



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Propagermanium for the treatment of focal segmental glomerulosclerosis

On 19 November 2018, orphan designation (EU/3/18/2100) was granted by the European Commission to Quality Regulatory Clinical Ireland Limited, Ireland, for propagermanium 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.

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 affected by the condition?

At the time of designation, focal segmental glomerulosclerosis affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 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, 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 propagermanium might be of significant benefit for patients with focal segmental glomerulosclerosis. Laboratory studies showed that 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 517,400,000 (Eurostat 2018).



medicine could be an alternative for patients in whom cyclosporin is not effective. 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?

Inflammation is an important aspect of focal segmental glomerulosclerosis. Propagermanium is expected to work by blocking a receptor (target) on the surface of kidney cells called CCR2, which is involved in the inflammatory response in the kidney. By blocking CCR2, the medicine is expected to reduce the inflammation associated with the condition, therefore reducing damage and scarring to the glomeruli and improving symptoms of the condition.

What is the stage of development of this medicine?

The effects of propagermanium have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with focal segmental glomerulosclerosis had been started.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 October 2018 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	Propagermanium	Treatment of focal segmental glomerulosclerosis
Bulgarian	Пропагерманиум	Лечение на огнищно-сегментна гломерулна склероза
Croatian	Propagermanium	Liječenje fokalne segmentalne glomeruloskleroze
Czech	Propagermanium	Léčba fokálně segmentální glomerulosklerózy
Danish	Propagermanium	Behandling af fokal segmental glomerulosklerose
Dutch	Propagermanium	Behandeling van focale segmentale glomerulosclerose
Estonian	Propagermaanium	Fokaal-segmentaalse glomeruloskleroosi ravi
Finnish	Propagermanium	Fokaalisen segmentaalisien glomeruloskleroosin hoito
French	Propagermanium	Traitement de la glomérulonéphrite focale segmentaire
German	Propagermanium	Behandlung der fokal segmentalen Glomerulosklerose
Greek	Προπαγερμάνιο	Θεραπεία εστιακής τμηματικής σπειραματοσκλήρυνσης
Hungarian	Propagermanium	Focalis szegmentális glomerulosclerosis kezelése
Italian	Propagermanium	Trattamento della glomerulosclerosi focale segmentaria
Latvian	Propagermānijs	Fokālas segmentāras glomerulosklerozes ārstēšana
Lithuanian	Propagermanis	Židininės segmentinės glomerulosklerozės gydymas
Maltese	Propagermanium	Kura tal-glomerulosklerożi segmentali fokali
Polish	Propagermanium	Leczenie ogniskowego segmentalnego szklwienia kłębuszków nerkowych
Portuguese	Propagermanio	Tratamento da glomeruloesclerose focal e segmentar
Romanian	Propagermaniu	Tratamentul glomerulosclerozei focale și segmentare
Slovak	Propagermanium	Liečba fokálnej segmentálnej glomerulosklerózy
Slovenian	Propagermanij	Zdravljenje fokalne segmentne glomeruloskleroze
Spanish	Propagermanio	Tratamiento de la glomeruloesclerosis focal y segmentaria
Swedish	Propagermanium	Behandling av fokal segmentell glomeruloskleros
Norwegian	Propagermanium	Behandling av fokal segmental glomerulosklerose
Icelandic	Propagermaníum	Meðferð við staðbundnu hlutanyrnaðrakersli

¹ At the time of designation