

11 July 2017
EMA/312774/2017

Public summary of opinion on orphan designation

Pentamer formyl thiophene acetic acid for the treatment of Creutzfeldt-Jakob disease

On 20 June 2017, orphan designation (EU/3/17/1883) was granted by the European Commission to NeuroScios GmbH, Austria, for pentamer formyl thiophene acetic acid (also known as NSC500) for the treatment of Creutzfeldt-Jakob disease.

What is Creutzfeldt-Jakob disease?

Creutzfeldt-Jakob disease is a disease of the brain which causes worsening dementia, difficulty with movement and problems with vision. It is caused by the incorrect folding of a protein known as a prion. The incorrect folding ('misfolding') makes the prion proteins clump together and build up in brain cells, damaging the cells.

Early symptoms of the disease include failing memory, behaviour changes, inability to control muscles (lack of coordination) and changes in sight. As the disease worsens, the patient may develop uncontrolled movements, blindness, weakness in the arms and legs and fall into a coma. Patients with the disease usually die within a year.

Creutzfeldt-Jakob disease is a fatal disease which is seriously debilitating because of the rapid progression of brain damage with loss of mental function, problems of coordination, personality changes and failing sight.

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

At the time of designation, Creutzfeldt-Jakob disease affected less than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 5,000 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).

^{*}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 515,700,000 (Eurostat 2017).

What treatments are available?

No satisfactory methods were authorised for the treatment of Creutzfeldt-Jakob disease at the time of application. Treatment was aimed at reducing pain and other symptoms and making the patient as comfortable as possible.

How is this medicine expected to work?

This medicine attaches to the misfolded prion protein in brain cells and interferes with the way these proteins clump together. It is expected that, by interfering with this clumping, the medicine makes the abnormal prion proteins more soluble and less damaging to brain cells. This is expected to slow down the progression of Creutzfeldt-Jakob disease and so delay the symptoms from worsening.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with Creutzfeldt-Jakob disease had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for Creutzfeldt-Jakob disease 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 12 May 2017 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

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pentamer formyl thiophene acetic acid	Treatment of Creutzfeldt-Jakob disease
Bulgarian	Пентамер формил тиофен оцетна киселина	Лечение на болест на Кройцфелд-Якоб
Croatian	Pentamer formil tiofen octena kiselina	Liječenje Creutzfeld-Jakobove bolesti
Czech	Pentamer formyl thiophen kyseliny octové	Léčba Kreuzfeld- Jakobovy choroby
Danish	Pentamerformylthiophen eddikesyre	Behandling af Creutzfeldt-Jakobs sygdom
Dutch	Pentameer formylthiofeenacetaat	Behandeling van Creutzfeldt-Jakob ziekte
Estonian	Pentameerne formüültiofeenäädikhape	Creutzfeldt-Jakobi haiguse ravi
Finnish	Tiofeeni etikkahapon pentameeri muoto	Creutzfeldt-Jakobin taudin hoito
French	Pentamère formyle acide acétique thiophène	Traitement de la maladie de Creutzfeldt-Jakob
German	Pentamer Formyl Thiophen Acetylsäure	Behandlung der Creutzfeldt-Jakob-Krankheit
Greek	Πενταμερές φορμυλο-θειοφαινοξικού οξέος	Θεραπεία της νόσου Creutzfeldt-Jakob
Hungarian	Pentamer formil tiofén ecetsav	Creutzfeldt-Jakob betegség kezelése
Italian	Pentamero formil tiofene acido acetico	Trattamento della malattia di Creutzfeld-Jakob
Latvian	Formiltiofēna etiķskābes pentamērs	Kreicfelda–Jakoba slimības ārstēšana
Lithuanian	Pentamero formiltiofeno acto rūgštis	<i>Creutzfeldt-Jakob ligos gydymas</i>
Maltese	Aċidu aċetiku Pentamer formil tijofen	Kura tal-marda Creutzfeldt-Jakob
Polish	Pentamer formylo tiofenu kwasu octowego	Leczenie choroby Creutzfelda-Jakoba
Portuguese	Pentâmero formiltiofeno ácido acético	Pentâmero formiltiofeno ácido acético
Romanian	Pentamer formil tiofen acid acetic	Tratamentul bolii Creutzfeldt-Jakob
Slovak	Pentamérformyltiofén octová kyselina	Liečba Creutzfeldt-Jakobovej choroby
Slovenian	Pentamer formil tiofen očetne kisline	Zdravljenje Creutzfeldt Jakobsove bolezni
Spanish	Ácido acético de pentâmero formiltiofeno	Tratamiento de la enfermedad de Creutzfeldt-Jakob
Swedish	Pentameros formyl thiophene eddiksyr	Behandling av Creutzfeldt-Jakobs sjukdom
Norwegian	Pentamer formyltiofeneddiksyr	Behandling av Creutzfeldt-Jakobs sykdom
Icelandic	Pentameros formýl tíófen edikssýra	Meðferð Creutzfeldt-Jakob sjúkdóms

¹ At the time of designation