EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised IgG1 monoclonal antibody against human KIR3DL2 for the treatment of cutaneous T-cell lymphoma

On 22 August 2014, orphan designation (EU/3/14/1322) was granted by the European Commission to Innate Pharma S.A., France, for humanised IgG1 monoclonal antibody against human KIR3DL2 for the treatment of cutaneous T-cell lymphoma.

What is cutaneous T-cell lymphoma?

Cutaneous T-cell lymphoma (CTCL) is a cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream.

In CTCL there is uncontrolled growth of the T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. The cancerous T cells appear in the skin, causing lesions (rashes, plaques and tumours) which can be itchy and painful.

CTCL usually happens in people aged between 40 and 60 years. In many cases, patients survive a long time with the disease; however, in some cases the disease can be serious and life threatening because it can develop into more aggressive forms of cancer and may have a large impact on quality of life, particularly because the skin lesions can cause disfigurement.

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

At the time of designation, cutaneous T-cell lymphoma affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer 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).

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What treatments are available?

At the time of designation, several products were authorised for the treatment of CTCL within the EU. Treatments for CTCL can be divided into topical (applied to the skin) and systemic (affecting the whole body):

- topical treatments included topical corticosteroids, the topical anticancer medicine carmustine, ultraviolet light and X-rays;
- systemic treatments included cytotoxic medicines (medicines that kill cells that are dividing, such as cancer cells) and interferon alfa (a medicine that helps the immune system to fight against the cancer cells).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with CTCL because early studies in experimental models indicate that the medicine is capable of killing the cancer cells. In addition, because of the medicine's mechanism of action which is different to currently authorised treatment it may be able to be used in combination with other medicines. 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 a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific target. The target for this medicine is a protein called KIR3DL2, which is present on the surface of many white blood cells but occurs in higher amounts in the cancerous T cells of patients with conditions such as CTCL. By attaching to KIR3DL2 on the cancer cells, the medicine is expected to activate certain components of the immune system to attack and kill the cancer cells.

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 cutaneous T-cell lymphoma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for cutaneous T-cell lymphoma 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 10 July 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:

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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	Humanised IgG1 monoclonal antibody against human KIR3DL2	Treatment of cutaneous T-cell lymphoma
Bulgarian	Хуманизирано IgG1 моноклонално антитяло срещу човешки KIR3DL2	Лечение на кожен Т-клетъчен лимфом
Croatian	Humanizirano IgG1 monoklonsko protutijelo protiv ljudskog KIR3DL2	Liječenje kožnog limfoma T-stanica
Czech	Humanizovaná IgG1 monoklonální protilátka proti lidskému KIR3DL2	Léčba kožního T-lymfomu
Danish	Humaniseret IgG1 monoklonalt antistof mod humant KIR3DL2	Behandling af kutant T-celle-lymfom
Dutch	Gehumaniseerd IgG1 monoclonaal antilichaam gericht tegen humaan KIR3DL2	Behandeling van cutaan T-cel-lymfoom
Estonian	Humaniseeritud IgG1 monoklonaalne antikeha inimese KIR3DL2 vastu	Kutaanse T-rakulise lümfoomi ravi
Finnish	Humanisoitu IgG1 monoklonaalinen vasta-aine ihmisen KIR3DL2 vastaan	Ihon T-solulymfooman hoito
French	Anticorps monoclonal humanisé de classe IgG1 dirigé contre la protéine KIR3DL2 humaine	Traitement des lymphomes cutanés à cellules T
German	Humanisierter monoklonaler IgG1 Antikörper gegen humanes KIR3DL2	Behandlung von kutanem T-Zell-Lymphomen
Greek	Ανθρωποποιημένο IgG1 μονοκλωνικό αντίσωμα έναντι της ανθρώπινης KIR3DL2	Θεραπεία του δερματικού λεμφώματος Τ-κυττάρων
Hungarian	Humán KIR3DL2-ellenes humanizált IgG1 monoklonális antitest	Kután T-sejtes lymphoma kezelése
Italian	Anticorpo Monoclonale umanizzato IgG1 contro la KIR3DL2 umana	Trattamento del linfoma cutaneo a cellule T
Latvian	Humanizēta IgG1 monoklonāla antiviela, kas vērsta pret cilvēka KIR3DL2	Ādas T-šūnu limfomas ārstēšana
Lithuanian	Žmogaus monokloninis antikūnas IgG1 prieš žmogaus KIR3DL2	Odos T ląstelių limfomos gydymas
Maltese	Antikorp monoklonali IgG1 umanizzat kontra KIR3DL2 uman	Kura tal-limfoma taċ-ċelluli tat-tip T tal-ġilda
Polish	Humanizowane przeciwciało monoklonalne IgG1 przeciw ludzkiemu receptorowi KIR3DL2	Leczenie chłoniaka skórnego T-komórkowego
Portuguese	Anticorpo monoclonal humanizado de tipo IgG1 anti- KIR3DL2 humana	Tratamento do linfoma cutâneo de células T
Romanian	Anticorp monoclonal umanizat de clasă IgG1 anti gena KIR3DL2 umană	Tratamentul limfomului cutanat cu celule T

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná monoklonálna protilátka IgG1 proti ľudskému KIR3DL2	Liečba kutánneho T-bunkového lymfómu
Slovenian	Humanizirano IgG1 monoklonsko protitelo proti humanemu KIR3DL2	Zdravljenje kožnega T-celičnega limfoma
Spanish	Anticuerpo monoclonal humanizado de tipo IgG1 anti-KIR3DL2 humana	Tratamiento del linfoma cutáneo de células T
Swedish	Humaniserad IgG1 monoklonal antikropp riktad mot human KIR3DL2	Behandling av kutant T-cellslymfom
Norwegian	Humanisert IgG1 monoklonalt antistoff mot human KIR3DL2	Behandling av kutant T-cellelymfom
Icelandic	Mannaaðlagað IgG1 einstofna mótefni gegn manna KIR3DL2	Meðferð T-eitilfrumukrabbameins í húð