



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

2 March 2010
EMA/COMP/819565/2009
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride for the treatment of Duchenne muscular dystrophy

On 2 February 2010, orphan designation (EU/3/09/725) was granted by the European Commission to AVI BioPharma International Ltd, United Kingdom, for RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride for the treatment of Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is an inherited disease that gradually causes the muscles to become weaker. It mainly affects boys, before the age of six years. The muscle weakness usually starts in the hips and legs, before reaching the chest, arms, and sometimes the heart. DMD is caused by abnormalities in the gene responsible for the production of a protein called dystrophin, so that patients with DMD do not have enough of this protein. As dystrophin is an important component of muscle fibres, the muscles of patients with DMD cannot grow, so they become weak 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 adolescents.

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

At the time of designation, DMD affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 15,000 people, and is below the threshold for orphan

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).



designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, there were no treatments available in the EU that could cure DMD. Physiotherapy is used to relieve symptoms and improve the patient's general condition. In addition, corticosteroids are used in an attempt to improve symptoms, although they are not authorised for use in this disease and are known to have severe side effects.

How is this medicine expected to work?

In DMD, the lack of dystrophin is caused by abnormal regions in the gene that is responsible for making dystrophin. This medicine uses 'antisense' technology to skip these abnormal regions of the gene during the production of dystrophin.

The medicine is made up of genetic material (a string of nucleotides) that has been designed to stop the abnormal regions on the gene from being 'read' when the dystrophin protein is being made in the muscle cells. The genetic material is attached to a peptide (a protein fragment) which carries the genetic material into the muscle cells, including the heart and respiratory muscles. This is expected to make the muscle cells produce a version of the protein that is shorter than normal dystrophin, but which is still able to work in the same way as the full-length protein.

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 this medicine in experimental models was ongoing.

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

At the time of submission, this medicine was not authorised anywhere in the EU for DMD. Orphan designation of the product had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 December 2009 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 Community) 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

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Patient associations' contact points

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Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active ingredient	Indication
English	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride	Treatment of Duchenne muscular dystrophy
Bulgarian	РНК, [P-деокси-P-(диметиламино)] (2',3'-дидеокси-2',3'-имино-2',3'-секо) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-хидроксиетокси)етокси]етокси]карбонил]-1-пиперазинил]-N,N-диметиламинофосфонамидат], 3'-[2'a-[N2-ацетил-L-аргинил-6-аминохексаноил-L-аргинил-L-аргинил-β-аланил-L-аргинил-L-аргинил-6-аминохексаноил-L-аргинил-L-аргинил-β-аланил-L-аргинил-6-аминохексаноил-β-аланил], октохидрохлорид	Лечение на мускулна дистрофия на Duchenne
Czech	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyetoxo)etoxo]etoxo]karbonyl]-1-piperazinyl]-N,N-dimethylaminofosfonamidát], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], oktahydrochlorid	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N- dimethylaminophosphonamidat], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochlorid	Behandling af Duchenne muskeldystrofi
Dutch	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N- dimethylaminofosfonamidite], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride	Behandeling van Duchenne spierdystrofie

Language	Active ingredient	Indication
Estonian	RNA, [P-desoksü-P-(dimetüülamino)] (2',3'-didesoksü-2',3'-imino-2',3'-seco)(2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hüdroksüetoksü)etoksü]etoksü]karbonüül]-1-piperasiinüül]-N,N- dimetüülaminofoosoonamidaat], 3'-[2'a-[N2-atsetüül-L-arginüül-6-aminoheksanoüül-L-arginüül-L-arginüül-β-alanüül-L-arginüül-L-arginüül-6-aminoheksanoüül-L-arginüül-L-arginüül-β-alanüül-L-arginüül-6-aminoheksanoüül-β-alanüül], oktahüdrokloriid	Duchenne'i lihasdüstroofia ravi
Finnish	RNA [P-deoksi-P-(dimetyyliamino)] (2',3'-dideoksi-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxietoksi)etoksi]etoksi]karbonyyli]-1-piperatsinyyli]-N,N-dimetyyliaminofosfonamidaatti], 3'-[2'a-[N2-asetyyli-L-arginyyli-6-aminoheksanoyyli-L-arginyyli-β-alanyyli-L-arginyyli-L-arginyyli-6-aminoheksanoyyli-L-arginyyli-β-alanyyli-L-arginyyli-6-aminoheksanoyyli-β-alanyyli] oktahydrokloridi	Duchennen lihasdystrofian hoito
French	ARN, octachlorhydrate de [P-désoxy-P-(diméthylamino)] (2',3'-didésoxy-2',3'-imino-2',3'-séco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyéthoxy)éthoxy]éthoxy]carbonyl]-1-pipérazinyl]-N,N- diméthylaminophosphonamidate], 3'-[2'a-[N2-acétyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl]	Traitement de la dystrophie musculaire de Duchenne
German	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N- dimethylaminophosphonamidat], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochlorid	Behandlung der Duchenne-Muskeldystrophie
Greek	RNA, [P-δεοξυ-P-(διμεθυλαμινο)] (2',3'-διδεοξυ-2',3'-ιμινο-2',3'-seco) (2'a→5')(C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-υδροξυαιθοξυ)αιθοξυ]αιθοξυ]καρβονυλο]-1-πιπεραζινυλο]-N,N-φωσφονικοαμιδικοδιμεθυλαμινο], 3'-[2'a-[N2-ακετυλ-L-αργινυλ-6-αμινοεξανυλ-L-αργινυλ-L-αργινυλ-β-αλανυλ-L-αργινυλ-L-αργινυλ-6-αμινοεξανυλ-L-αργινυλ-β-αλανυλ-L-αργινυλ-6-αμινοεξανυλ-β-αλανυλ], οκταυδροχλωρίδιο	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	RNS, [P-dezoxi-P-(dimetilamino)] (2',3'-didezoxi-2',3'-imino-2',3'-szeko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroxietoxi)etoxi]etoxi]karbonil]-1-piperazinil]-N,N-dimetilaminofoszfamidát], 3'-[2'a-[N2-acetil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminohexanoil-β-alanil], oktahidroklorid	Duchenne dystrophia kezélése

