



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation Fresolimumab for the treatment of focal segmental glomerulosclerosis

On 5 August 2011, orphan designation (EU/3/11/882) was granted by the European Commission to Genzyme Europe BV, the Netherlands, for fresolimumab for the treatment of focal segmental glomerulosclerosis.

What is focal segmental glomerulosclerosis?

Focal segmental glomerulosclerosis is a disease where the glomeruli (small filtering units in the kidneys which filter waste products from the blood and start urine production) are damaged and become scarred. This stops them from working properly, so that substances needed by the body, such as proteins, pass into the urine. Focal segmental glomerulosclerosis can affect both adults and children. Symptoms include swelling, low blood protein levels and high blood pressure. It is called 'focal segmental' because only parts of some glomeruli are affected.

The cause of focal segmental glomerulosclerosis is usually unknown but there are some factors such as certain diseases and drugs that may trigger it.

Focal segmental glomerulosclerosis is a long-term debilitating and life-threatening disease because it may lead to kidney failure.

What is the estimated number of patients?

At the time of designation, focal segmental glomerulosclerosis affected not more than 4.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 228,000 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).

*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 506,300,000 (Eurostat 2011).



What treatments are available?

At the time of designation, ciclosporin was authorised in some EU countries specifically for the treatment of focal segmental glomerulosclerosis, while other medicines were authorised to treat the symptoms

The sponsor has provided sufficient information to show that fresolimumab might be of significant benefit for patients with focal segmental glomerulosclerosis because it works in a different way to existing treatments and early studies show that it might improve kidney function. 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?

Fresolimumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to 'transforming growth factor beta' (TGF- β), a protein that controls tissue growth. In focal segmental glomerulosclerosis, TGF- β is found at high levels and is thought to be responsible for damaging the glomeruli. By attaching to TGF- β , fresolimumab is expected to block its action, thus helping to reduce scarring in the glomeruli.

What is the stage of development of this medicine?

The effects of fresolimumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with fresolimumab in patients with focal segmental glomerulosclerosis had finished.

At the time of submission, fresolimumab was not authorised anywhere in the EU for focal segmental glomerulosclerosis. Orphan designation of this medicine had been granted in United States of America for primary focal segmental glomerulosclerosis.

In accordance with Regulation (EC) No 1411/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 May 2011 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:

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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	Fresolimumab	Treatment of focal segmental glomerulosclerosis
Bulgarian	Фрезолимумаб	Лечение на огнищно-сегментна гломерулна склероза
Czech	Fresolimumab	Léčba fokálně segmentální glomerulosklerózy
Danish	Fresolimumab	Behandling af fokal segmental glomerulosklerose
Dutch	Fresolimumab	Behandeling van focale segmentale glomerulosclerose
Estonian	Fresolimumab	Fokaal-segmentaarse glomeruloskleroosi ravi
Finnish	Fresolimumabi	Fokaalisen segmentaalisen glomeruloskleroosin hoito
French	Frésolimumab	Traitement de la glomérulonéphrite focale segmentaire
German	Fresolimumab	Behandlung der fokal segmentalen Glomerulosklerose
Greek	Φρεσολιμουμαμπη	Θεραπεία εστιακής τμηματικής σπειραματοσκλήρυνσης τ
Hungarian	Frezolimumab	Focalis szegmentális glomerulosclerosis kezelésére
Italian	Fresolimumab	Tattamento della glomerulosclerosi focale segmentaria
Latvian	Frezolimumab	Fokālas segmentāras glomerulosklerozes ārstēšana
Lithuanian	Frezolimumabas	Židininės segmentinės glomerulosklerožės gydymas
Maltese	Fresolimumab	Kura tal-glomeruloskleroži segmentali fokali
Polish	Frezolimumab	Leczenie ogniskowego segmentalnego szklwienia kłębuszków nerkowych
Portuguese	Fresolimumab	Tratamento da glomerulosclerose focal e segmentar
Romanian	Fresolimumab	Tratamentul glomerulosclerozei focale și segmentare
Slovak	Fresolimumab	Liečba fokálnej segmentálnej glomerulosklerózy
Slovenian	Fresolimumab	Zdravljenje fokalne segmentne glomeruloskleroze
Spanish	Fresolimumab	Tratamiento de la glomeruloesclerosis focal y segmentaria
Swedish	Fresolimumab	Behandling av fokal segmentell glomeruloskleros
Norwegian	Fresolimumab	Behandling av fokal segmental glomerulosklerose
Icelandic	Fresólímúmaþ	Meðferð við staðbundnu hlutanýrnaðnahersli

¹ At the time of designation