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Public summary of opinion on orphan designation

Sintilimab for the treatment of peripheral T-cell lymphoma

On 28 February 2020, orphan designation EU/3/20/2257 was granted by the European Commission to Parexel International GmbH, Germany, for sintilimab for the treatment of peripheral T-cell lymphoma.

What is peripheral T-cell lymphoma?

Peripheral T-cell lymphoma 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 peripheral T-cell lymphoma there is uncontrolled growth of T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. Peripheral T-cell lymphomas include types that mainly occur in the lymph nodes (primary nodal) and types that occur mainly outside the lymph nodes (primary extranodal).

The symptoms of the disease vary according to the type of lymphoma, but the first sign may be a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. The lymphoma may also affect other organs in the body such as the bone marrow, liver and the skin.

Peripheral T-cell lymphoma is a long-term debilitating and life-threatening condition because in most cases the disease does not respond well to therapy, usually comes back within one year and is associated with early death.

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

At the time of designation, peripheral T-cell lymphoma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 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, the main treatment for peripheral T-cell lymphoma was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation).

*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 519,200,000 (Eurostat 2020).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with peripheral T-cell lymphoma because early studies showed that the medicine can be of benefit in patients whose disease had not improved with previous treatments. 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?

Peripheral T-cell lymphoma cells produce proteins (PD-L1) that switch off cancer-killing cells of the immune system called T cells by attaching to a target on the T-cells called PD-1. This prevents the T cells from attacking the cancer. Sintilimab is a monoclonal antibody, a type of protein, designed to attach to PD-1 without switching the T cells off itself. This blocks the cancer cell proteins from attaching and switching the cells off, thereby increasing the ability of the immune system to kill cancer cells.

What is the stage of development of this medicine?

The effects of sintilimab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sintilimab in patients with peripheral T-cell lymphoma were ongoing.

At the time of submission, sintilimab was not authorised anywhere in the EU for the treatment of peripheral T-cell lymphoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 22 January 2020, 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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	Sintilimab	Treatment of peripheral T-cell lymphoma
Bulgarian	Синтилимаб	Лечение на периферен Т-клетъчен лимфом
Croatian	Sintilimab	Liječenje perifernog limfoma T-stanica
Czech	Sintilimab	Léčba periferních T-lymfomů
Danish	Sintilimab	Behandling af perifer T-celle lymfom
Dutch	Sintilimab	Behandeling van perifere T-cel lymfomen
Estonian	Sintilimab	Perifeerse T-rakulise lümfoomi ravi
Finnish	Sintilimabi	Perifeerisen T-solulymfooman hoito
French	Sintilimab	Traitement du lymphome périphérique à cellules T
German	Sintilimab	Behandlung des peripheren T-Zell-Lymphoms
Greek	Σιντιλιμάμπη	Θεραπεία του λεμφώματος περιφερικών κυττάρων Τ
Hungarian	Szintilimab	Perifériás T-sejtes lymphoma kezelése
Italian	Sintilimab	Trattamento del linfoma periferico a cellule T
Latvian	Sintilimabs	Perifēriskās T-šūnu limfomas ārstēšana
Lithuanian	Sintilimabas	Periferinės T-ląstelių limfomos gydymas
Maltese	Sintilimab	Kura tal-limfoma taċ-ċelloli T periferali
Polish	Sintilimab	Leczenie obwodowego chłoniaka T-komórkowego
Portuguese	Sintilimab	Tratamento do linfoma periférico das células T
Romanian	Sintilimab	Tratamentul limfomului periferic cu celule T
Slovak	Sintilimab	Liečba periférneho T-bunkového lymfómu
Slovenian	Sintilimab	Zdravljenje perifernega limfoma celic T
Spanish	Sintilimab	Tratamiento del linfoma periférico de células T
Swedish	Sintilimab	Behandling av perifert T-cellslymfom
Norwegian	Sintilimab	Behandling av perifert T-celle-lymfom
Icelandic	Sintilímab	Meðferð við útlægu T-eitilfrumukrabbameini

[1] At the time of designation