blocks the receptor to which CSF1 usually attaches, called CSF1R. This prevents CSF1 from working and is therefore expected to prevent outgrowths in the joints and thus delay the onset of symptoms of the disease.

What is the stage of development of this medicine?

The effects of cabiralizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with cabiralizumab in patients with tenosynovial giant cell tumour, localised and diffuse type were ongoing.

At the time of submission, cabiralizumab was not authorised anywhere in the EU for tenosynovial giant cell tumour, localised and diffuse. Orphan designation of the medicine had been granted in the United States for pigmented villonodular synovitis and for tenosynovial giant cell tumor.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 November 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.

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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.

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cabiralizumab	Treatment of tenosynovial giant cell tumour, localised and diffuse type
Bulgarian	Кабирализумаб	Лечение на теносиновиален гигантоклетъчен тумор, локализиран и дифузен тип
Croatian	Kabiralizumab	Liječenje gigantocelularnog tenosinovijalnog tumora, lokaliziranog i difuznog tipa
Czech	Kabiralizumab	Léčba tenosynoviálního obrovskobuněčného tumoru, lokalizovaného a difuzního typu
Danish	Cabiralizumab	Behandling af tenosynovial kæmpecelletumor, lokaliseret og diffus type
Dutch	Cabiralizumab	Behandeling van tenosynoviale reusceltumoren, van het gelokaliseerde en diffuse type
Estonian	Kabiralizumaab	Lokaalset ja difuusset tüüpi tenosünoviaalse hiidrakulise kasvaja ravi
Finnish	Kabiralitsumabi	Jännetupen jättisolukasvaimen hoito, paikallinen ja levinnyt tyyppi
French	Cabiralizumab	Traitement des tumeurs ténosynoviales à cellules géantes, de type localisées et diffuses
German	Cabiralizumab	Behandlung des tenosynovialen Riesenzelltumors vom lokalisierten und diffusen Typ
Greek	Καμπιραλιζουμάμπη	Θεραπεία του τενοντο-αρθρικού γιγαντοκυτταρικού όγκου, εντοπισμένου και διάχυτου τύπου
Hungarian	Kabiralizumab	Lokalizált és diffúz típusú tenosynovialis óriássejtes tumor kezelésére
Italian	Cabiralizumab	Trattamento di sinovite pigmentata villonodulare e tumore tenosinoviale a cellule giganti di tipo localizzato e diffuso
Latvian	Kabiralizumabs	Norobežota vai difūza tipa tenosinoviālo gigantisko šūnu audzēja ārstēšana
Lithuanian	Kabiralizumabas	Tenosinovialinių stambiųjų ląstelių lokalaus ir išplitusio tipo naviko gydymas
Maltese	Cabiralizumab	Kura ta' tumur tenosinovjali ta' ċelluli ġganti, tat-tip lokalizzat u mxerred
Polish	Kabiralizumab	Leczenie guza olbrzymiokomórkowego pochewki ścięgnistej, miejscowego i rozsianego typu
Portuguese	Cabiralizumab	Tratamento do tumor tenosinovial de células gigantes, localizado e difuso.
Romanian	Cabiralizumab	Tratamentul tumorilor tenosinoviale cu celule gigant, localizate şi difuze
Slovak	Kabiralizumab	Liečba tendosynoviálneho obrovskobunkového nádora, lokalizovaného a difúzneho typu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Kabiralizumab	Zdravljenje tenosinuvialnega gigantocelularnega lokaliziranega ali difuznega tumorja
Spanish	Cabiralizumab	Tratamiento de tumores tenosinoviales de células gigantes, formas difusa y localizada
Swedish	Kabiralizumab	Behandling av tenosynovial jättecellstumör, lokal och diffuse typ
Norwegian	Kabiralizumab	Behandling av tenosynovial kjempecelletumor, lokalisert og diffus type
Icelandic	Kabiralízúmab	Meðferð við litaðri totu-hnökrahálahimnubólgu

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