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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous adipose tissue-derived mesenchymal stem cells for the treatment of thromboangiitis obliterans (Buerger's disease)

On 20 March 2017, orphan designation (EU/3/17/1854) was granted by the European Commission to SPC GmbH, Germany, for autologous adipose tissue-derived mesenchymal stem cells (also known as VascoStem) for the treatment of thromboangiitis obliterans (Buerger's disease).

What is thromboangiitis obliterans (Buerger's disease)?

Thromboangiitis obliterans, also known as Buerger's disease, is a disease in which the blood vessels become inflamed. Because of the inflammation, clots form within the blood vessels, blocking them and causing pain. The small blood vessels in the limbs (especially in the hands, legs and feet) are most often affected, and the lack of blood flow can cause skin ulcers (sores) and gangrene (decay and death of tissue). Ultimately, the limb may need to be amputated. Although the causes of the inflammation in thromboangiitis obliterans are not known, the disease is known to be closely linked to smoking.

Thromboangiitis obliterans is a long-term debilitating condition because of the pain, the development of ulcers and gangrene, and the risk of amputation.

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

At the time of designation, thromboangiitis obliterans affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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, iloprost was authorised in some EU Member States for the treatment of advanced thromboangiitis obliterans. Other medicines were also used to control the symptoms of the

*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 515,700,000 (Eurostat 2017).

disease such as pain, and to promote the healing of skin ulcers. Vascular surgery (surgery on the blood vessels) was used in a small number of cases.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with thromboangiitis obliterans because results of early studies in patients who could not be given surgery suggest it increased the distance they could walk without pain. 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 composed of mesenchymal stem cells (cells that can develop into certain other types of cells such as muscle and bone cells) that are extracted from the patient's own fat tissue. After extraction they are grown in the laboratory to increase their number before being injected back into the patient.

Although the way they work in the condition is not fully known, these stem cells produce various substances that help the growth and repair of body tissues. It is expected that treatment with the medicine will therefore reduce damage to the blood vessels and so improve the symptoms of the condition.

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 thromboangiitis obliterans were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for thromboangiitis obliterans. Orphan designation had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 February 2017 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 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Autologous adipose tissue-derived mesenchymal stem cells	Treatment of thromboangiitis obliterans (Buerger's disease)
Bulgarian	Мезенхимни стволови клетки получени от автоложна мастна тъкан	Лечение на облитериращ тромбоангиит (Болест на Бюргер)
Croatian	Autologne mezenhimne matične stanice podrijetlom iz masnog tkiva	Liječenje thromboangiitis obliterans (Buergerove bolesti)
Czech	Autologní mezenchymální kmenové buňky derivované z tukové tkáně	Léčba obliterující tromboangiitidy (Bürgerova choroba)
Danish	Autologe, fedtvævfledte mesenkymale stamceller	Behandling af thromboangiitis obliterans (Buergers sygdom)
Dutch	Uit autoloog vetweefsel afkomstige mesenchymale stamcellen	Behandeling van thromboangiitis obliterans (Buerger's ziekte)
Estonian	Autoloogsed rasvkoest pärit mesenhümaalsed tüvirakud	Oblitereeruva tromboangiidi (Buergeri haigus) ravi
Finnish	Autologisesta rasvakudoksesta saadut mesenkymaaliset kantasolut	Thromboangitis obliteransin (Bürgerin tauti) hoito
French	Cellules souches mésenchymateuses dérivées de tissu adipeux autologue	Traitement de la thromboangéite oblitérante (maladie de Buerger)
German	Aus autologem Fettgewebe stammende mesenchymale Stammzellen	Behandlung der Thrombangiitis obliterans (Morbus Winiwater-Buerger)
Greek	Μεσεγγυματικά βλαστικά κύτταρα προερχόμενα από αυτόλογο λιπώδη ιστό	Θεραπεία της αποφρακτικής θρομβοαγγειίτιδας (νόσος Buerger)
Hungarian	Zsírszövetből származó autológ mezenchymalis őssejtek	Thromboangiitis obliterans (Bürger-kór) kezelésére
Italian	Cellule staminali mesenchimali derivate da tessuto adiposo autologo	Trattamento della tromboangioite obliterante (malattia di Buerger)
Latvian	Autologas no taukaudiem iegūtas mezenhimālas cilmes šūnas	Obliterējoša endarterīta (Bergera slimības) ārstēšana
Lithuanian	Autologinis riebalinis audinys išskirtas iš mezenchimos kamieninių ląstelių	Obliteruojančio trombangito (<i>Buerger liga</i>) gydymas
Maltese	Ċelluli staminali mesenkimali derivati minn tessut adipuż awtologu	Kura tat-tromboangite obliterans (marda ta' Buerger)
Polish	Autologiczne mezenchymalne komórki macierzyste otrzymane z tkanki tłuszczowej	Leczenie zakrzepowo-zarostowego zapalenie tętnic (choroby Buergera)
Portuguese	Células estaminais mesenquimais derivadas do tecido adiposo autólogo	Tratamento da tromboangeite obliterante (Doença de Buerger)
Romanian	Celule stem mezenchimale derivate din țesutul adipos autolog	Tratamentul trombangeitei obliterante (boala Búrger)
Slovak	Autológne mezenchýmové kmeňové bunky pochádzajúce z tukového tkaniva	Liečba obliterujúcej trombangiitídy (Bürgerovej choroby)

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Mezenhimske matične celice iz avtolognega adipoznega tkiva	Zdravljenje obliterantnega trombangiitisa (Buergerjeva bolezen)
Spanish	Células madre mesenquimales derivadas de tejido adiposo autólogo	Tratamiento de la tromboangitis obliterante (enfermedad de Buerger)
Swedish	Autologa fettvävnadsderiverade mesenkymala stamceller	Behandling av thromboangiitis obliterans (Buergers sjukdom)
Norwegian	Autologe fettvevsavledede mesenkymale stamceller	Behandling av thromboangiitis obliterans (Buergers sykdom)
Icelandic	Samgena mesenkímal stofnfrumur úr fituvef	Meðferð við thromboangiitis obliterans (Bürgers sjúkdómi)