10 May 2021
EMADOC-628903358-3732

Public summary of opinion on orphan designation

(S)-N-(5-(4-(1-(Benzo[d][1,3]dioxol-5-yl)ethyl)piperazin-1-yl)-1,3,4-thiadiazol-2-yl)acetamide, hydrochloride salt for the treatment of progressive supranuclear palsy

On 9 December 2020, orphan designation EU/3/20/2381 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for (S)-N-(5-(4-(1-(benzo[d][1,3]dioxol-5-yl)ethyl)piperazin-1-yl)-1,3,4-thiadiazol-2-yl)acetamide, hydrochloride salt (also known as ASN90) 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 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 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.

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 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 31,000 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).

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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 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 to the stomach was used. Medicines developed to treat Parkinson's disease were also used in some patients.

How is this medicine expected to work?

In patients with progressive supranuclear palsy, changes to the tau protein cause it to fold abnormally and become tangled. One of these changes is the removal of carbohydrate groups from the protein. The medicine is expected to work by preventing the removal of these groups, which will in turn prevent the tau proteins from folding incorrectly. This is expected to reduce the deterioration of brain tissue and slow down or reduce the symptoms of the disease.

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, no clinical trials with the medicine in patients with progressive supranuclear palsy had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of progressive supranuclear palsy. Orphan designation had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 November 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.