



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

2-(2-(¹⁸F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine for the diagnosis of corticobasal degeneration

On 19 October 2020, orphan designation EU/3/20/2340 was granted by the European Commission to Life Molecular Imaging GmbH, Germany, for 2-(2-(¹⁸F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine (also known as [¹⁸F]PI-2620) for the diagnosis of corticobasal degeneration.

What is corticobasal degeneration?

Corticobasal degeneration is a progressive disorder that involves the gradual deterioration of brain cells in specific areas of the brain, including those connected with movement, reasoning and memory. Patients with corticobasal degeneration have deposits of a protein called 'tau' in nerve cells, which are thought to cause the gradual deterioration of brain tissue seen in these patients.

Symptoms include difficulty controlling limbs on one side of the body, muscle stiffness, slow movement, problems with balance and co-ordination, personality changes and dementia (loss of intellectual function). The disease usually starts in people aged over 50 years.

Corticobasal degeneration is a debilitating and life-threatening disease that leads to paralysis and premature death.

What is the estimated number of patients eligible for the diagnosis of the condition?

At the time of designation, the number of patients eligible for diagnosis of corticobasal degeneration was approximately 0.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 47,000 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).

*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).



What methods of diagnosis are available?

At the time of designation, no satisfactory methods were authorised in the EU for the diagnosis of corticobasal degeneration. The disease was generally diagnosed based on clinical evaluation.

How is this medicine expected to work?

The medicine is a radiopharmaceutical, a medicine that emits small amounts of radiation. It works by attaching to tau deposits present in the brain. 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 tau deposits that are present, and so helping to distinguish the condition from other brain disorders.

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 corticobasal degeneration were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the diagnosis of corticobasal degeneration 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 10 September 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

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.