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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised IgG1 kappa antibody against serum amyloid A and AL amyloid for the treatment of amyloid light-chain amyloidosis

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Rev.1: sponsor's name and address change	24 April 2015

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 8 February 2013, orphan designation (EU/3/13/1100) was granted by the European Commission to Onclave Therapeutics Limited, Ireland, for humanised IgG1 kappa antibody against serum amyloid A and AL amyloid for the treatment of amyloid light-chain amyloidosis.

In March 2015, Onclave Therapeutics Limited changed name to Prothena Therapeutics Limited.

What is amyloid light-chain amyloidosis?

Amyloid light-chain amyloidosis belongs to a group of diseases called systemic amyloidosis in which deposits of proteins (called amyloids) accumulate and cause damage in body organs. In amyloid light-chain amyloidosis, the amyloids consist of components of defective proteins (called light chains) that are produced by malfunctioning cells. The condition can cause serious damage to organs such as the kidneys, liver, gut, heart and the nervous system.

Amyloid light-chain amyloidosis is a long-term debilitating condition because of damage to organs such as the heart and kidneys.

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

At the time of designation, amyloid light-chain amyloidosis affected approximately 1.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 56,000 people*, and is

*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.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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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, no medicines were authorised in the EU for the treatment of amyloid light-chain amyloidosis. Stem cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow) was used in a small group of newly diagnosed patients.

How is this medicine expected to work?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (called an antigen). When injected into the patient's vein, it is expected to attach to the light chain proteins in the amyloids, breaking them down and allowing their removal from the body. This is expected to reduce the amyloid deposits in the organs and thereby the organ damage caused by the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicinal product in experimental models was ongoing.

At the time of submission, no clinical trials with the medicinal product in patients with amyloid light-chain amyloidosis had been started.

At the time of submission, the medicinal product was not authorised anywhere in the EU for amyloid light-chain amyloidosis. Orphan designation of the medicinal product had been granted in the United States of America for amyloid light-chain amyloidosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2013 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Humanised IgG1 kappa antibody against serum amyloid A and AL amyloid	Treatment of amyloid light-chain amyloidosis
Bulgarian	Хуманизирано IgG1 капа антитяло срещу серумен амилоид А и анти-AL амилоид	Лечение на амилоидоза с леки амилоидни вериги
Czech	Humanizovaná protilátka IgG1 kappa proti sérovému amyloidu A a AL amyloidu	Léčba amyloidózy z depozice amyloidu z lehkých řetězců
Danish	Humaniseret IgG1 kappa antistof mod serum amyloid A og AL amyloid	Behandling af amyloid letkæde amyloidose
Dutch	Gehumaniseerd IgG1 kappa-antilichamen tegen serum amyloïd A en AL-amyloïd	Behandeling van amyloïde lichte keten amyloïdose
Estonian	Seerumi A-amüloidi ja AL-amüloidi vastane humaniseeritud IgGI–kapa antikeha	Kergete ahelate amüloidist põhjustatud amüloidoosi ravi
Finnish	Humanisoitu IgG1-kappa-vasta-aine seerumin amyloidi A:ta ja AL-amyloidia vastaan	Kevytketjuamyloidoosin hoito
French	Anticorps IgG1 anti-kappa humanisé dirigé contre la protéine amyloïde A sérique et la protéine amyloïde AL	Traitement de l'amylose à chaînes légères
German	Humansierter IgG1-kappa-Antikörper gegen Serum-Amyloid A und AL-Amyloid	Behandlung der Leichtketten-(AL-) Amyloidose
Greek	Ανθρωποποιημένοο IgG1καντίσωμα έναντι του αμυλοειδούς Α και AL	Θεραπεία της αμυλοείδωσης της ελαφριάς αλυσίδας αμυλοειδούς
Hungarian	Szérum amyloid A és AL amyloid ellenes humanizált IgG1 kappa antitest	Amyloid könnyű láncú AL amyloidosis kezelése
Italian	Anticorpo IgG1 kappa umanizzato anti-amiloide A sierica e anti-amiloide AL	Trattamento dell'amiloidosi correlata ad amiloide a catena leggera
Latvian	Humanizēta IgG1 kappa antiviela pret seruma amiloīdu A un AL amiloīdu	Amiloīda vieglās ķēdes amiloidozes ārstēšana
Lithuanian	Žmogaus IgG1 kapa antikūnas prieš serumo amiloidą A ir amiloidą AL	Lengvųjų grandžių amiloido amiloidozės gydymas
Maltese	Antikorp IgG1 kappa umanizzat kontra amilojde A tas-serum u amilojde AL	Kura tal-amilojdosi b'amilojde ta' katina ħafifa
Polish	Humanizowane przeciwciało IgG1 kappa przeciw amyloidowi A i amyloidowi AL surowicy	Leczenie amyloidozy łańcuchów lekkich
Portuguese	Anticorpo humanizado IgG1 kappa anti amilóide A e anti amilóide AL séricos	Tratamento da amiloidose por amilóides de cadeia leve
Romanian	Anticorp kappa IgG1 uman împotriva amiloidului seric A şi amiloidului AL	Tratamentul amiloidozei cu lanţuri uşoare
Slovak	Humanizovaná IgG1 kappa protilátka proti sérovému amyloidu A a AL amyloidu	Liečba amyloidózy z ľahkých reťazcov amyloidu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humanizirano IgGl kapa protitelo proti serumskemu amiloidu A in amiloidu AL	Zdravljenje amiloidoze lahko amiloidne verige
Spanish	Anticuerpo IgG1 kappa humanizado contra amiloide sérico A y AL amiloide	Tratamiento de la amiloidosis de amiloide de cadena ligera
Swedish	Humaniserad IgG1 kappa antikropp mot serum amyloid A och AL amyloid	Behandling av AL amyloidos
Norwegian	Humanisert IgG1 kappa antistoff mot serum amyloid A og AL-amyloid	Humanisert IgG1 kappa antistoff mot serum amyloid A og AL-amyloid
Icelandic	Manna IgG1 kappa mótefni gegn sermis A mýlildi í sermi og AL mýlildi	Meðferð við léttkeðjumýlildi