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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mogamulizumab for the treatment of cutaneous T-cell lymphoma

On 14 October 2016, orphan designation (EU/3/16/1756) was granted by the European Commission to Kyowa Kirin Limited, United Kingdom, for mogamulizumab for the treatment of cutaneous T-cell lymphoma.

What is cutaneous T-cell lymphoma?

Cutaneous T-cell lymphoma (CTCL) is a cancer of the T lymphocytes (T cells), a type of white blood cell. 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, CTCL affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 134,000 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 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 cancer medicine carmustine and ultraviolet light;

[*]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).

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- 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 mogamulizumab might be of significant benefit for patients with CTCL because early studies show that patients whose disease had come back after previous treatment responded to treatment with this medicine. 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to receptors called C-C chemokine receptor 4 (CCR4), which are found in high amounts on the surface of CTCL cells. By attaching to CCR4, this medicine is expected to activate cells of the immune system (the body's natural defences), which then kill the cancer cells.

What is the stage of development of this medicine?

The effects of mogamulizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with mogamulizumab in patients with CTCL were ongoing.

At the time of submission, mogamulizumab was authorised in Japan for CTCL.

At the time of submission, mogamulizumab was not authorised anywhere in the EU for CTCL. Orphan designation of mogamulizumab had been granted in the US and Japan for CTCL.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 2016 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:

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.

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

Language	Active ingredient	Indication
English	Mogamulizumab	Treatment of cutaneous T-cell lymphoma
Bulgarian	Могамулизумаб	Лечение на кожен Т-клетъчен лимфом
Croatian	Mogamulizumab	Liječenje kožnog limfoma T-stanica
Czech	Mogamulizumab	Léčba kožního T-lymfomu
Danish	Mogamulizumab	Behandling af kutant T-celle-lymfom
Dutch	Mogamulizumab	Behandeling van cutaan T-cel-lymfoom
Estonian	Mogamulisumab	Kutaanse T-rakulise lümfoomi ravi
Finnish	Mogamulitsumabi	Ihon T-solulymfooman hoito
French	Mogamulizumab	Traitement des lymphomes cutanés à cellules T
German	Mogamulizumab	Behandlung von kutanem T-Zell- Lymphom
Greek	Μογκαμουλιζουμάμπη	Θεραπεία του δερματικού λεμφώματος Τ-κυττάρων
Hungarian	Mogamulizumab	Kután T-sejtes lymphoma kezelése
Italian	Mogamulizumab	Trattamento del linfoma cutaneo a cellule T
Latvian	Mogamulizumabs	Ādas T-šūnu limfomas ārstēšana
Lithuanian	Mogamulizumabas	Odos T ląstelių limfomos gydymas
Maltese	Mogamulizumab	Kura tal-limfoma taċ-ċelluli tat-tip T tal-ġilda
Polish	Mogamulizumab	Leczenie chłoniaka skórnego T-komórkowego
Portuguese	Mogamulizumab	Tratamento do linfoma cutâneo de células T
Romanian	Mogamulizumab	Tratamentul limfomului cutanat cu celule T
Slovak	Mogamulizumab	Liečba kutánneho T-bunkového lymfómu
Slovenian	Mogamulizumab	Zdravljenje kožnega T-celičnega limfoma
Spanish	Mogamulizumab	Tratamiento del linfoma cutáneo de células T
Swedish	Mogamulizumab	Behandling av kutant T-cellslymfom
Norwegian	Mogamulizumab	Behandling av kutant T-cellelymfom
Icelandic	Mógamúlízúmab	Meðferð T-eitilfrumukrabbameins í húð

[1] At the time of designation