Language	Active ingredient	Indication
Italian	RNA, [P-deossi-P-(dimetilamino)] (2',3'-dideoossi-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-idrossietossi)etossi]etossi]carbonil]-1-piperazinil]-N,N-dimetilaminofosfonamidato], 3'-[2'a-[N2-acetil-L-arginil-6-aminoesanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminoesanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminoesanoil-β-alanil], ottaidrocloruro	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	RNS, [P-deoksi-P-(dimetilamino)] (2',3'-dideoksi-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroksietoksi)etoksi]etoksi]karbonil]-1-piperazinil]-N,N-dimetilaminofosfonamidāts], 3'-[2'a-[N2-acetil-L-arginil-6-aminoheksanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminoheksanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminoheksanoil-β-alanil], oktahidrohlorīds	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	RNR, [P-deoksi-P-(dimetilamino)] (2',3'-dideoksi-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroksietoksi)etoksi]etoksi]karbonil]-1-piperazinil]-N,N-dimetilaminofosfonamidatas], 3'-[2'a-[N2-acetil-L-arginil-6-aminoheksanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminoheksanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminoheksanoil-β-alanil], oktahidrochloridas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	RNA, [P-deoksy-P-(dimetyloamino)] (2',3'-dideoksy-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroksyetoksy)etoksy]etoksy]karbonylo]-1-piperazyńnylo]-N,N-dimetyloaminofosfonoamidany], okta-chlorowodorek 3'-[2'a-[N2-acetylo-L-arginylo-6-aminoheksanoilo-L-arginylo-L-arginylo-β-alanylo-L-arginylo-L-arginylo-6-aminoheksanoilo-L-arginylo-L-arginylo-β-alanylo-L-arginylo-6-aminoheksanoilo-β-alanylowy]	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	ARN, [P-desoxi-P-(dimetilamina)] (2',3'-didesoxi-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroxietoxi)etoxi]etoxi]carbonilo]-1-piperazinilo]-N,N-dimetilaminofosfonamida], 3'-[2'a-[N2-acetil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminohexanoil-β-alanil], octahidrocloreto	Tratamento da distrofia muscular de Duchenne

Language	Active ingredient	Indication
Romanian	ARN, octohidroclorură de [P-deoxi-P-(dimetilamino)] (2',3'-dideoxi-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroxietoxi)etoxi]etoxi]carbonil]-1-piperazinil]-N,N-dimetilaminofosfonamidat], 3'-[2'a-[N2-acetil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminohexanoil-β-alanil]	Tratamentul distrofiei musculare Duchenne
Slovak	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyetoxi)etoxi]etoxi]karbonyl]-1-piperazinyl]-N,N-dimethylaminofosfonamidát], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], oktahydrochlorid	Liečba Duchennevej muskulárnej dystrofie
Slovenian	RNA, [P-deoksi-P-(dimetilamino)] (2',3'-dideoksi-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroksietoksi)etoksi]etoksi]karbonil]-1-piperazinil]-N,N-dimetilaminofosfonamidat], 3'-[2'a-[N2-acetil-L-arginil-6-aminoheksanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminoheksanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminoheksanoil-β-alanil], oktahidroklorid	Zdravljenje Duchenneve mišične distrofije
Spanish	ARN, octahidrocloruro de [P-deoxi-P-(dimetilamina)] (2',3'-dideoxi-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hidroxietoxi)etoxi]etoxi]carbonilo]-1-piperazinilo]-N,N-dimetilaminofosfonamida], 3'-[2'a-[N2-acetil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-L-arginil-6-aminohexanoil-L-arginil-L-arginil-β-alanil-L-arginil-6-aminohexanoil-β-alanil]	Tratamiento de la distrofia muscular de Duchenne
Swedish	RNA, [P-deoxi-P-(dimethylamino)] (2',3'-dideoxi-2',3'-imino-2',3'-seko) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroxyetoxi)etoxi]etoxi]Karbonyl]-1-piperazinyl]-N,N-dimethylaminofosfonamidat], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], oktohydroklorid	Behandling av Duchennes muskeldystrofi
Norwegian	RNA, [P-deoksy-P-(dimethylamino)] (2',3'-dideoksy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hydroksyetoksy)etoksy]etoksy]karbonyl]-1-piperazinyl]-N,N-dimethylaminofosfonamidat], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminoheksanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminoheksanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminoheksanoyl-β-alanyl], oktahydroklorid	Behandling av Duchennes muskeldystrofi

Language	Active ingredient	Indication
Icelandic	RKS (RNA), [P-deoxý-P-(dímetýlamínó)] (2',3'-tvídeoxý-2',3'-ímínó-2',3'-secó) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5'-[P-[4-[[2-[2-(2-hýdróxyetoxý)etoxý]etoxý]karbónýl]-1-píperasínýl]-N,N-dímetýlamínófosfónamídat], 3'-[2'a-[N2-acetýl-L-arginýl-6-amínóhexanoyl-L-arginýl-L-arginýl-β-alanýl-L-arginýl-L-arginýl-6-amínóhexanoyl-L-arginýl-L-arginýl-β-alanýl-L-arginýl-6-amínóhexanoyl-β-alanýl], oktahýdróklóríð	Meðferð á Duchenne vöðvarýrnun