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Update as of 7 August 2025:

The company for Nurzigma has requested a re-examination of EMA's July 2025 opinion. Upon receipt of the grounds of this request, the Agency will re-examine its opinion and issue a final recommendation.

Refusal of the marketing authorisation for Nurzigma (pridopidine)

The European Medicines Agency has recommended the refusal of the marketing authorisation for Nurzigma, a medicine intended for the treatment of adults with Huntington's disease.

Huntington's disease is an inherited condition that worsens over time and causes brain cells to die. This leads to problems with movement, cognition (perception, awareness, thinking and judgement) and mental health.

The Agency issued its opinion on 24 July 2025. The company that applied for authorisation, Prilenia Therapeutics B.V., may ask for re-examination of the opinion within 15 days of receiving the opinion.

What is Nurzigma and what was it intended to be used for?

Nurzigma was developed as a medicine for the treatment of adults with Huntington's disease. During the assessment, the company proposed to restrict the indication to adults with early Huntington's disease who are not treated with medicines known as antidopaminergic medicines. These antidopaminergic medicines are commonly used in patients with Huntington's disease to treat chorea (jerky and involuntary movements) and behavioural symptoms (such as aggression).

Nurzigma contains the active substance pridopidine and was to be available as hard capsules.

Nurzigma was designated an 'orphan medicine' (a medicine used in rare diseases) on 20 June 2005 for the treatment of Huntington's disease. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu-3-05-288.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

How does Nurzigma work?

The active substance in Nurzigma, pridopidine, activates a protein called sigma-1 receptor (S1R). S1R is found inside the cells and is involved in cellular processes that contribute to the health and survival of nerve cells. By activating S1R, pridopidine was expected to improve cellular processes that are involved in nerve cell damage and Huntington's disease.

What did the company present to support its application?

The company presented results from a main study involving 499 adults aged 25 years and older with early Huntington's disease. Patients in the study were given either Nurzigma or placebo (a dummy treatment). The main measure of effectiveness was the change in the total functional capacity (TFC) score after 65 weeks of treatment. The TFC score measures how well a person with diseases that affect the nervous system can perform daily tasks and activities. The company also presented results from analyses in a subgroup of 208 patients from the main study, namely adults with early Huntington's disease who were not treated with antidopaminergic medicines. In addition, the company presented results from 3 supportive studies in adults with Huntington's disease.

What were the main reasons for refusing the marketing authorisation?

The Agency considered that the main study and the supportive studies failed to provide evidence that Nurzigma is effective in patients with early Huntington's disease. The Agency noted that the validity and relevance of the results of the analyses in the subgroup of patients (adults with early Huntington's disease who were not treated with antidopaminergic medicines) from the main study have not been demonstrated.

Therefore, the Agency's opinion was that the effectiveness of Nurzigma had not been demonstrated. Although the company applied for a conditional marketing authorisation, the medicine did not meet the criteria for granting this type of authorisation. As a result, the Agency recommended refusing the conditional marketing authorisation.

Does this refusal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes with Nurzigma.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your clinical trial doctor.