



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN) for the treatment of systemic sclerosis

On 28 October 2005, orphan designation (EU/3/05/326) was granted by the European Commission to Digna Biotech S.L., Spain, for peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN) for the treatment of systemic sclerosis.

What is systemic sclerosis?

Systemic sclerosis involves the abnormal growth of connective tissue, which supports the skin and internal organs. The abnormal growth is due to too much production of a protein called collagen. The disease not only includes the skin, but also involves the tissues around the blood vessels and major organs. Systemic sclerosis is typically broken down into *diffuse* and *limited* disease. In diffuse form, the skin becomes thicker over much of the body, such as the upper arms, upper legs, chest, and stomach. Inside the body, the disease can damage key organs such as the heart, lungs, and kidneys. The limited form typically affects the skin only in certain areas: the fingers, hands, face, lower arms, and legs. Many people with limited disease have Raynaud's phenomenon (a condition in which the small blood vessels of the hands and/or feet contract in response to cold or anxiety: they turn white and cold, then blue) for years before the skin starts to become thicker. People with limited disease are more prone to develop hypertension of the heart-lung circulation (pulmonary hypertension) than people with diffuse disease. Due to the thicker skin and the damage to key internal organs, the disease is considered seriously debilitating and potentially life-threatening.

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

At the time of designation, systemic sclerosis affected not more than 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 92,000 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 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 based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004).



What treatments are available?

There is no treatment that stops the build-up of collagen. Available treatment is aimed at relieving symptoms and limiting damage. Several products with anti-inflammatory activity (products that limit the reaction of tissues against damage) were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation. Peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN) might be of potential significant benefit for the treatment of systemic sclerosis in particular because it may act differently from other medicinal products. This benefit will have to be confirmed at the time of marketing authorisation and will be necessary to maintain the orphan status.

How is this medicine expected to work?

Transforming growth factor beta-1 (TGF- β 1) is a substance secreted by cells in the human body that plays a role in the formation of the abnormal growth of connective tissue (fibrosis). Peptide 144 TGF- β 1-inhibitor (TSLDASIIWAMMQN) is expected to stop the activation of this substance and thereby it might stop or reduce the production of connective tissue (fibrosis) and thus reduce the thickness of the skin.

What is the stage of development of this medicine?

The effects of peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN) were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with systemic sclerosis were initiated.

The medicinal product was not authorised anywhere worldwide for systemic sclerosis 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 9 September 2005 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 European Union) 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

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Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Peptide 144 TGF- β 1 inhibitor (TSLDASIIWAMMQN)	Treatment of systemic sclerosis
Czech	Inhibitor TGF- β 1 peptidů 144 (TSLDASIIWAMMQN)	Léčba systémové sklerodermie
Danish	Peptidhæmmer 144 TGF- β 1 (TSLDASIIWAMMQN)	Behandling af systemisk sklerose
Dutch	Peptideremmer 144 TGF- β 1 (TSLDASIIWAMMQN)	Behandeling van systemische sclerose
Estonian	Peptiid 144 TGF- β 1 inhibiitor (TSLDASIIWAMMQN)	Süsteemse sklerodermia ravi
Finnish	Peptidi 144 TGF- β 1 inhibitor (TSLDASIIWAMMQN)	Systeemisen skleroosin hoito
French	Peptide 144 TGF- β 1 inhibiteur (TSLDASIIWAMMQN)	Traitement de la sclérose systémique
German	Peptid 144 TGF- β 1 Inhibitor (TSLDASIIWAMMQN)	Behandlung systemischer Sklerose
Greek	Αναστολέας πεπτιδίων 144 TGF- β 1 (TSLDASIIWAMMQN)	Θεραπεία της συστηματικής σκλήρυνσης
Hungarian	144 TGF- β 1 peptid inhibitor (TSLDASIIWAMMQN)	Szisztémás scleroderma kezelése
Italian	Peptide 144 TGF- β 1 inibitori (TSLDASIIWAMMQN)	Trattamento della sclerosi sistemica
Latvian	Peptīdu 144 TGF- β 1 inhibitors (TSLDASIIWAMMQN)	Sistēmiskas sklerozes ārstēšana
Lithuanian	Peptidinis 144 inhibitorius TGF- β 1 (TSLDASIIWAMMQN)	Sisteminės odos sklerozės gydymas
Polish	Peptyd 144 inhibitor TGF- β 1 (TSLDASIIWAMMQN)	Leczenie twardziny narządowej
Portuguese	Péptido 144 inibidor do TGF- β 1 (TSLDASIIWAMMQN)	Tratamento da esclerose sistémica
Slovak	Peptid 144 inhibítor TGF- β 1 (TSLDASIIWAMMQN)	Liečba systémovej sklerózy
Slovenian	Inhibitor TGF- β 1 peptidov 144 (TSLDASIIWAMMQN)	Zdravljenje sistemske skleroze
Spanish	Péptido 144 inhibidor del TGF- β 1 (TSLDASIIWAMMQN)	Tratamiento de la esclerodermia sistémica
Swedish	Peptidhämmare 144 TGF- β 1 (TSLDASIIWAMMQN)	Behandling av systemisk skleroderma

¹ At the time of designation

Language	Active Ingredient	Indication
Norwegian	Peptid 144 TGF- β 1 inhibitor (TSLDASIIWAMMQN)	Behandling av systemisk sklerose
Icelandic	Peptíð144 TGF- β 1 hemill(TSLDASIIWAMMQN)	Meðferð við dreifðum herslum