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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Naloxone hydrochloride dihydrate for the treatment of cutaneous T-cell lymphoma

On 8 November 2012, orphan designation (EU/3/12/1057) was granted by the European Commission to Winston Laboratories Ltd, United Kingdom, for naloxone hydrochloride dihydrate 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, the disease is long lasting, however, it can be a serious and life-threatening disease 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 2.6 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 132,000 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).

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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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 include topical corticosteroids, the topical anticancer medicine carmustine, ultraviolet light and X-rays;
- systemic treatments include 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 naloxone hydrochloride dihydrate might be of significant benefit for patients with CTCL because early studies show that, when applied to the skin, it may improve patient care by reducing the persistent itching which is a symptom 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?

Naloxone hydrochloride dihydrate (a form of naloxone) is an opioid antagonist, a substance that counteracts the effects of opioids (medicines widely used as painkillers, such as morphine). Opioids work by attaching to certain receptors on the surfaces of cells in the body. Naloxone blocks these receptors. As these receptors also appear to be involved in producing the sensation of itching, naloxone is expected to reduce the persistent itching in CTCL. The medicine is expected to be applied directly to the skin.

What is the stage of development of this medicine?

The sponsor has provided data in experimental models from the published literature to support this application for orphan designation.

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

At the time of submission, naloxone hydrochloride dihydrate was not authorised anywhere in the EU for CTCL. Orphan designation of naloxone hydrochloride dihydrate had been granted in the United States of America for the topical treatment of pruritus associated with mycosis fungoides.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2012 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:

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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 substance	Indication
English	Naloxone hydrochloride dihydrate	Treatment of cutaneous T-cell lymphoma
Bulgarian	Налоксон хидрохлорид дихидрат	Лечение на кожен Т-клетъчен лимфом
Czech	Naloxon-hydrochlorid dihydrát	Léčba kožního T-lymfomu
Danish	Naloxonhydrokloriddihydrat	Behandling af kutant T-celle-lymfom
Dutch	Naloxonehydrochloridedihydraat	Behandeling van cutaan T-cel-lymfoom
Estonian	Naloksoonhüdrokloriiddihüdraat	Kutaanse T-rakulise lümfoomi ravi
Finnish	Naloksonihydrokloridididihydraatti	Ihon T-solulymfooman hoito
French	Chlorhydrate de naloxone dihydraté	Traitement des lymphomes cutanés à cellules T
German	Naloxonhydrochlorid-Dihydrat	Behandlung von kutanem T-Zell- Lymphomen
Greek	Υδροχλωρική διϋδρική ναλοξόνη	Θεραπεία του δερματικού λεμφώματος Τ-κυττάρων
Hungarian	Naloxon hidroklorid dihidrát	Kután T-sejtes lymphoma kezelése
Italian	Naloxone cloridrato diidrato	Trattamento del linfoma cutaneo a cellule T
Latvian	Naloksona hidrohlorīda dihidrāts	Ādas T-šūnu limfomas ārstēšana
Lithuanian	Naloksono hidrochlorido dihidratas	Odos T ląstelių limfomos gydymas
Maltese	Naloxone hydrochloride dihydrate	Kura tal-limfoma taċ-ċelluli tat-tip T tal-ġilda
Polish	Chlorowodorek naloksonu dwuwodny	Leczenie chłoniaka skórnego T-komórkowego
Portuguese	Cloridrato di-hidratado de naloxona	Tratamento do linfoma cutâneo de células T
Romanian	Naloxon hidroclorid dihidrat	Tratamentul limfomului cutanat cu celule T
Slovak	Naloxón hydrochlorid dihydrát	Liečba kutánneho T-bunkového lymfómu
Slovenian	Naloksonijev hidrokloriddihidrat	Zdravljenje kožnega T-celičnega limfoma
Spanish	Clorhidrato dihidrato de naloxona	Tratamiento del linfoma cutáneo de células T
Swedish	Naloxonhydrokloriddihydrat	Behandling av kutant T-cellslymfom
Norwegian	Naloksonhydrokloriddihydrat	Behandling av kutant T-cellelymfom
Icelandic	Naloxón-hýdróklóríð-díhýdrat	Meðferð T-eitilfrumukrabbameins í húð

[1] At the time of designation