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Public summary of opinion on orphan designation

Florbetaben (18F) for the diagnosis of AL amyloidosis

On 22 April 2020, orphan designation EU/3/20/2268 was granted by the European Commission to Life Molecular Imaging GmbH, Germany, for florbetaben (18F) for the diagnosis 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.

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 eligible for the diagnosis of the condition?

At the time of designation, AL amyloidosis affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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 methods of diagnosis are available?

At the time of designation, gadolinium contrast agents used with magnetic resonance imaging (MRI) to improve image quality were authorised in the EU for the diagnosis of AL amyloidosis.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with AL amyloidosis because the medicine is used in a different body scan method called PET (positron emission tomography) and can be used in patients who cannot undergo MRI of the heart with currently authorised contrast agents.

This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Florbetaben (18F) is a radiopharmaceutical, a medicine that emits small amounts of radiation. It works by attaching to amyloid deposits present in patients with AL amyloidosis. After it attaches to the deposits, the radiation it emits can be detected by the PET scanner, enabling doctors to see the location and extent of any deposits that are present.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials with florbetaben (18F) in patients with AL amyloidosis were ongoing.

At the time of submission, florbetaben (18F) was authorised in the EU under the name Neuraceq for the detection of another type of protein deposit called β-amyloid plaques, which are present in the brain of patients with Alzheimer's disease and other types of dementia.

At the time of submission, florbetaben (18F) was not authorised anywhere in the EU for the diagnosis of AL amyloidosis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 19 March 2020, 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.

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	Florbetaben (^{18}F)	Diagnosis of AL amyloidosis
Bulgarian	Флорбетабен (^{18}F)	Диагноза на лековерижна (AL) амилоидоза
Croatian	Florbetaben (^{18}F)	Dijagnosticiranje AL amiloidoze
Czech	Florbetaben (^{18}F)	Diagnóza AL amyloidózy
Danish	Florbetaben (^{18}F)	Diagnose af AL (amyloid let-kæde) amyloidose
Dutch	Florbetaben (^{18}F)	Diagnose van AL amyloïdose
Estonian	Florbetabeen (^{18}F)	AL-amüloidoosi diagnoosimiseks
Finnish	Florbetabeeni (^{18}F)	AL-amyloidoosin diagnosointi
French	Florbetaben (^{18}F)	Diagnostic de l'amyloïdose de type AL
German	Florbetaben (^{18}F)	Diagnose der AL Amyloidose
Greek	Florbetaben (^{18}F)	Διάγνωση της AL-αμυλοείδωσης
Hungarian	Florbetabén (^{18}F)	AL amiloidózis diagnosztizálása
Italian	Florbetaben (^{18}F)	Diagnosi dell'amiloidosi di tipo AL
Latvian	Florbetabēns (^{18}F)	Vieglo ķēžu (AL) amiloidozes diagnostika
Lithuanian	Florbetabenas (^{18}F)	AL amiloidozės diagnozė
Maltese	Florbetaben (^{18}F)	Dijanjosi tal-amilojdosi tat-tip AL
Polish	Florbetaben (^{18}F)	Rozpoznawanie układowej amyloidozy łańcuchów lekkich (AL)
Portuguese	Florbetabeno (^{18}F)	Diagnóstico da Amilóidose primária
Romanian	Florbetaben (^{18}F)	Diagnosticul amiloidozei de tip AL
Slovak	Florbetabenu (^{18}F)	Diagnóza AL amyloidózy
Slovenian	Florbetaben (^{18}F)	Diagnosticiranje AL amiloidoze
Spanish	Florbetaben (^{18}F)	Diagnóstico del AL amiloidosis
Swedish	Florbetaben (^{18}F)	Diagnos av AL amyloidos
Norwegian	Florbetaben (^{18}F)	Diagnose av AL-amyloidose
Icelandic	Florbetaben (^{18}F)	Sjúkdómsgreining við AL mýlildi

[1] At the time of designation