15 January 2019
EMA/776496/2018

Public summary of opinion on orphan designation

Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp for the treatment of multiple system atrophy

On 19 November 2018, orphan designation (EU/3/18/2095) was granted by the European Commission to United Neuroscience Limited, Ireland, for Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp (also known as UB-312) for the treatment of multiple system atrophy.

What is multiple system atrophy?

Multiple system atrophy is a progressive disease of the nervous system, where nerve cells in certain areas of the brain and spinal cord gradually deteriorate, causing loss of voluntary and involuntary muscle function. This leads to symptoms such as loss of bladder control as well as shaking, rigidity and loss of muscle coordination, light-headedness due to excessive drop in blood pressure when standing up, and difficulties with speech and breathing. Some of these features are similar to those seen in Parkinson's disease, which makes it hard to distinguish the two disorders in the early stages of the disease.

Multiple system atrophy is a long-term debilitating and life-threatening disease because of the gradual loss of muscle function and its effects on muscles used for breathing.

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

At the time of designation, multiple system atrophy affected less than 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 16,000 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 517,400,000 (Eurostat 2018).

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What treatments are available?

At the time of designation, there were no satisfactory methods authorised in the EU for the treatment of multiple system atrophy. Different treatments were used to relieve the symptoms of the disease, such as beta blockers and vasopressin for the treatment of hypotension (low blood pressure) and anticholinergic medicines to treat bladder problems. Parkinson medicines such as levodopa are not effective in treating the Parkinson-type symptoms of multiple system atrophy.

How is this medicine expected to work?

In patients with multiple system atrophy, a protein called alpha-synuclein does not work properly and builds up in the brain and spinal cord forming deposits and damaging nerve cells.

This medicine is intended to work as a vaccine and stimulate the immune system (the body's natural defences) to produce antibodies against alpha-synuclein deposits, so that the deposits are broken down and cleared from the body. This is expected to improve 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 multiple system atrophy had yet been started.

At the time of submission, the medicine was not authorised anywhere in the EU for multiple system atrophy 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 18 October 2018 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	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Treatment of multiple system atrophy
Bulgarian	Иле-Сер-Иле-Тре-Глу-Иле-Лиз-Гли-Вал-Иле-Вал-Хис-Арг-Иле-Глу-Тре-Иле-Лев-Фен-Лиз-Лиз-Лиз-Лиз-Глу-Мет-Про-Сер–Глу-Глу-Гли-Тир-Глн-Асп	Лечение на мултисистемна атрофия
Croatian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Liječenje multisistemne atrofije
Czech	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Léčba multisystémové atrofie
Danish	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Behandling af multipel systematrofi
Dutch	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Behandeling van multisysteematrofie
Estonian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Multisüsteemse atroofia ravi
Finnish	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Monijärjestelmäsurkastuman hoito.
French	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Traitement de l'atrophie multisystématisée.
German	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Behandlung einer Multisystematrophie
Greek	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Θεραπεία ατροφίας πολλαπλών συστημάτων
Hungarian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Multi-szisztémás atrófia kezelése
Italian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Trattamento dell'atrofia multisistemica
Latvian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Multisistēmas atrofijas ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Daugiasisteminės atrofijos gydymas
Maltese	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Kura tal-atrofija ta' sistemi multipli
Polish	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Tratamento da atrofia multisistémica
Portuguese	Ile-Ser-Ile-Thr-Glu-Ile-Lis-Gli-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Fen-Lis-Lis-Lis-Lis-Glu-Met-Pro-Ser-Glu-Glu-Gli-Tir-Gln-Asp	Tratamento da atrofia de múltiplos sistemas
Romanian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Tratamentul atrofiei sistemice multiple
Slovak	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Liečba multisystémovej atrofie.
Slovenian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Zdravljenje multiple sistemske atrofije
Spanish	Ile-Ser-Ile-Tr-Glu-Ile-Lis-Gli-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Fe-Lis-Lis-Lis-Lis-Glu-Met-Pro-Ser-Glu-Glu-Gli-Tir-Gln-Asp	Tratamiento de la atrofia multisistémica
Swedish	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Behandling av multipel systematrofi
Norwegian	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Behandling ved multippel systematrofi (MSA)
Icelandic	Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp	Meðferð á fjölkerfarýrnun