EMA/COMP/110465/2007 Rev.2
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Siplizumab for the treatment of T-cell and NK-cell neoplasms

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| First publication | 14 May 2009 |
| Rev.1: sponsor's name change | 19 August 2009 |
| Rev.2: withdrawal from the Community Register | 4 April 2013 |

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2013 on request of the Sponsor.***

On 29 June 2006, orphan designation (EU/3/06/383) was granted by the European Commission to MedImmune Oncology, Inc., The Netherlands, for siplizumab for the treatment of T-cell and NK-cell neoplasms.

MedImmune Oncology, Inc. changed its name to MedImmune, LLC. in July 2009.

### What are T-cell and NK-cell neoplasms?

T-cell and NK-cell neoplasms is a group of cancers of white blood cells known as T-lymphocytes and natural killer-cells (NK-cells). Both these types of cells belong to the immune system (body's natural defence against pathogens and disease) and, under normal circumstances, they act against infections or tumours. NK-cells fight tumours and cells infected by viruses by releasing small granules of proteins that cause the target cells to die by apoptosis (programmed cell death) or by necrosis (cell death caused by a process other than apoptosis).

T-cell and NK-cell neoplasms belong to a group of lymphomas called non-Hodgkin's lymphomas. There are more than 20 different types of non-Hodgkin's lymphomas. Because of their similarities, such as the molecules they express on their surfaces and how the tumour cells function, T-cell and NK-cell neoplasms are grouped together. Patients with T-cell and NK-cell neoplasms often present with enlargement of lymph nodes and liver, although the symptoms vary depending on the specific subtype

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
**Telephone** +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
**E-mail** info@ema.europa.eu **Website** www.ema.europa.eu

of the disease. Weight loss and skin involvement may also be seen. The condition is chronically debilitating and life-threatening.

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

At the time of designation, T-cell and NK-cell neoplasm affected less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 141,000 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 medicinal products were authorised in the Community for treatment of "T-cell and NK-cell neoplasms" including prednisolone, bleomycin, chlorambucil, carmustine, doxorubicin, mitoxantrone, metotrexate, vinblastin and vincristine. Siplizumab might be of potential significant benefit for the treatment of T-cell and NK-cell neoplasms because it could improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

## How is this medicine expected to work?

Most of the cells that multiply in T-cell and NK-cell neoplasms have a membrane receptor called CD2. The product is a human immunoglobulin (protein used by the immune system to identify and neutralise foreign objects) that has the capacity to bind CD2. By binding to the CD2 receptor on the surface of the lymphoma cells, the product activates a cascade of cytotoxic events of the immune system, ultimately leading to the destruction of the lymphoma cells.

## What is the stage of development of this medicine?

The effects of siplizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with T-cell and NK-cell neoplasms were ongoing.

Siplizumab was not authorised anywhere worldwide for T-cell and NK-cell neoplasms or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 May 2006 recommending the granting of this designation.

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*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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:

MedImmune, LLC
Lagelandseweg 78
6545 CG Nijmegen
The Netherlands
Telephone: +31 24 3717 310
Telefax: +31 24 3731 519
E-mail: info@medimmune.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 | Siplizumab | Treatment of T-cell and NK-cell neoplasms |
| Czech | Siplizumab | Léčba malignit T – a NK- lymfocytů. |
| Danish | Siplizumab | Behandling af T-celle-og NK-celle neoplasmer |
| Dutch | Siplizumab | Behandeling van T-cel en NK-cel neoplasme |
| Estonian | Siplizumab | T-rakulise ja NK-rakulise kasvajate ravi. |
| Finnish | Siplizumabi | T-solu ja NK-soluneoplasmojenn hoito |
| French | Siplizumab | Traitement des néoplasies à cellules T et NK |
| German | Siplizumab | Behandlung von T-Zell und NK-ZellNeoplasmen |
| Greek | Σιπλιζουμάβη | Θεραπεία νεοπλασμάτων από Τ και ΝΚ κύτταρα |
| Hungarian | Siplizumab | T-sejtes és NK-sejtes neoplasma kezelése |
| Italian | Siplizumab | Trattamento delle neoplasie delle cellule T e NK |
| Latvian | Siplizumabs | T-šūnu un NK-šūnu neoplazmu ārstēšana |
| Lithuanian | Siplizumabas | T ir NK-ląstelių limfoproliferacinių ligų gydymas |
| Polish | Siplizumab | Leczenie nowotworów wywodzących się z komórek T i NK |
| Portuguese | Siplizumab | Tratamento de neoplasmas das células T e NK |
| Slovak | Siplizumab | Liečba T/NK bunkového nádoru |
| Slovenian | Siplizumab | Zdravljenje T- in NK- celičnih neoplazem |
| Spanish | Siplizumab | Tratamiento de las neoplasias de células T y células NK |
| Swedish | Siplizumab | Behandlig av T-cell och naturliga mördarcell neoplasmer |
| Norwegian | Siplizumab | Behandling av T-celle og NK-celle neoplasmer |
| Icelandic | Siplízúmab | Meðferð við T-frumu- og NK frumu eitilæxlum |

[1] At the time of designation