



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminy-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide) for the treatment of C3 glomerulopathy

On 17 February 2016, orphan designation (EU/3/16/1608) was granted by the European Commission to Amyndas Pharmaceuticals S.A., Greece, for S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminy-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide) for the treatment of C3 glomerulopathy.

What is C3 glomerulopathy?

C3 glomerulopathy is a condition in which a protein of the immune system (known as C3) accumulates in the kidneys, damaging them and impairing their function. The condition is caused by over-activation of part of the immune system and can be either genetic or acquired.

Patients with C3 glomerulopathy usually have signs of kidney problems, such as blood or protein in urine, high blood pressure, blurred vision and swelling in the hands and feet.

C3 glomerulopathy is life threatening and debilitating in the long term because it can lead to kidney failure.

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

At the time of designation, C3 glomerulopathy affected approximately 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 72,000 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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).



What treatments are available?

At the time of orphan designation, no satisfactory treatments for C3 glomerulopathy were authorised in the EU. Because the disease involves the body's immune system, immunosuppressant medicines were usually used. Other treatments included plasmapheresis (a blood purification procedure), plasma infusion and antiplatelet agents. Patients developing end-stage kidney disease received dialysis and kidney transplantation.

How is this medicine expected to work?

The medicine works by attaching to the C3 protein. This protein is part of a group of proteins known as the 'complement system', which normally helps the immune system to fight infections but which in patients with C3 glomerulopathy causes damage to the kidneys. By attaching to this protein, the medicine is expected to inactivate it, thereby slowing down the damage to the kidneys.

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, no clinical trials with the medicine in patients with C3 glomerulopathy had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for C3 glomerulopathy 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 21 January 2016 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	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Treatment of C3 glomerulopathy
Bulgarian	S3,S13-цикло(D-тиролзил-L-изолевкил-L-цистеинил-L-валил-1-метил-L-триптофил-L-глутаминил-L-аспартил-L-триптофил-N-метил-L-глицил-L-аланил-L-хистидил-L-агринил-L-цистеинил-N-метил-L-изолевкинамид)	Лечение на C3 гломерулопатия
Croatian	S3,S13-ciklo(D-tirozil-L-izoleucil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutaminil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-izoleucinamid)	Liječenje C3 glomerulopatije
Czech	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Léčba C3 glomerulopatie
Danish	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamid)	Behandling af C3 glomerulopati
Dutch	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Behandeling van C3 glomerulopathie
Estonian	S3,S13-tsüklo(D-türolsüül-L-isoletsüül-L-tsüsteinüül-L-valüül-1-metüül-L-trüptofüül-L-glutaminüül-L-aspartüül-L-trüptofüül-N-metüül-L-glütsüül-L-alanüül-L-histidüül-L-arginüül-L-tsüsteinüül-N-meüül-L-isoletsinamiid)	C3 glomerulopaatia ravi
Finnish	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	C3 glomerulopatian hoito
French	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Traitement de la glomérulopathie à dépôts de C3
German	S3,S13-Cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyL-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamid)	Behandlung von C3 Glomerulopathie

¹ At the time of designation

Language	Active ingredient	Indication
Greek	S3,S13-κυκλο(D-τυρολσουλ-L-ισολευκυλ-L-κυστεΐνουλ-L-βαλυλ-1-μεθυλ-L-τρυπτοφυλ-L-γλουταμινουλ-L-ασπαρτυλ-L-τρυπτοφυλ-N-μεθυλ-L-γλυκυλ-L-αλανουλ-L-ιστιδουλ-L-αργινουλ-L-κυστενουλ-N-μεθυλ-L-ισολευκιναμίδιο)	Θεραπεία της C3 σπειραματοπάθειας
Hungarian	S3,S13-cyclo(D-tyrolyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminy-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	C3 glomerulopátia kezelése
Italian	S3,S13-cyclo(D-tyrolyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminy-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Trattamento della glomerulopatia C3
Latvian	S3,S13-ciklo(D-tirolzil-L-izoleicil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutamīnil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-izoleicīnamīds)	C3 glomerulopātijas ārstēšana
Lithuanian	S3,S13-ciklo(D-tirolzil-L-izoleucil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutaminil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-izoleucinamidas)	C3 glomerulopatijos gydymas
Maltese	S3,S13-cyclo(D-tyrolyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminy-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Kura tal-glomerulopatija C3
Polish	S3,S13-cyklo(D-tyrozyl-L-izoleucyl-L-cysteinyl-L-walil-1-metyl-L-tryptofanyl-L-glutaminy-L-aspartyl-L-tryptofanyl-N-metyl-L-glicyl-L-alanyl-L-histydyl-L-arginyl-L-cysteinyl-N-metyl-L-izoleucynamid)	Leczenie glomerulopatii C3
Portuguese	S3,S13-ciclo(D-tirolsil-L-izoleucil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutaminil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-izoleucinamida)	Tratamento da glomerulopatia por C3
Romanian	S3,S13-ciclo(D-tirolzil-L-izoleucil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutaminil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-izoleucinamidă)	Tratamentul glomerulopatiei C3
Slovak	S3,S13-cyklo(D-tyrolyl-L-isoleucyl-L-cysteinyl-L-valyl-1-metyl-L-tryptofyl-L-glutaminy-L-aspartyl-L-tryptofyl-N-metyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-metyl-L-izoleucinamid)	Liečba C3 glomerulopatie
Slovenian	S3,S13-ciklo(D-tirosil-L-izoleucil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutaminil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-izoleucinamid)	Zdravljenje C3 glomerulopatije

Language	Active ingredient	Indication
Spanish	S3,S13-ciclo(D-tirolsil-L-isoileucil-L-cisteinil-L-valil-1-metil-L-triptofil-L-glutaminil-L-aspartil-L-triptofil-N-metil-L-glicil-L-alanil-L-histidil-L-arginil-L-cisteinil-N-metil-L-isoileucinamida	Tratamiento de la glomerulopatía C3
Swedish	S3,S13-cyclo(D-tyrolsyl-L-isoileucyl-L-cysteinyL-L-valyl-1-metyl-L-tryptofyl-L-glutaminyL-L-aspartyl-L-tryptofyl-N-metyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyL-N-metyl-L-isoileucinamid)	Behandling av C3 glomerulopati
Norwegian	S3,S13-syklo(D-tyrolsyl-L-isoileucyl-L-cysteinyL-L-valyl-1-metyl-L-tryptofyl-L-glutaminyL-L-aspartyl-L-tryptofyl-N-metyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyL-N-metyl-L-isoileucinamid)	Behandling av C3-glomerulopati
Icelandic	S3,S13-cýcló(D-týrólsýl-L-ísóleucýl-L-cýsteinýl-L-valýl-1-methýl-L-trýptóphýl-L-glútaminýl-L-aspartýl-L-trýptóphýl-N-methýl-L-glýcýl-L-alanýl-L-histidýl-L-arginýl-L-cýsteinýl-N-methýl-L-ísóleucínamið)	Meðferð við C3 glomerulopathy