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EMA/COMP/360634/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant monoclonal antibody to human serum amyloid P component for the treatment of AL amyloidosis

On 29 July 2014, orphan designation (EU/3/14/1293) was granted by the European Commission to GlaxoSmithKline Trading Services Limited, Ireland, for recombinant monoclonal antibody to human serum amyloid P component for the treatment of AL amyloidosis.

What is AL amyloidosis?

AL amyloidosis belongs to a group of diseases called systemic amyloidosis in which deposits of proteins (called amyloids) accumulate and cause damage in tissues and organs such as the kidneys, liver, gut, heart and nerves.

In AL amyloidosis, the deposits come from proteins (called immunoglobulin light chains) produced in excess by malfunctioning white blood cells in the bone marrow. These deposits also contain serum amyloid P (SAP), a protein normally found in blood.

Symptoms of the condition vary widely depending on which organs are affected by the deposits and how much deposits have accumulated in them.

AL amyloidosis is a life-threatening and long-term debilitating condition because of damage to organs, particularly the heart and kidneys.

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

At the time of designation, AL 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 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 511,100,000 (Eurostat 2014).

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What treatments are available?

At the time of designation, no medicines were authorised in the EU for the treatment of AL amyloidosis. Patients often received treatment with medicines (chemotherapy) originally designed to treat cancers of white blood cells, in order to target the malfunctioning white blood cells. 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 an antibody, a type of protein designed to attach to the SAP protein in amyloid deposits. Patients are first treated with another medicine called carboxy pyrrolidine hexanoyl pyrrolidine carboxylate, which removes the SAP that normally circulates in the blood, to which the antibody would also attach. The antibody is expected to attach to the SAP in the amyloid deposits, thus stimulating the immune system to remove the unwanted material. This is expected to help reduce the deposits of amyloid that cause damage to the organs.

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 AL amyloidosis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for AL amyloidosis 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 12 June 2014 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	Recombinant monoclonal antibody to human serum amyloid P component	Treatment of AL amyloidosis
Bulgarian	Рекомбинантно моноклонално антитяло срещу човешки серумен P амилоиден компонент	Лечение на лековерижна (AL) амилоидоза
Croatian	Rekombinantno monoklonsko protutijelo protiv P-komponente amiloida iz ljudskog seruma	Liječenje AL amiloidoze
Czech	Rekombinantní monoklonální protilátka proti lidské sérové P komponentě amyloidu	Léčba AL amyloidózy
Danish	Rekombinant monoklonalt antistof mod human serumamyloid P-komponent	Behandling af AL (amyloid let-kæde) amyloidose
Dutch	Recombinant monoklonaal antilichaam gericht tegen de menselijke serum amyloïde-P-component	Behandeling van AL amyloïdose
Estonian	Inimese seerumi amüloid-P komponendi vastane rekombinantne monoklonaalne antikeha	AL-amüloidoosi ravi
Finnish	Ihmisen seerumiamyloidi P- komponentin rekombinantti monoklonaalinen vasta-aine	AL-amyloidoosin hoito
French	Anticorps monoclonal recombinant dirigé contre la composante P de l'amyloïde sérique humaine	Traitement de l'amyloïdose de type AL
German	Rekombinanter monoklonaler Antikörper gegen die humane Serum Amyloid P Komponente	Behandlung der AL Amyloidose
Greek	Ανασυνδυασμένο μονοκλωνικό αντίσωμα έναντι του ανθρώπινου συστατικού του αμυλοειδούς P του ορού	Θεραπεία της AL-αμυλοειδωσης
Hungarian	Humán szérum amiloid-P-komponens elleni rekombináns monoklonális antitest	Amiotrófiás laterális amiloidózis kezelése
Italian	Anticorpo monoclonale ricombinante contro la componente P dell'amiloide sierica umana	Trattamento dell'amiloidosi di tipo AL
Latvian	Rekombinanta monoklonāla antiviela pret cilvēka seruma amiloīda P komponentu	Vieglo ķēžu (AL) amiloidozes ārstēšana
Lithuanian	Rekombinantinis monokloninis antikūnas, prieš žmogaus serumo amiloido P komponentą	AL amiloidozės gydymas
Maltese	Antikorp monoklonali rikombinanti għall-komponent amilojd P tas-serum uman	Kura tal-amilojdosi tat-tip AL
Polish	Rekombinowane przeciwciało monoklonalne przeciw amyloidowi P ludzkiego osocza	Leczenie układowej amyloidozy łańcuchów lekkich (AL)
Portuguese	Anticorpo monoclonal recombinante anti componente P da amiloide sérica humana	Tratamento da Amilóidose primária
Romanian	Anticorp monoclonal recombinant anti-componenta P a amiloidului seric uman	Tratamentul amiloidozei de tip AL

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Rekombinantná monoklonálna protilátka proti ľudskej sérovej komponente P amyloidu	Liečba AL amyloidózy
Slovenian	Rekombinantno monoklonsko protitelo proti komponenti humanega serumskega amiloida P	Zdravljenje AL amiloidoze
Spanish	Anticuerpo monoclonal recombinante del componente amiloide P de suero humano	Tratamiento del AL amiloidosis
Swedish	Rekombinant monoklonal antikropp mot human serum-amyloid P-komponent	Behandling av AL amyloidos
Norwegian	Rekombinant monoklonalt antistoff mot human serum-amyloid P-komponent	Behandling av AL amyloidose
Icelandic	Raðbrigða einstofna mótefni gegn manna sermis-amýlóíð-P-þætti	Meðferð við AL mýlildi