



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Humanised IgG4 monoclonal antibody against extracellular tau for the treatment of progressive supranuclear palsy

On 28 July 2015, orphan designation (EU/3/15/1522) was granted by the European Commission to Bristol-Myers Squibb Pharma EEIG, United Kingdom, for humanised IgG4 monoclonal antibody against extracellular tau for the treatment of progressive supranuclear palsy.

#### What is progressive supranuclear palsy?

Progressive supranuclear palsy, which is also known as Steele-Richardson-Olszewski syndrome, is a rare disease that involves the gradual deterioration of brain cells. 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 progressive supranuclear palsy 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.

Progressive supranuclear palsy is a debilitating and life-threatening disease that leads to parkinsonism, paralysis and premature death.

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

At the time of designation, progressive supranuclear palsy affected approximately 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 72,000 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 satisfactory methods were authorised in the EU for the treatment of progressive supranuclear palsy. Because of their tendency to fall, patients were often offered walking

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\*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 512,900,000 (Eurostat 2015).



aids, as well as special glasses to help them to look down. Physiotherapy was used to keep the joints flexible. For patients unable to swallow, a feeding tube leading through the tummy to the stomach was used. Medicines developed to treat Parkinson's disease were also used in some patients, but their effect was usually limited, and did not last long.

### **How is this medicine expected to work?**

In patients with progressive supranuclear palsy, the tau protein has extra phosphate groups attached at multiple points (a process called phosphorylation); the changes to the protein cause it to fold wrongly and become tangled. This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to some of the tau protein and to neutralise it, thereby potentially reducing the symptoms of progressive supranuclear palsy.

### **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 progressive supranuclear palsy were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for progressive supranuclear palsy 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 18 June 2015 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Humanised IgG4 monoclonal antibody against extracellular tau	Treatment of progressive supranuclear palsy
Bulgarian	Хуманизирано IgG4 моноклонално антитяло насочено срещу екстрацелуларния тау	Лечение на прогресивна супрануклеарна парализа
Croatian	Humanizirano monoklonsko protutijelo IgG4 protiv izvanstaničnog proteina tau	Liječenje progresivne supranuklearne paralize
Czech	Humanizovaná IgG4 monoklonální protilátka proti extracelulárnímu tau proteinu	Léčba progresivní supranukleární paralýzy
Danish	Humaniseret monoklonalt IgG4-antistof rettet mod ekstracellulært tau	Behandling af progressiv, supranukleær parese
Dutch	Gehumaniseerd IgG4 monoklonaal antilichaam gericht tegen extracellulair tau	Behandeling van progressieve supranucleaire paralyse
Estonian	Ekstratsellulaarse tau vastu suunatud humaniseeritud IgG4 monoklonaalne antikeha	Progressiivse supranukleaarse halvatus ravi
Finnish	Ekstrasellulaarista tau-proteiinia vastaan suunnattu humanisoitu monoklonaalinen IgG4-luokan vasta-aine	Progressiivisen supranukleaarisen halvauksen hoito
French	Anticorps monoclonal humanisé de type IgG4 dirigé contre tau extracellulaire	Traitement de la paralysie supranucléaire progressive
German	Gegen extrazelluläres Tau gerichteter humanisierter IgG4 monoklonaler Antikörper	Behandlung der progressiven supranukleären Lähmung
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα IgG4 κατευθυνόμενο έναντι της εξωκυτταρικής πρωτεΐνης tau	Θεραπεία προϊούσας υπερπυρηνικής παράλυσης
Hungarian	Extracelluláris tau elleni humán IgG4 monoklonális antitest	Progresszív supranuclearis bénulás kezelése
Italian	Anticorpo monoclonale umanizzato IgG4 diretto contro il tau extracellulare	Trattamento della paralisi sopranucleare progressiva
Latvian	Humanizēta IgG4 monoklonālā anti viela pret ekstracelulāro tau	Progresējošās supranukleārās triekas ārstēšana
Lithuanian	Humanizuotas IgG4 monokloninis antikūnas prieš ekstraląstelinį tau	Progresuojančio supranuklearinio paralyžiaus gydymas
Maltese	Antikorp monoklonali IgG4 umanizzat dirett kontra tau ekstraċellulari	Kura ta' paralizi supranukleari progressiva
Polish	Humanizowane przeciwciało monoklonalne IgG4 skierowane przeciwko zewnątrzkomórkowemu białku tau	Leczenie postępującego porażenia nadjądrowego
Portuguese	Anticorpo monoclonal humanizado IgG4 contra a proteína tau extracelular	Tratamento da paralisia supranuclear progressiva
Romanian	Anticorp monoclonal umanizat IgG4 îndreptat împotriva proteinei tau extracelulare	Tratamentul paraliziei supra-nucleare progresive

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná monoklonálna protilátka IgG4 proti extracelulárnemu proteínu tau	Liečba progresívnej supranukleárnej paralýzy
Slovenian	Humanizirano monoklonsko protitelo IgG4 proti zunajceličnemu proteinu tau	Zdravljenje progresivne supranuklearne paralize
Spanish	Anticuerpo monoclonal humanizado IgG4 dirigido contra tau extracelular	Tratamiento de parálisis supranuclear progresiva
Swedish	Humaniserad monoklonal IgG4- antikropp riktad mot extracellulärt tau	Behandling av progressiv supranukleär pares
Norwegian	Humanisert monoklonalt IgG4-antistoff rettet mot ekstracellulært tau-protein	Behandling av progressiv supranukleær parese
Icelandic	Mannaðlagað IgG4 einstofna mótefni gegn utanfrumu tau	Meðferð við ágengri ofankjarnalömun