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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Terguride for the treatment of systemic sclerosis

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in October 2013 on request of the Sponsor.

On 8 February 2013, orphan designation (EU/3/13/1104) was granted by the European Commission to High Tech Participations GmbH, Germany, for terguride for the treatment of systemic sclerosis.

What is systemic sclerosis?

Systemic sclerosis is a complex disease in which the immune system (the body's natural defences) is overactivated, causing inflammation and excess production of various proteins, particularly collagen. The reason why the immune system is overactivated is not known. Collagen is an important component of connective tissue (the tissue that supports the skin and internal organs).

The overproduction of collagen leads to the abnormal growth of connective tissue, causing the skin to become thick and hard. It can also damage tissues in the blood vessel walls of the internal organs, such as the heart, lungs and kidneys. This makes it more difficult for the blood to move through the vessels, causing tissue damage, circulation problems and high blood pressure.

Systemic sclerosis is a long-lasting debilitating disease and may be life threatening because of its possible effects on the gut, heart, lungs and kidneys.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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

At the time of designation, systemic sclerosis affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 81,000 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?

At the time of designation, there were no treatments for systemic sclerosis that could stop the build-up of collagen. Treatments authorised in the EU were aimed at relieving the symptoms of the disease and limiting the damage it causes. Several medicines were used to reduce inflammation and circulation problems. Bosentan was authorised in the EU specifically to treat patients with systemic sclerosis who have high blood pressure in the lungs or 'digital ulcers' (sores on the fingers and toes).

The sponsor has provided sufficient information to show that terguride might be of significant benefit for patients with systemic sclerosis because it works in a different way to existing treatments by targeting the disease itself rather than its consequences and early studies show that it may reduce the build-up of connective tissue. 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?

Terguride mainly blocks the action of serotonin, a neurotransmitter (a substance that allows nerve cells to communicate with each other) which is found in high amounts in patients with systemic fibrosis. In the blood vessel walls, serotonin attaches to certain receptors on the surfaces of cells and is involved in the production of fibrous connective tissue. Terguride is expected to attach to the receptors for serotonin on the cells in the blood vessel walls, preventing serotonin from attaching to them. This is expected to block the normal action of serotonin, thereby reducing the abnormal growth of connective tissue.

What is the stage of development of this medicine?

The effects of terguride have been evaluated in experimental models.

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

At the time of submission, terguride was not authorised anywhere in the EU for systemic sclerosis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2013 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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).

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:

High Tech Participations GmbH
Kreuzstrasse 34
D 40210 Düsseldorf
Germany
Telephone: +49 211 86 289 450
Telefax: +49 211 86 289 455
E-mail: PFolle@triginta-capital.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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Terguride	Treatment of systemic sclerosis
Bulgarian	Тергурид	Лечение на системна склероза
Czech	Tergurid	Léčba systémové sklerodermie
Danish	Tergurid	Behandling af systemisk sklerose
Dutch	Terguride	Behandeling van systeem sclerose
Estonian	Terguriid	Süsteemse sklerodermia ravi
Finnish	Terguridi	Systeemisen skleroosin hoito
French	Terguride	Traitement de la sclérose systémique
German	Tergurid	Behandlung der systemischen Sklerose
Greek	Τεργουρίδη	Θεραπεία της συστηματικής σκλήρυνσης
Hungarian	Tergurid	Szisztémás scleroderma kezelése
Italian	Terguride	Trattamento della sclerosi sistemica
Latvian	Tergurīds	Sistēmiskas sklerozes ārstēšana
Lithuanian	Terguridas	Sisteminės sklerozės gydymas
Maltese	Terguride	Kura tas-sklerosi sistemika
Polish	Terguryd	Leczenie twardziny narządowej
Portuguese	Terguride	Tratamento da esclerose sistémica
Romanian	Terguridă	Tratamentul sclerozei sistemice
Slovak	Tergurid	Liečba systémovej sklerózy
Slovenian	Tergurid	Zdravljenje sistemske skleroze
Spanish	Tergurida	Tratamiento de la esclerosis sistémica
Swedish	Tergurid	Behandling av systemisk skleros
Norwegian	Tergurid	Behandling av systemisk sklerose
Icelandic	Tergúríð	Meðferð við dreifðum herslismeinum

[1] At the time of designation