



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 localised scleroderma

On 28 October 2005, orphan designation (EU/3/05/329) was granted by the European Commission to Digna Biotech S.L., Spain, for peptide 144 TGF-beta1-inhibitor (TSLDASIIWAMMQN) treatment of localised scleroderma.

What is localised scleroderma?

Localised scleroderma involves the abnormal growth of connective tissue, which supports the skin leading to a chronic, localised hardening and thickening of the skin. Depending on the way they grow, lesions may be categorised as "morphea" (shape of a drop) or "linear" (forming lines). It is more common in women than in men. Due to the damage caused by thickening of the skin, which can cause severe restrictions of movements, the disease is considered seriously debilitating.

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

At the time of designation, localised scleroderma affected not more than 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 138,000 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).

What treatments are available?

One medicinal product with anti-fibrotic activity (limiting new formation of a basic substance of connective tissue) was authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation. Other disease-modifying products were generally used for the condition 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 localised scleroderma 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.

*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).



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 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 localised scleroderma were initiated.

The medicinal product was not authorised anywhere worldwide for localised scleroderma 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 the localised scleroderma
Czech	Inhibitor TGF- β 1 peptidů 144 (TSLDASIIWAMMQN)	Léčba lokalizované sklerodermie
Danish	Peptidhæmmer 144 TGF- β 1 (TSLDASIIWAMMQN)	Behandling af lokaliseret sklerodermi
Dutch	Peptideremmer 144 TGF- β 1 (TSLDASIIWAMMQN)	Behandeling van locale sclerodermie
Estonian	Peptiid 144 TGF- β 1 inhibiitor (TSLDASIIWAMMQN)	Lokaalse sklerodermia ravi
Finnish	Peptidi 144 TGF- β 1 inhibitor (TSLDASIIWAMMQN)	Paikallisen skleroderman hoito
French	Peptide 144 TGF- β 1 inhibiteur (TSLDASIIWAMMQN)	Traitement de la sclérodermie dermatologique localisée
German	Peptid 144 TGF- β 1 Inhibitor (TSLDASIIWAMMQN)	Behandlung lokaler Sklerodermie
Greek	Αναστολέας πεπτιδίου TGF- β 1 144 (TSLDASIIWAMMQN)	Θεραπεία του εντοπισμένου σκληροδέρματος
Hungarian	144 TGF- β 1 peptid inhibitor (TSLDASIIWAMMQN)	Lokalizált scleroderma kezelése
Italian	Peptide 144 TGF- β 1 inibitori (TSLDASIIWAMMQN)	Trattamento della sclerodermia localizzata
Latvian	Peptīda 144 TGF- β 1 inhibitors (TSLDASIIWAMMQN)	Lokalizētas sklerodermijas ārstēšana
Lithuanian	Peptidinis 144 inhibitorius TGF- β 1 (TSLDASIIWAMMQN)	Lokaliuos sklerodermos gydymas
Polish	Peptyd 144 inhibitor TGF- β 1 (TSLDASIIWAMMQN)	Leczenie ograniczonej twardziny skóry
Portuguese	Péptido 144 inibidor do TGF- β 1 (TSLDASIIWAMMQN)	Tratamento da esclerodermia localizada
Slovak	Peptid 144 inhibítor TGF- β 1 (TSLDASIIWAMMQN)	Liečba lokalizovanej sklerodermie
Slovenian	Inhibitor TGF- β 1 peptidov 144 (TSLDASIIWAMMQN)	Zdravljenje lokalizirane skleroderme
Spanish	Péptido 144 inibidor del TGF- β 1 (TSLDASIIWAMMQN)	Tratamiento de la esclerodermia localizada
Swedish	Peptidhämmare 144 TGF- β 1 (TSLDASIIWAMMQN)	Behandling av den lokala skleroderma

¹ At the time of designation

Language	Active Ingredient	Indication
Norwegian	Peptid 144 TGF- β 1 inhibitor (TSLDASIIWAMMQN)	Behandling av lokalisert sklerodermi
Icelandic	Peptíð144 TGF- β 1 hemill(TSLDASIIWAMMQN)	Meðferð við staðbundnum húðherslissjúkdómi