19 May 2015
EMA/COMP/211156/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rimeporide for the treatment of Duchenne muscular dystrophy

On 24 April 2015, orphan designation (EU/3/15/1478) was granted by the European Commission to EUDRAC Limited, United Kingdom, for rimeporide for the treatment of Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is a genetic disease that gradually causes weakness and atrophy (wasting) of the muscles. It mainly affects boys, and is usually diagnosed before the age of six 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 adolescence or 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, Translarna (ataluren) was authorised in the EU to treat patients with DMD who have certain mutations called 'stop-codon nonsense mutations' in their dystrophin gene.

The sponsor has provided sufficient information to show that rimeporide might be of significant benefit for patients with DMD; early studies in experimental models indicate that the medicine could lead to

*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 512,900,000 (Eurostat 2015).

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improved outcomes, which will not be limited to patients with a specific mutation and will therefore target a wider patient population. 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?

In DMD patients, because of the lack of dystrophin, muscle cells often have high sodium and calcium levels and high pH, which eventually damage cells. Rimeporide is a small molecule that blocks an enzyme on the surface of muscle cells called sodium-proton exchanger type 1 (NHE-1). This is expected to lead to a reduction in sodium and calcium levels and of the pH, thereby reducing damage to muscle cells.

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 rimeporide in experimental models was ongoing.

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

At the time of submission, rimeporide was not authorised anywhere in the EU for DMD 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 19 March 2015 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:

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

[1] At the time of designation