



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

24 February 2020  
EMADOC-628903358-1670

## Public summary of opinion on orphan designation

### Pamrevlumab for the treatment of Duchenne muscular dystrophy

On 16 December 2019, orphan designation EU/3/19/2234 was granted by the European Commission to Voisin Consulting S.A.R.L., France, for pamrevlumab 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, Duchenne muscular dystrophy affected approximately 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 41,000 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, who can walk. Patients also received supportive treatment such as physiotherapy.

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\*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 patients with Duchenne muscular dystrophy. This is because preliminary studies indicate that the medicine improves heart and lung function in some patients for whom no treatment is authorised.

This assumption 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?**

Patients with Duchenne muscular dystrophy have higher than normal levels of CTGF, a protein involved in several processes in the body, including the formation of scar tissue.

Pamrevlumab is an antibody (a type of protein) designed to target and attach to CTGF. By attaching to CTGF, it is expected to prevent the formation of scar tissue in muscles, including those of the heart. This is expected to improve symptoms of the disease.

## **What is the stage of development of this medicine?**

The effects of pamrevlumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pamrevlumab in patients with Duchenne muscular dystrophy were ongoing.

At the time of submission, pamrevlumab was not authorised anywhere in the EU for the treatment of Duchenne muscular dystrophy. Orphan designation had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 7 November 2019, 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](#).

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<sup>1</sup>, Norwegian and Icelandic

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

<sup>1</sup> At the time of designation