6 May 2020
EMADOC-628903358-1831

Public summary of opinion on orphan designation

Romilkimab for the treatment of systemic sclerosis

On 9 January 2020, orphan designation EU/3/19/2246 was granted by the European Commission to Sanofi-Aventis Groupe, France, for romilkimab 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.

Collagen is an important component of connective tissue (the tissue that supports the skin and internal organs). Overproduction leads to abnormal growth of connective tissue, causing the skin to become thick and hard. Initial symptoms include swelling of fingers and hands, followed by a thickening of the 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 can be life threatening because of its possible effects on the heart, lungs and kidneys.

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

At the time of designation, systemic sclerosis affected less than 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 181,000 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 and abnormal growth of connective tissue. Treatments authorised in the EU were aimed at

*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 518,400,000 (Eurostat 2019).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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 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) and patients with pulmonary arterial hypertension secondary to systemic sclerosis.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with systemic sclerosis. Preliminary data suggest that the medicine might be able to reduce thickening of the skin; when used in combination with standard treatment to reduce inflammation, the medicine was effective in the most severe form of the condition (diffuse cutaneous systemic sclerosis).

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?

Romilkimab is a monoclonal antibody, a type of protein, designed to attach to 2 different components of the immune system called IL-4 and IL-13. These components are involved in the inflammation and excessive production of collagen in patients with systemic sclerosis. By binding to IL-4 and IL-13, the medicine is expected to relieve the symptoms of the disease and slow down its progression.

What is the stage of development of this medicine?

The effects of romilkimab have been evaluated in experimental models.

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

At the time of submission, romilkimab was not authorised anywhere in the EU for the treatment of systemic sclerosis. Orphan designation of romilkimab had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 December 2019, 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.

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	Romilkimab	Treatment of systemic sclerosis
Bulgarian	Ромилкимаб	Лечение на системна склероза
Croatian	Romilkimab	Liječenje sistemske skleroze
Czech	Romilkimab	Léčba systémové sklerodermie
Danish	Romilkimab	Behandling af systemisk sklerose
Dutch	Romilkimab	Behandeling van systeem sclerose
Estonian	Romilkimab	Süsteemse sklerodermia ravi
Finnish	Romilkimabi	Systeemisen skleroosin hoito
French	Romilkimab	Traitement de la sclérodermie systémique
German	Romilkimab	Behandlung der systemischen Sklerose
Greek	Ρομιλκιμάμπη	Θεραπεία της συστηματικής σκλήρυνσης
Hungarian	Romilkimab	Szisztémás scleroderma kezelése
Italian	Romilkimab	Trattamento della sclerosi sistemica
Latvian	Romilkimabs	Sistēmiskas sklerozes ārstēšana
Lithuanian	Romilkimabas	Sisteminės sklerozės gydymas
Maltese	Romilkimab	Kura tas-sklerosi sistemika
Polish	Romilkimab	Leczenie twardziny narządowej
Portuguese	Romilkimab	Tratamento da esclerose sistémica
Romanian	Romilkimab	Tratamentul sclerozei sistemice
Slovak	Romilkimab	Liečba systémovej sklerózy
Slovenian	Romilkimab	Zdravljenje sistemske skleroze
Spanish	Romilkimab	Tratamiento de la esclerosis sistémica
Swedish	Romilkimab	Behandling av systemisk skleros
Norwegian	Romilkimab	Behandling av systemisk sklerose
Icelandic	Romilkimab	Meðferð við dreifðum herslismeinum

[1] At the time of designation