12 August 2015
EMA/COMP/432824/2015
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Synthetic hypericin for the treatment of cutaneous T-cell lymphoma

On 28 July 2015, orphan designation (EU/3/15/1515) was granted by the European Commission to Kinesys Consulting Ltd, United Kingdom, for synthetic hypericin 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 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 103,000 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 512,900,000 (Eurostat 2015).

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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 synthetic hypericin might be of significant benefit for patients with CTCL because early clinical studies indicate that the medicine may be used in the early stages of the disease. 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?

Synthetic hypericin is a photosensitising agent (a substance that changes when exposed to light). It will be available as an ointment to be applied onto the skin lesions of patients with CTCL. With light, hypericin is activated and reacts with oxygen in skin cells to create toxic molecules containing oxygen called 'reactive oxygen species', which are expected to kill the cancer cells.

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

The effects of synthetic hypericin have been evaluated in experimental models.

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

At the time of submission, synthetic hypericin was not authorised anywhere in the EU for CTCL. Orphan designation of hypericin had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 June 2015 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 | Synthetic hypericin | Treatment of cutaneous T-cell lymphoma |
| Bulgarian | Синтетичен хиперицин | Лечение на кожен Т-клетъчен лимфом |
| Croatian | Sintetski hipericin | Liječenje kožnog limfoma T-stanica |
| Czech | Syntetický hypericin | Léčba kožního T-lymfomu |
| Danish | Syntetisk hypericin | Behandling af kutant T-celle-lymfom |
| Dutch | Synthetische hypericine | Behandeling van cutaan T-cel-lymfoom |
| Estonian | Sünteetiline hüperitsiin | Kutaanse T-rakulise lümfoomi ravi |
| Finnish | Synteettinen hyperisiini | Ihon T-solulymfooman hoito |
| French | Hypéricine synthétique | Traitement des lymphomes cutanés à cellules T |
| German | Synthetisches Hypericin | Behandlung von kutanem T-Zell- Lymphom |
| Greek | Συνθετική υπερισίνη | Θεραπεία του δερματικού λεμφώματος Τ-κυττάρων |
| Hungarian | Szintetikus hypericin | Kután T-sejtes lymphoma kezelése |
| Italian | Ipericina sintetica | Trattamento del linfoma cutaneo a cellule T |
| Latvian | Sintētisks hipericīns | Ādas T-šūnu limfomas ārstēšana |
| Lithuanian | Sintetinis hipericinas | Odos T ląstelių limfomos gydymas |
| Maltese | Hypericin sintetiku | Kura tal-limfoma taċ-ċelluli tat-tip T tal-ġilda |
| Polish | Syntetyczna hiperycyna | Leczenie chłoniaka skórnego T-komórkowego |
| Portuguese | Hipericina sintética | Tratamento do linfoma cutâneo de células T |
| Romanian | Hipericină sintetică | Tratamentul limfomului cutanat cu celule T |
| Slovak | Syntetický hypericín | Liečba kutánneho T-bunkového lymfómu |
| Slovenian | Sintetični hipericin | Zdravljenje kožnega T-celičnega limfoma |
| Spanish | Hipericina sintética | Tratamiento del linfoma cutáneo de células T |
| Swedish | Syntetisk hypericin | Behandling av kutant T-cellslymfom |
| Norwegian | Syntetisk hypericin | Behandling av kutant T-cellelymfom |
| Icelandic | Tilbúið hýpericín | Meðferð T-eitilfrumukrabbameins í húð |

[1] At the time of designation