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Public summary of opinion on orphan designation

Ex-vivo fused autologous human bone marrow-derived mesenchymal stem cell with allogenic human myoblast for the treatment of Duchenne muscular dystrophy

On 31 July 2018, orphan designation (EU/3/18/2045) was granted by the European Commission to Dystrogen Therapeutics S.A., Poland, for ex-vivo fused autologous human bone marrow-derived mesenchymal stem cell with allogenic human myoblast 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 approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of 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).

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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 it improves levels of dystrophin and muscle function. 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?

This medicine is prepared individually for patients with DMD by mixing in the laboratory certain stem cells (cells that can develop into different types of cells) from the patient with muscle stem cells from a relative that contain dystrophin. When given back to the patient, the cells are expected to become part of the muscle and produce the dystrophin protein, restoring the levels of the missing protein. This is expected to slow down the worsening 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 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 21 June 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	Activeingredient	Indication
English	Ex-vivo fused autologous human bone marrow-derived mesenchymal stem cell with allogenic human myoblast	Treatment of Duchenne muscular dystrophy
Bulgarian	Ex-vivo автоложна човешка костно-мозъчна мезенхимална стволова клетка, свързана с алогенен човешки миобласт	Лечение на мускулна дистрофия на Duchenne
Croatian	Ex-vivo spojena autologna mezenhimna matična stanica iz ljudske koštane srži s alogenim humanim mioblastom	Liječenje Duchenneove mišićne distrofije
Czech	Autologní mezenchymální kmenová buňka lidské kostní dřeně fuzovaná ex vivo s alogenním humánním myoblastem	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Ex-vivo kondenseret autolog humant knoglemarv-afledt mesenkymal stamcelle med allogen human myoblast	Behandling af Duchenne muskeldystrofi
Dutch	Ex-vivo gefuseerde autologe humane beenmerg afgeleide mesenchymale stamcel met allogene menselijke myoblast	Behandeling van Duchenne spierdystrofie
Estonian	Kehaväliselt kokku liidetud inimese autoloogsed luuüdist pärinevad mesenhümaalsed tüvirakud ja inimese allogeensed müoblastid	Duchenne'i lihasdüstroofia ravi
Finnish	Ex vivo fuusioitu autologinen, ihmisen luuydinperäinen mesenkymaalinen kantasolu yhdessä ihmisen allogeenisen myoblastin kanssa	Duchennen lihasdystrofian hoito
French	Cellule souche mésenchymateuse dérivée de moelle osseuse humaine autologue fusionnée ex vivo avec myoblaste humain allogénique	Traitement de la dystrophie musculaire de Duchenne
German	Autologe humane mesenchymale Stammzellen aus dem Knochenmark, ex-vivo fusioniert mit allogenem humanem Myoblasten	Behandlung der Duchenne-Muskeldystrophie
Greek	Ex-vivo συγχωνευμένο αυτόλογο ανθρώπινο μεσεγχυματικό βλαστικό κύτταρο προερχόμενο απο μυελό των οστών, με αλλογενή ανθρώπινη μυοβλάστη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Ex-vivo fuzionált autológ humán csontvelő eredetű mesenchymális őssejt allogén humán myoblaszttal	Duchenne dystrophia kezelése
Italian	Cellule staminali mesenchimali derivate da midollo osseo autologo umano fuse ex-vivo con mioblasti umani allogenici	Trattamento della distrofia muscolare di tipo Duchenne

[1] At the time of designation

Language	Activeingredient	Indication
Latvian	Ar alogēniem cilvēka mioblastiem *ex-vivo* sapludinātas autologas no cilvēka kaulu smadzenēm iegūtas mezenhīmas cilmes šūnas	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Mezenchimos kamieninės ląstelės, išskirtos iš autologinių žmogaus kaulų čiulpų, sulietos su alogeniniu žmogaus mioblastu *ex-vivo*	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Ċellola staminali mesenċimali derivata mill-mudullun tal-bniedem awtologa fuża ex-vivo b'mijoblast allogeniku tal-bniedem	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Autologiczna ludzka mezenchymalna komórka macierzysta pochodząca ze szpiku kostnego fuzjowana ex vivo z allogenicznym ludzkim mioblastem	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Células estaminais mesenquimais autólogas de medula óssea humana fundidas ex-vivo com mioblasto humano alogénico	Tratamento da distrofia muscular de Duchenne
Romanian	Celule stem mezenchimale derivate din măduva osoasă umană autologă fuzionată ex vivo cu mioblaşti umani alogenici	Tratamentul distrofiei musculare Duchenne
Slovak	Ex vivo fúzovaná autológna mezenchýmová kmeňová bunka s alogénnym humánnym myoblastom z ľudskej kostnej drene	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Avtologna človeška matična celica kostnega mozga, ex-vivo združena z alogenim humanim mioblastom	Zdravljenje Duchennove mišične distrofije
Spanish	Células madre mesenquimales derivadas de la médula ósea autóloga ex vivo fusionada con mioblasto humano alogénico	Tratamiento de la distrofia muscular de Duchenne
Swedish	Ex-vivo kondenserad autolog human benmärg-härledd mesenkym stamceller med allogen human myoblast	Behandling av Duchennes muskeldystrofi
Norwegian	Autologe humane benmargsderiverte mesenkymale stamceller fusjonert ex vivo med allogene humane myoblaster	Behandling av Duchennes muskeldystrofi
Icelandic	Samgena bandvefsstofnfrumur úr beinmergi manna blandaðar ósamgena vöðvakímfrumum úr mönnum utan líkamans (ex-vivo)	Meðferð á Duchenne vöðvarýrnun