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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

adenovirus-interferon gamma – coding DNA sequence 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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in September 2010 on request of the sponsor.

On 9 July 2003, orphan designation (EU/3/03/150) was granted by the European Commission to Transgene S.A., France, for adenovirus-interferon gamma – coding DNA sequence for the treatment of cutaneous T-cell lymphoma.

What is cutaneous T-cell lymphoma?

Cutaneous T-cell lymphoma is a type of cancer of the lymphatic system. It belongs to a group of lymphomas called non-Hodgkin's lymphoma (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 lymphoma, cutaneous T-cell lymphoma mainly affects the skin. The cancer develops in the skin and can cause infections and spoil the appearance. Cutaneous T-cell lymphoma is a serious and life-threatening condition.

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

At the time of designation, cutaneous T-cell lymphoma affected approximately 0.63 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 24,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Current treatment for cutaneous T-cell lymphoma can be divided into local treatment and systemic treatment (directed to the whole 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 called glucocorticosteroids (a group of medicines that are similar to cortisone also used as agents against itching), cytotoxic agents (medicines that kill cells), interferon-alfa (a compound that can help the immune system to fight against cancer cells) and photopheresis. With this last therapeutic method, white blood cells are exposed to ultraviolet light while they are flowing through a system outside the body. Adenovirus-interferon gamma – coding DNA sequence 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?

Interferon gamma is a substance that can help the immune system to fight cancer. Adenovirus-interferon gamma – coding DNA sequence is a medicinal product which uses a virus to carry the genetic material necessary for the production of interferon gamma near the tumour. A virus is a small organism that infects cell with genetic material. The type of virus (adenovirus) used in this medicinal product does not cause any disease in humans.

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.

Adenovirus-interferon gamma – coding DNA sequence 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 13 June 2003 recommending the granting of this designation.

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[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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	Adenovirus-Interferon gamma – coding DNA sequence	Treatment of Cutaneous T Cell Lymphoma
Danish	Adenovirus-interferon-gamma kodende DNA sekvens (Ad-IFNγ))	Behandling af kutant T-celle lymfom
Dutch	Adenovirus-Interferon gamma – coderende DNA sequentie	Behandeling van cutaan T cel lymfoom
Finnish	Adenovirus-interferoni gamma koodaava DNA sekvenssi	Ihon T-solulymfooman hoito
French	Adéno-interféron gamma (Ad-IFNγ) – séquence codante d'ADN	Traitement du lymphome T cutané
German	Adenovirus-Interferon gamma (Ad-IFNγ) –codierende DNA Sequenz	Behandlung von kutanem T-Zell-Lymphom
Greek	Αδενοϊός-που περιέχει την αλληλουχία DNA που κωδικοποιεί την ιντερφερόνη γ(Ad-IFNγ))	Θεραπεία δερματικού Τ- λεμφοκυτταρικού λεμφώματος
Italian	Sequenza di DNA codificante Adenovirus interferone gamma (Ad-IFNγ)	Trattamento del linfoma cutaneo a cellule T
Portuguese	Adenovírus contendo sequência de DNA que codifica para o interferão gama	Tratamento do linfoma cutâneo das células T
Spanish	Adenovirus que contiene la secuencia ADN que codifica para interferón gamma	Tratamiento del linfoma cutáneo de células T
Swedish	Adenovirus-interferon-gamma-kodande DNA sekvens	Behandling av kutant T-cells lymfom

[1] At the time of designation