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Public summary of opinion on orphan designation

Daratumumab for the treatment of AL amyloidosis

On 25 May 2018, orphan designation (EU/3/18/2020) was granted by the European Commission to Janssen-Cilag International N.V., Belgium, for daratumumab 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 are made up of proteins (called immunoglobulin light chains) produced in excess by malfunctioning white blood cells in the bone marrow. These deposits also contain a protein normally found in the blood called serum amyloid P.

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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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, no medicines were authorised in the EU for the treatment of AL amyloidosis. Patients often received treatment with medicines (chemotherapy) authorised for the treatment of cancers of white blood cells, in order to target the malfunctioning white blood cells.

[*]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).

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How is this medicine expected to work?

The medicine is a monoclonal antibody (a type of protein) designed to recognise and attach to a specific structure called 'CD38' which is found in great numbers on the white blood cells that produce immunoglobulin light chains. Once attached, it is expected to activate the immune system to attack and kill the white blood cells. This is expected to reduce the deposits of proteins, and so improve symptoms of the disease.

What is the stage of development of this medicine?

The effects of daratumumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with daratumumab in patients with AL amyloidosis were ongoing.

At the time of submission, daratumumab (Darzalex) was authorised in the EU for the treatment of multiple myeloma.

At the time of submission, daratumumab was not authorised anywhere in the EU for AL amyloidosis. Orphan designation of daratumumab 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 19 April 2018 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	Daratumumab	Treatment of AL amyloidosis
Bulgarian	Даратумумаб	Лечение на лековерижна (AL) амилоидоза
Croatian	Daratumumab	Liječenje AL amiloidoze
Czech	Daratumumab	Léčba AL amyloidózy
Danish	Daratumumab	Behandling af AL (amyloid let-kæde) amyloidose
Dutch	Daratumumab	Behandeling van AL amyloïdose
Estonian	Daratumumab	AL-amüloidoosi ravi
Finnish	Daratumumabi	AL-amyloidoosin hoito
French	Daratumumab	Traitement de l'amyloïdose de type AL
German	Daratumumab	Behandlung der AL Amyloidose
Greek	Δαρατουμουμάμπη	Θεραπεία της AL-αμυλοειδωσης
Hungarian	Daratumumab	Amiotrófiás laterális amiloidózis kezelése
Italian	Daratumumab	Trattamento dell'amiloidosi di tipo AL
Latvian	Daratumumabs	Vieglo ķēžu (AL) amiloidozes ārstēšana
Lithuanian	Daratumumabas	AL amiloidozės gydymas
Maltese	Daratumumab	Kura tal-amilojdosi tat-tip AL
Polish	Daratumumab	Leczenie układowej amyloidozy łańcuchów lekkich (AL)
Portuguese	Daratumumab	Tratamento da Amilóidose primária
Romanian	Daratumumab	Tratamentul amiloidozei de tip AL
Slovak	Daratumumab	Liečba AL amyloidózy
Slovenian	Daratumumab	Zdravljenje AL amiloidoze
Spanish	Daratumumab	Tratamiento del AL amiloidosis
Swedish	Daratumumab	Behandling av AL amyloidos
Norwegian	Daratumumab	Behandling av AL amyloidose
Icelandic	Daratúmúmab	Meðferð við AL mýlildi

[1] At the time of designation