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Public summary of opinion on orphan designation

Ex vivo fused normal allogeneic human myoblast with autologous human myoblast derived from Duchenne muscular dystrophy affected donor for the treatment of Duchenne muscular dystrophy

On 19 November 2018, orphan designation (EU/3/18/2089) was granted by the European Commission to Dystrogen Therapeutics S.A., Poland, for ex vivo fused normal allogeneic human myoblast with autologous human myoblast derived from Duchenne muscular dystrophy affected donor 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 muscles. It mainly affects boys, and usually starts 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 early adulthood.

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

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

*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).

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.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with DMD because early laboratory data indicate that the medicine may benefit a larger number of patients than the currently authorised medicine. 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?

The medicine is made from the patient's own muscle cells and muscle cells from a healthy donor, preferably a relative of the patient, that are 'fused' (combined) together. When the fused cells are injected into the muscle or bone of the patient, they are expected to make normal dystrophin, the protein missing in DMD patients. This is expected to improve the symptoms of the disease.

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 DMD had been started.

At the time of submission, the medicine 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 18 October 2018 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	Ex vivo fused normal allogeneic human myoblast with autologous human myoblast derived from Duchenne muscular dystrophy affected donor	Treatment of Duchenne muscular dystrophy
Bulgarian	Ex vivo слят нормален алогенен човешки миобласт с аутоложен човешки миобласт, получен от донор засегнат от мускулна дистрофия на Duchenne	Лечение на мускулна дистрофия на Duchenne
Croatian	Ex vivo spojeni normalni alogeni humani mioblast s autognim humanim mioblastom dobivenim od donora oboljelog od Duchenneove mišićne distrofije	Liječenje Duchenneove mišićne distrofije
Czech	Ex vivo fúzované normální alogenní lidské myoblasty s autologním humánním myoblastem získaným z svalové dystrofie Duchenne postiženého dárce	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Normal allogene human myoblast kooondenseret ex vivo med autolog human myoblast afledt af Duchenne muskeldystrofi påvirket donor	Behandling af Duchenne muskeldystrofi
Dutch	Ex vivo gefuseerde normale allogene menselijke myoblast met autologe humane myoblast afgeleid van de door Duchenne spierdystrofie aangetaste donor	Behandeling van Duchenne spierdystrofie
Estonian	Normaalne allogeenne inimese müoblast, mis on kehaväliselt fuseeritud inimese autoloogse müoblastiga, mis pärineb Duchenne'i lihasdüstroofiast põdevalt doonorilt	Duchenne'i lihasdüstroofia ravi
Finnish	Ex vivo-fuusioitu normaali allogeenininen ihmisen myoblasti yhdessä autologisen ihmisen myoblastin kanssa, joka on peräisin Duchennen lihasdystrofiaa sairastavalta luovuttajalta	Duchennen lihasdystrofian hoito
French	Myoblaste humain allogénique normal fusionné ex vivo avec un autre myoblaste humain autologue dérivé d'un donneur affecté de la dystrophie musculaire de Duchenne	Traitement de la dystrophie musculaire de Duchenne
German	Ex vivo fusionierter normaler allogener humaner Myoblasten mit autologem humanem Myoblasten, abgeleitet von Duchenne-Muskeldystrophie betroffenen Spender	Behandlung der Duchenne-Muskeldystrophie
Greek	Ex vivo συντηγμένη φυσιολογική αλλογενή ανθρώπινη μυοβλάστη με αυτόλογη ανθρώπινη μυοβλάστη που προέρχεται από δότη προσβεβλημένο από μυϊκή δυστροφία Duchenne	Θεραπεία της μυϊκής δυστροφίας Duchenne

¹ At the time of designation

Language	Active ingredient	Indication
Hungarian	Ex vivo fuzionált normál allogén humán myoblaszt egy Duchenne izomsorvadással érintett donortól származó autológ humán myoblasztal	Duchenne dystrophia kezelése
Italian	Mioblasto umano allogenico normale fuso ex vivo con mioblasto umano autologo derivato da donatore affetto da distrofia muscolare di Duchenne	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	<i>Ex vivo</i> sapludināts normāls alogēns cilvēka mioblasts ar autologu cilvēka mioblastu, kas iegūts donora ar Dišēna muskuļu distrofiju	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	<i>Ex vivo</i> sulietas normalus alogeninis žmogaus mioblastas su autologiniu žmogaus mioblastu, išskirtu iš <i>Duchenne</i> raumenų distrofija sergančio donoro	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Mijoblast uman alloġeniku normali ex vivo mdewweb ma' mijoblast uman awtologu derivat minn donatur affettwat mid-distrofija muskolari Duchenne	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Ex vivo zfuzjowany normalny allogeniczny ludzki mioblast z autologicznym ludzkim mioblastem pochodzącym od dawcy z dystrofią mięśniową Duchenne'a	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Mioblasto humano alogénico normal fundido <i>ex vivo</i> com mioblastos autólogos humanos de dador afetado pela distrofia muscular de Duchenne	Tratamento da distrofia muscular de Duchenne
Romanian	Mioblast uman normal allogenic fuzionat ex vivo cu un myoblast uman autolog provenit de la un donator afectat de distofia musculară Duchenne	Tratamentul distrofiei musculare Duchenne
Slovak	Ex vivo fúzovaný normálny alogénny ľudský myoblast s autológny m humánnym myoblastom pochádzajúcim z darcu postihnutého Duchenneho svalovou dystrofiou	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Normalen alogenski človeški mioblast, ex vivo spojen z avtolognim človeškim mioblastom darovalca z Duchennovo mišično distrofijo	Zdravljenje Duchennove mišične distrofije
Spanish	Mioblastos humanos alogénicos normales fusionados ex vivo con mioblastos autólogos humanos derivados de donantes afectados por distrofia muscular de Duchenne	Tratamiento de la distrofia muscular de Duchenne
Swedish	Ex vivo fuserad normal allogen human myoblast med autolog human myoblast härrörande från Duchennes muskeldystrofipåverkad givare	Behandling av Duchennes muskeldystrofi
Norwegian	Normal allogen human myoblast fusjonert ex vivo med autolog human myoblast fra donor affisert av Duchennes muskeldystrofi	Behandling av Duchennes muskeldystrofi

Language	Active ingredient	Indication
Icelandic	Ex vivo samtengd eðlileg, ósamgena mannavöðvakímfruma með samgena mannavöðvakímfrumu afleiddri frá gjafa með Duchenne vöðvarýrnun	Meðferð á Duchenne vöðvarýrnun