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EMA/COMP/241978/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised recombinant IgG4 anti-human tau antibody for the treatment of progressive supranuclear palsy

On 28 April 2016, orphan designation (EU/3/16/1649) was granted by the European Commission to Abbvie Ltd, United Kingdom, for humanised recombinant IgG4 anti-human tau antibody (also known as ABBV-8E12) 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 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 77,000 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

*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 513,700,000 (Eurostat 2016).

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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 inserted through the skin into the stomach was used. Medicines developed to treat Parkinson's disease were also used in some patients, but their effect was usually limited and short-lasting.

How is this medicine expected to work?

In patients with progressive supranuclear palsy, the tau protein folds wrongly and becomes tangled. The abnormally folded proteins can spread between brain cells, causing other tau proteins to become tangled. This medicine is a monoclonal antibody (a type of protein) that has been designed to attach to the abnormal tau protein found outside cells, thereby reducing the spread of tangles to other cells and potentially reducing the symptoms of progressive supranuclear palsy.

What is the stage of development of this medicine?

The effects of this 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. Orphan designation of the medicine 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 23 March 2016 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	Humanised recombinant IgG4 anti-human tau antibody	Treatment of progressive supranuclear palsy
Bulgarian	Рекомбинантно хуманизирано IgG4 антиантитяло насочено срещу човешкия тау-протеин	Лечение на прогресивна супрануклеарна парализа
Croatian	Humanizirano rekombinantno IgG4 anti-humano tau antitijelo	Liječenje progresivne supranuklearne paralize
Czech	Humanizová rekombinantní protilátka IgG4 vůčitau proteinu	Léčba progresivní supranukleární paralýzy
Danish	Humaniseret rekombinant IgG4 anti humant tau antistof	Behandling af progressiv, supranukleær parese
Dutch	Gehumaniseerd recombinant IgG4 anti-humaan tau antilichaam	Behandeling van progressieve supranucleaire paralyse
Estonian	Rekombinantne humaniseeritud IgG4 inimese tau(-valgu) vastane antikeha	Progressiivse supranukleaarse halvatuse ravi
Finnish	Tau-proteiinia vastaan suunnattu humanisoitu monoklonaalinen IgG4-luokan vasta-aine	Progressiivisen supranukleaarisen halvauksen hoito
French	Anticorps humanisé recombinant IgG4 anti-Tau humaine	Traitement de la paralysie supranucléaire progressive
German	Humanisierter rekombinanter IgG4-Anti-Human-Tau-Antikörper	Behandlung der progressiven supranukleären Lähmung
Greek	Ανθρωποποιημένο ανασυνδυασμένο IgG4 αντίσωμα έναντι της ανθρώπινης πρωτεΐνης tau	Θεραπεία προϊούσας υπερπυρηνικής παράλυσης
Hungarian	Humanizált rekombináns IgG4 humán tau-ellenes antitest	Progresszív supranuclearis bénulás kezelése
Italian	Anticorpo IgG4 umanizzato ricombinante anti-tau umana	Trattamento della paralisi sopranucleare progressiva
Latvian	Rekombinēta humanizēta IgG4 antiviela pret cilvēka Tau	Progresējošās supranukleārās triekas ārstēšana
Lithuanian	Humanizuotas rekombinantinis IgG4 prieš žmogaus tau antikūną	Progresuojančio supranuklearinio paralyžiaus gydymas
Maltese	Antikorp IgG4 kontra tau uman, rikombinanti umanizzat	Kura ta' paraliżi supranukleari progressiva
Polish	Humanizowane rekombinowane przeciwciało klasy IgG4 przeciwko ludzkiemu tau	Leczenie postępującego porażenia nadjądrowego
Portuguese	Anticorpo humanizado recombinante IgG4 anti-tau humana	Tratamento da paralisia supranuclear progressiva
Romanian	Anticorp IgG4 umanizat recombinant anti-tau uman	Tratamentul paraliziei supra-nucleare progresive
Slovak	Humanizovaná rekombinantná antihumánna IgG4 tau protilátka	Liečba progresívnej supranukleárnej paralýzy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humanizirano rekombinantno protitelo IgG4 proti beljakovini tau	Zdravljenje progresivne supranuklearne paralize
Spanish	Anticuerpo IgG4 humanizado recombinante anti – tau humano	Tratamiento de parálisis supranuclear progresiva
Swedish	Humaniserad rekombinant IgG4 anti-human tau-antikropp	Behandling av progressiv supranukleär pares
Norwegian	Humanisert rekombinant IgG4 anti-human tau antistoff	Behandling av progressiv supranukleær parese
Icelandic	Mannaaðlagað raðbrigða IgG4 mótefni gegn manna tau	Meðferð við ágengri ofankjarnalömun