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Public summary of opinion on orphan designation

Avacopan for the treatment of C3 glomerulopathy

On 20 June 2017, orphan designation (EU/3/17/1880) was granted by the European Commission to ChemoCentryx Limited, United Kingdom, for avacopan (also known as CCX168 or (2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide) for the treatment of C3 glomerulopathy.

What is C3 glomerulopathy?

C3 glomerulopathy is a condition in which a protein of the immune (defence) 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 called the complement system.

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 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 41,000 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 orphan designation, no satisfactory treatments were authorised in the EU for C3 glomerulopathy. Because the disease involves the body's immune system, immunosuppressant medicines (that reduce the activity of the immune system) were usually used. Other treatments included plasmapheresis (a procedure to remove unwanted substances from plasma, the liquid part of

*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).

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the blood), infusion of healthy plasma and medicines to reduce blood pressure. Patients developing end-stage kidney disease received dialysis and kidney transplantation.

How is this medicine expected to work?

This medicine blocks a receptor (target) on cells called 'complement 5a (C5a) receptor', which is normally activated by C5a, another of the proteins of the complement system.

By blocking C5aR, the medicine is expected to reduce the inflammation caused by the activation of the complement system, thus reducing kidney damage and improving the symptoms of the disease.

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 C3 glomerulopathy were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for C3 glomerulopathy. Orphan designating of this medicine has 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 12 May 2017 recommending the granting of this designation.

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Avacopan	Treatment of C3 glomerulopathy
Bulgarian	Авакопан	Лечение на С3 гломерулопатия
Croatian	Avakopan	Liječenje C3 glomerulopatije
Czech	Avacopan	Léčba C3 glomerulopatie
Danish	Avacopan	Behandling af C3 glomerulopati
Dutch	Avacopan	Behandeling van C3 glomerulopathie
Estonian	Avakopaan	C3 glomerulopaatia ravi
Finnish	Avakopaani	C3 glomerulopatian hoito
French	Avacopan	Traitement de la glomérulopathie à dépôts de C3
German	Avacopan	Behandlung von C3 Glomerulopathie
Greek	Αβακοπάνη	Θεραπεία της C3 σπειραματοπάθειας
Hungarian	Avacopan	C3 glomerulopátia kezelése
Italian	Avacopan	Trattamento della glomerulopatia C3
Latvian	Avakopāns	C3 glomerulopātijas ārstēšana
Lithuanian	Avakopanas	C3 glomerulopatijos gydymas
Maltese	Avacopan	Kura tal-glomerulopatija C3
Polish	Awakopan	Leczenie glomerulopatii C3
Portuguese	Avacopano	Tratamento da glomerulopatia por C3
Romanian	Avacopan	Tratamentul glomerulopatiei C3
Slovak	Avacopan	Liečba C3 glomerulopatie
Slovenian	Avakopan	Zdravljenje C3 glomerulopatije
Spanish	Avacopán	Tratamiento de la glomerulopatía C3
Swedish	Avacopan	Behandling av C3 glomerulopati
Norwegian	Avakopan	Behandling av C3-glomerulopati
Icelandic	Avakópan	Meðferð við C3 glomerulopathy

[1] At the time of designation