



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Suberoylanilide hydroxamic acid for the treatment of cutaneous T-cell lymphoma

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2013 on request of the Sponsor.

On 21 June 2004, orphan designation (EU/3/04/205) was granted by the European Commission to Stringer Consultancy Services Ltd, United Kingdom, for suberoylanilide hydroxamic acid (SAHA) for the treatment of cutaneous T-cell lymphoma.

The sponsorship was transferred to Merck Sharp & Dohme Limited, United Kingdom, in September 2004.

What is cutaneous T-cell lymphoma?

Cutaneous T cell lymphoma is a type of cancer of the lymphatic system localized in the skin. It belongs to a group of lymphomas called non-Hodgkin's lymphomas (there are more than 20 different types of non-Hodgkin's lymphoma). The lymphatic system is part of the body's immune system and helps fighting infections. It is a complex system made up of organs such as the bone marrow, the thymus (a gland behind the breast bone), the spleen (an organ in the abdomen, near the stomach), and the lymph nodes (or lymph glands, located throughout the body), which are connected by a network of tiny lymphatic vessels. There are two main types of cells, which make up the lymphatic tissue. These cells are called lymphocytes and belong to the group of white blood cells. The two types are called B lymphocytes (B cells) and T lymphocytes (T cells). Most lymphocytes start growing in the bone marrow. The T cells go from the bone marrow to the thymus and mature there. Cutaneous T-cell lymphoma is a cancer of the T-lymphocytes and most often occurs in people aged between 40 and 60. Unlike other forms of non-Hodgkin's lymphomas, cutaneous T-cell lymphoma mainly affects the skin. It is caused by the uncontrolled growth of T-cells within the skin. Cutaneous T-cell lymphoma is a serious and life-threatening condition.



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

At the time of designation, cutaneous T-cell lymphoma affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 46,000 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?

Treatments for cutaneous T-cell lymphoma can be local (limited to the part of the body concerned by the disease) and systemic (distributed throughout the patient's body). Local treatments include medicines applied to the skin, therapies using light of a particular wavelength (ultraviolet light) and x-rays. Systemic treatments include medicines such as glucocorticosteroids (a group of medicines derived from cortisone, also used as agents against itching), cytotoxic agents (medicines that kill cells), immunotherapy (using drugs that stimulate the body's own immune system to kill the cancer cells) and photopheresis (white blood cells are modified by exposure to ultraviolet light). Several products were authorised for the treatment of cutaneous T-cell lymphoma within the Community at the time of submission of the application for orphan drug designation.

Suberoylanilide hydroxamic acid (SAHA) might be of potential significant benefit for the treatment of cutaneous T-cell lymphoma. This assumption remains to be proven. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Enzymes are proteins produced by the human body that speed up the conversion of certain substances into other substances. SAHA appears to block the activity of an enzyme, called histone deacetylase, which is necessary for cell growth and multiplication. As these enzymes are no more available to help the formation of new genetic material, this might lead to the arrest of cell growth. Since cutaneous T-cell lymphoma is caused by the uncontrolled growth of the T-cells, SAHA might help in slowing down or stopping this uncontrolled cell growth.

What is the stage of development of this medicine?

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

Suberoylanilide hydroxamic acid (SAHA) was not marketed anywhere worldwide for cutaneous T cell lymphoma 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 14 May 2004 recommending the granting of this designation.

*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 464,200,000 (Eurostat 2004).

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:

Merck Sharp & Dohme Limited
Hertford Road
Hoddesdon
Hertfordshire EN11 9BU
United Kingdom
Telephone: +44 1992 46 72 72
Telefax: +44 1992 47 91 91
E-mail: medicalinformationuk@merck.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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Suberoylanilide hydroxamic acid	Treatment of cutaneous T-cell lymphoma
Czech	Suberoylanilid kyseliny hydroxamové	Léčba kožního T- lymfomu
Danish	Suberoylanilid-hydroxamsyre	Behandling af kutant T-celle-lymfom
Dutch	Suberoylanilide hydroxaamzuur	Behandeling van cutaan T-cel-lymfoom
Estonian	Suberoülaniidid-hüdroksüamiidhape	Kutaanse T-rakulise lümfoomi ravi
Finnish	Suberoilaniidid-hydroksaamihappo	Ihon T-solulymfooman hoito
French	Acide Hydroxamique Suberoylanilide	Traitement des lymphomes cutanés à cellules T
German	Suberoylanilid-Hydroxamsäure	Behandlung von kutanem T-Zell- Lymphom
Greek	Φελλονοϋλανιδινικό υδροξαμικό οξύ	Θεραπεία του δερματικού λεμφώματος T κυττάρων
Hungarian	Suberoylanilide hidroxámsav	T-sejtes cutan lymphoma kezelése
Italian	Acido idrossamico suberoylanilide	Trattamento del linfoma cutaneo a cellule T
Latvian	Suberoilaniīda hidroksamskābe	Ādas T-šūnu limfomas ārstēšana
Lithuanian	Suberoilaniido hidroksamo rūgštis	Odos T ląstelių limfomos gydymas
Maltese	Suberoylanilide hydroxamic acid	Treatment of cutaneous T-cell lymphoma
Polish	Suberojlanilid kwasu hydroksamowego	Leczenie chłoniaka skórniego T-komórkowego
Portuguese	Ácido hidroxâmico suberoilaniído	Tratamento do linfoma cutâneo de células T
Slovak	Suberoylový anilid kyseliny hydroxamovej	Liečba kutánneho T-bunkového lymfómu
Slovenian	Suberojlanilidna hidroksamična kislina	Zdravljenje kožnega T-celičnega limfoma
Spanish	Ácido hidroxámico suberoilaniído	Tratamiento del linfoma cutáneo de células T
Swedish	Suberoylanilidhydroxamsyra	Behandling av kutant T-cellslymfom
Norwegian	Suberoylanilidhydroxamsyre	Behandling av kutant T-cellelymfom
Icelandic	Suberóýlanilíð hýdroxamsýra	Meðferð T-eitilfrumukrabbameins í húð

¹ At the time of designation