



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Davunetide for the treatment of progressive supranuclear palsy

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2013 on request of the Sponsor.

On 23 March 2010, orphan designation (EU/3/10/728) was granted by the European Commission to FGK Representative Service GmbH, Germany, for davunetide for the treatment 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), difficulty swallowing, personality changes and dementia (loss of intellectual function). The disease usually starts in people aged over 40 years and gradually gets worse over a number of years.

PSP is a debilitating and life-threatening disease that leads to a progressive inability to move and poor long-term survival.



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

At the time of designation, PSP affected approximately 1 in 10,000 people in the European Union (EU). This is equivalent to a total of around 51,000 people^{*}, and is below the threshold 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 PSP. Because of their tendency to fall, patients were often offered walking 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 PSP patients, but their effect was usually limited, and did not last long.

How is this medicine expected to work?

PSP is thought to be caused by proteins called tau sticking together and forming tangled filaments. These filaments build up in brain cells, damaging various areas of the brain. The way that davunetide works in PSP is not fully understood, but it is thought to prevent tau sticking together, reducing the damage to brain cells.

What is the stage of development of this medicine?

The effects of davunetide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with davunetide had been started in patients with PSP.

At the time of submission, davunetide was not authorised anywhere in the EU for PSP 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 6 January 2010 recommending the granting of this designation.

^{*}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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Davunetide	Treatment of progressive supranuclear palsy
Bulgarian	давунетид	Лечение на прогресивна супрануклеарна парализа
Czech	Davunetid	Léčba progresivní supranukleární paralýzy
Danish	Davunetid	Behandling af progressiv, supranuklear lammelse
Dutch	Davunetide	Behandeling van progressieve supranucleaire paralyse
Estonian	Davunetiid	Progressiivse supranukleaarse halvatus ravi
Finnish	Davunetidi	Progressiivisen supranukleaarisen halvauksen hoito
French	Davunétide	Traitement de la paralysie supranucléaire progressive
German	Davunetide	Behandlung der progressiven supranukleären Lähmung
Greek	Δαβουνετίδη	Θεραπεία προϊούσας υπερπυρηνικής παράλυσης
Hungarian	Davunetide	Progresszív supranuclearis bénulás kezelése
Italian	Davunetide	Trattamento della paralisi sopranucleare progressiva
Latvian	Davunetīds	Progresējošās supranukleārās triekas ārstēšana
Lithuanian	Davunetidas	Progresuojančio supranuklearinio paralyžiaus gydymas
Maltese	Davunetide	Kura ta' paralizi supranukleari progressiva
Polish	dawunetyd	Leczenie postępującego porażenia nadjądrowego
Portuguese	Davunetide	Tratamento da paralisia supranuclear progressiva
Romanian	Davunetide	Tratamentul paraliziei supra-nucleare progresive
Slovak	Davunetid	Liečba progresívnej supranukleárnej paralýzy
Slovenian	Davunetid	Zdravljenje progresivne supranuklearne paralize
Spanish	Davunetida	Tratamiento de parálisis supranuclear progresiva
Swedish	Davunetid	Behandling av progressiv supranukleär pares
Norwegian	Davunetid	Behandling av progressiv supranukleær parese
Icelandic	Davúnetíð	Meðferð við ágengri ofankjarnalömun

¹ At the time of designation