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Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Resiquimod for the treatment of cutaneous T-cell lymphoma

On 28 April 2016, orphan designation (EU/3/16/1653) was granted by the European Commission to Galderma R&D, France, for resiquimod 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, CTCL affected less than 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 128,000 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):

[*]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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- topical treatments included topical corticosteroids, the topical cancer 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 resiquimod might be of significant benefit for patients with CTCL because early clinical studies indicate that the medicine may improve the condition in patients where alternative treatments have failed. 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?

Resiquimod is an immune-response modifier, which means that it uses the immune system (the body's natural defences) to bring about its effect. When resiquimod is applied to the skin, it is expected to attach to proteins known as toll-like receptor 7 and toll-like receptor 8 on the surface of cells of the immune system, mainly dendritic cells in the skin. This triggers the production of cytokines (a type of protein that activates the immune system) and stimulates immune cells to attack and kill cancer cells in the skin.

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

The effects of resiquimod have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with resiquimod in patients with CTCL had finished and further studies were planned.

At the time of submission, resiquimod was not authorised anywhere in the EU for CTLC 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 23 March 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 | Resiquimod | Treatment of cutaneous T-cell lymphoma |
| Bulgarian | Резиквимод | Лечение на кожен Т-клетъчен лимфом |
| Croatian | Rezikvimod | Liječenje kožnog limfoma T-stanica |
| Czech | Resiquimod | Léčba kožního T-lymfomu |
| Danish | Resiquimod | Behandling af kutant T-celle-lymfom |
| Dutch | Resiquimod | Behandeling van cutaan T-cel-lymfoom |
| Estonian | Resiquimood | Kutaanse T-rakulise lümfoomi ravi |
| Finnish | Resikimodi | Ihon T-solulymfooman hoito |
| French | Resiquimod | Traitement des lymphomes cutanés à cellules T |
| German | Resiquimod | Behandlung von kutanem T-Zell- Lymphom |
| Greek | Ρεσικιμόδη | Θεραπεία του δερματικού λεμφώματος Τ-κυττάρων |
| Hungarian | Resiquimod | Kután T-sejtes lymphoma kezelése |
| Italian | Resiquimod | Trattamento del linfoma cutaneo a cellule T |
| Latvian | Rezikvimods | Ādas T-šūnu limfomas ārstēšana |
| Lithuanian | Rezikvimodas | Odos T ląstelių limfomos gydymas |
| Maltese | Resiquimod | Kura tal-limfoma taċ-ċelluli tat-tip T tal-ġilda |
| Polish | Rezikwimod | Leczenie chłoniaka skórnego T-komórkowego |
| Portuguese | Resiquimod | Tratamento do linfoma cutâneo de células T |
| Romanian | Resichimod | Tratamentul limfomului cutanat cu celule T |
| Slovak | Resiquimod | Liečba kutánneho T-bunkového lymfómu |
| Slovenian | Resikvimod | Zdravljenje kožnega T-celičnega limfoma |
| Spanish | Resiquimod | Tratamiento del linfoma cutáneo de células T |
| Swedish | Resiquimod | Behandling av kutant T-cellslymfom |
| Norwegian | Resiquimod | Behandling av kutant T-cellelymfom |
| Icelandic | Resikvímód | Meðferð T-eitilfrumukrabbameins í húð |

[1] At the time of designation