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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 progressive supranuclear palsy

On 21 August 2020, orphan designation EU/3/20/2313 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 for the diagnosis of progressive supranuclear palsy.

What is progressive supranuclear palsy?

Progressive supranuclear palsy (PSP), which is also known as Steele-Richardson-Olszewski syndrome, is a rare disease that involves the gradual deterioration of parts of the brain. Symptoms include loss of balance with unexplained falls, stiffness, difficulty moving the eyes, particularly up and down, personality changes and dementia (loss of intellectual function). The disease usually starts in people aged over 60 years and gradually gets worse over a number of years.

Patients with PSP have abnormal tangles of a protein called 'tau' in their brain, which are thought to cause the gradual deterioration of brain tissue seen in these patients.

PSP is a debilitating and life-threatening disease that leads to parkinsonism, 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 progressive supranuclear palsy was estimated to be approximately 1.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 93,000 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 eligible for diagnosis 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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What methods of diagnosis are available?

No satisfactory methods of diagnosis of PSP were authorised at the time of application. Magnetic resonance imaging (MRI) was used to support diagnosis based on clinical signs and symptoms in late stages of the disease.

How is this medicine expected to work?

The medicine is a radiopharmaceutical (a medicine that emits a small amount of radioactivity) that binds specifically to tau protein in the patient's brain. The resulting build-up of radiation makes it possible to see the protein tangles using a technique called positron emission tomography (PET) scan and help to determine which patients are suffering from the condition.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials with 2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine in patients with progressive supranuclear palsy were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the diagnosis of PSP 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 16 July 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.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine	Diagnosis of progressive supranuclear palsy
Bulgarian	2-(2-(18F)флуоропиридин-4-ил)-9H-пироло[2,3-b:4,5-c']дипиридин	на прогресивна супрануклеарна парализа
Croatian	2-(2-(18F)fluoropiridin-4-il)-9H-pirolo-[2,3-b:4,5-c']dipiridin	Dijagnosticiranje progresivne supranuklearne paralize
Czech	2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridin	Diagnóza progresivní supranukleární obrny
Danish	2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridin	Diagnose af progressiv, supranukleær parese
Dutch	2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine	Diagnose van progressieve supranucleaire paralyse
Estonian	2-(2-(18F)fluoropüridiin-4-üül)-9H-pürrolo[2,3-b:4,5-c']dipüridiin	Progressiivse supranukleaarse halvatuse diagnoosimine
Finnish	2-(2-(18F)fluoripyridiini-4-yyli)-9H-pyrrolo[2,3-b:4,5-c']dipyridiini	Progressiivisen supranukleaarisen halvauksen diagnosointi
French	2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine	Diagnostic de la paralysie supranucléaire progressive
German	2-(2-(18F)Fluorpyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridin	Diagnose der progressiven supranukleären Blickparese
Greek	2-(2-(18F)φθοροπυριδιν-4-υλ)-9H-πυρρολο[2,3-b:4,5-c']διπυριδίνη	Διάγνωση προϊούσας υπερπυρηνικής παράλυσης
Hungarian	2-(2-(18F)fluor-piridin-4-il)-9H-pirrolo[2,3-b:4,5-c']dipiridin	Progresszív supranuclearis bénulás diagnosztizálása
Italian	2-(2-(18F)fluor-piridin-4-il)-9H-pirrolo[2,3-b:4,5-c']dipiridin	Diagnosi della paralisi sopranucleare progressiva
Latvian	2-(2-(18F)fluoropiridīn-4-il)-9H-pirrolo[2,3-b:4,5-c']dipiridīns	Progresējošās supranukleārās paralīzes diagnostika
Lithuanian	2-(2-(18F)fluoropiridin-4-il)-9H-pirolo[2,3-b:4,5-c']dipiridinas	Progresuojančio supranuklearinio paralyžiaus diagnozė
Maltese	2-(2-(18F)fluworopiridina-4-il)-9H-pirrolo[2,3-b:4,5-c']dipiridina	Dijanjoži ta’ paraliżi supranukleari progressiva

[1] At the time of designation

Language	Active ingredient	Indication
Polish	2-(2-(18F)fluoropirydyn-4-ylo)-9H-pirolo[2,3-b:4,5-c']dipirydyna	Diagnoza postępującego porażenia nadjądrowego
Portuguese	2-(2-(18F)fluoropiridin-4-il)-9H-pirrolo[2,3-b:4,5-c']dipiridina	Diagnóstico da paralisia supranuclear progressiva
Romanian	2-(2-(18F)fluoropiridin-4-il)-9H-pirolo[2,3-b:4,5-c']dipiridină	Diagnosticul paraliziei supranucleare progresive
Slovak	2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrol[2,3-b:4,5-c']dipyridine	Diagnóza progresívnej supranukleárnej paralýzy
Slovenian	2-(2-(18F)fluoropiridin-4-il)-9H-pirolo[2,3-b:4,5-c']dipiridin	Diagnosticiranje progresivne supranuklearne paralize
Spanish	2-(2-(18F)fluoropiridin-4-il)-9H-pirrolo[2,3-b:4,5-c']dipiridina	Diagnóstico de parálisis supranuclear progresiva
Swedish	2-(2-(18F)fluorpyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridin	Diagnos av progressiv supranukleär pares
Norwegian	2-(2-(18F)fluorpyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridin	Diagnose av progressiv supranukleær parese
Icelandic	2-(2-(18F)flúorópýridín-4-ýl)-9H-pýrróló[2,3-b:4,5-c']dípýridín	Greining á ágengri ofankjarnalömun