



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 September 2019
EMADOC-628903358-1037

Public summary of opinion on orphan designation

Rasagiline for the treatment of Duchenne muscular dystrophy

On 28 June 2019, orphan designation EU/3/19/2174 was granted by the European Commission to TMC Pharma (EU) Limited, Ireland, for rasagiline for the treatment of Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is a genetic disease that gradually causes inflammation, weakness and atrophy (wasting) of muscles. It mainly affects boys, and usually starts before the age of 6 years. The muscle weakness usually starts in the hips and legs, before affecting the arms, chest and the heart. Patients with DMD lack normal dystrophin, a protein found in muscles. Because this protein helps to protect muscles from injury as muscles contract and relax, in patients with DMD the muscles become weaker and eventually stop working.

DMD causes long-term disability and is life threatening because of its effects on the heart and the respiratory muscles (muscles that are used to breathe). The disease usually leads to death in early adulthood.

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

At the time of designation, DMD affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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).

What treatments are available?

At the time of designation, the medicine Translarna (ataluren) was authorised in the EU for the treatment of a small group of patients with DMD caused by a particular type of mutation (change), called a nonsense mutation, in the dystrophin gene. Patients also received supportive treatment such as physiotherapy.

*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 518,400,000 (Eurostat 2019).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for a wider patient population than the currently authorised treatment. This assumption is based on data from studies in the laboratory and will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Rasagiline is a 'monoamine-oxidase-B inhibitor', which means that it blocks the enzyme monoamine oxidase type B. This enzyme is thought to contribute to the inflammation and loss of muscle function seen in patients with DMD. By blocking the enzyme, the medicine is expected to improve muscle strength and other symptoms of the disease.

What is the stage of development of this medicine?

The effects of rasagiline have been evaluated in experimental models.

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

Rasagiline has been authorised in the EU since 2005 for the treatment of Parkinson's disease.

At the time of submission, rasagiline was not authorised anywhere in the EU for the treatment of DMD or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a Positive opinion on 23 May 2019, 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](#).

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	Rasagiline	Treatment of Duchenne muscular dystrophy
Bulgarian	Разагилин	Лечение на мускулна дистрофия на Duchenne
Croatian	Razagilin	Liječenje Duchenneove mišićne distrofije
Czech	Rasagilin	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Rasagilin	Behandling af Duchenne muskeldystrofi
Dutch	Rasagiline	Behandeling van Duchenne spierdystrofie
Estonian	Rasagiliin	Duchenne'i lihasdüstroofia ravi
Finnish	Rasagiliini	Duchennen lihasdystrofian hoito
French	Rasagiline	Traitement de la dystrophie musculaire de Duchenne
German	Rasagilin	Behandlung der Duchenne-Muskeldystrophie
Greek	Ρασαγιλίνη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Razagilin	Duchenne dystrophia kezelése
Italian	Rasagilina	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Rasagilīns	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Razagilinas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Rasagiline	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Rasagilina	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Rasagilina	Tratamento da distrofia muscular de Duchenne
Romanian	Rasagilină	Tratamentul distrofiei musculare Duchenne
Slovak	Rasagilín	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Razagilin	Zdravljenje Duchennove mišične distrofije
Spanish	Rasagilina	Tratamiento de la distrofia muscular de Duchenne
Swedish	Rasagilin	Behandling av Duchennes muskeldystrofi
Norwegian	Rasagilin	Behandling av Duchennes muskeldystrofi
Icelandic	Rasagílín	Meðferð á Duchenne vöðvarýrnun

¹ At the time of designation