



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Autologous stromal vascular cell fraction from adipose tissue for the treatment of systemic sclerosis

On 28 April 2016, orphan designation (EU/3/16/1643) was granted by the European Commission to Cytori Ltd, United Kingdom, for autologous stromal vascular cell fraction from adipose tissue for the treatment of systemic sclerosis.

#### What is systemic sclerosis?

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

Overproduction of collagen leads to abnormal growth of connective tissue, causing the skin to become thick and hard. Initial symptoms include swollen fingers and hands, followed by thickened skin over the arms, legs, face and trunk. The disease can also damage the walls of blood vessels of internal organs such as the heart, lungs and kidneys. This makes it more difficult for the blood to flow, causing tissue damage and circulation problems.

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.

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

At the time of designation, systemic sclerosis affected approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 180,000 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).

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\*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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).



## **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 has been authorised in the EU specifically to treat patients with systemic sclerosis in whom poor blood circulation caused by the disease has led to the development of 'digital ulcers' (sores on the fingers and toes).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit in systemic sclerosis because preliminary studies suggest it can produce improvement in patients whose condition has not responded to existing treatments. 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?**

The medicine is prepared from the patient's own body fat (adipose tissue). The fat is broken down in the laboratory using digestive enzymes to remove fat cells but leave behind other cells such as leukocytes and macrophages (types of white blood cells), and stem cells (cells that can develop into different types of cell). Injecting these cells back under the patient's skin is expected to improve blood supply and promote tissue repair in the damaged areas, helping to slow down or stop the progression of the disease in the treated area.

## **What is the stage of development of this medicine?**

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with systemic sclerosis were ongoing.

At the time of submission, the medicine 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 23 March 2016 recommending the granting of this designation.

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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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Autologous stromal vascular cell fraction from adipose tissue	Treatment of systemic sclerosis
Bulgarian	Автоложна стромална съдово-клетъчна фракция от мастна тъкан	Лечение на системна склероза
Croatian	Autologne stanice stromalne vaskularne frakcije dobivene iz adipoznog tkiva	Liječenje sistemske skleroze
Czech	Autologní stromální vaskulární buněčná frakce pocházející z tukové tkáně	Léčba systémové sklerodermie
Danish	Autolog fedtvæv-afledt stromale vaskulære fraktion celler	Behandling af systemisk sklerose
Dutch	Autologe stromale vasculaire fractiecellen uit vetweefsel	Behandeling van systeem sclerose
Estonian	Autoloogsed rasvkoest saadud strooma vaskulaarse fraktsiooni rakud	Süsteemse sklerodermia ravi
Finnish	Autologinen rasvakudoksesta peräisin oleva stromaallinen vaskulaarinen solufraktio	Systeemisen skleroosin hoito
French	Fraction de cellule stroma-vasculaire autologue du tissu adipeux	Traitement de la sclérose systémique
German	Gefäßstromazellen aus autologem Fettgewebe	Behandlung der systemischen Sklerose
Greek	Κλάσμα αυτόλογων στρωματικών αγγειακών κυττάρων από λιπώδη ιστό	Θεραπεία της συστηματικής σκλήρυνσης
Hungarian	Zsírszövetből származó autológ sztromális vaszkuláris sejtfrakció	Szisztémás scleroderma kezelése
Italian	Frazione cellulare vascolare stromale autologa da tessuto adiposo	Trattamento della sclerosi sistemica
Latvian	Autologo stromas vaskulāro šūnu frakcija, kas iegūta no taukaudiem	Sistēmiskas sklerozes ārstēšana
Lithuanian	Autologinių kraujagyslių stromos ląstelių frakcija, išskirta iš riebalinio audinio	Sisteminės sklerozės gydymas
Maltese	Frazzjoni ta' ċelluli vaskulari stromali awtologużi minn tessut xaħmi	Kura tas-sklerosi sistemika
Polish	Autologiczne komórki uzyskane z frakcji podporowo-naczyniowej tkanki tłuszczowej	Leczenie twardziny narządowej
Portuguese	Fração celular autóloga do estroma vascular do tecido adiposo	Tratamento da esclerose sistémica
Romanian	Celule stromale vasculare autologe fracționate, derivate din țesut adipos	Tratamentul sclerozei sistemice
Slovak	Autológna stromálna vaskulárna bunková frakcia z adipózneho tkaniva	Liečba systémovej sklerózy

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Slovenian	Avtologna stromalna vaskularna celična frakcija maščobnega tkiva	Zdravljenje sistemske skleroze
Spanish	Fracción celular vascular estromal autóloga de tejido adiposo	Tratamiento de la esclerosis sistémica
Swedish	Autolog, vaskulär stromalcellfraktion från fettvävnad	Behandling av systemisk skleros
Norwegian	Autolog stromal vaskulær cellefraksjon fra fettvev	Behandling av systemisk sklerose
Icelandic	Samgena æða uppistöðuvefs frumupáttar úr fituvef	Meðferð við dreifðum herslismeinum

Withdrawn