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Dolna 17, Warsaw,
Poland 00-773
+48 226 0 227 03
editorial_office@rsglobal.pl

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REPAIRING GENES, RESTORING STRENGTH: THE PROMISE OF MODERN GENE THERAPY IN DUCHENNE MUSCULAR DYSTROPHY

Katarzyna Kozon *(Corresponding Author, Email: kasia.kozon1066@gmail.com)*
Masovian Bródnowski Hospital, ul. Ludwika Kondratowicza 8, 03-242 Warsaw, Poland
ORCID ID: 0009-0009-6536-946X

Agnieszka Floriańczyk
Masovian Bródnowski Hospital, ul. Ludwika Kondratowicza 8, 03-242 Warsaw, Poland
ORCID ID: 0009-0003-5136-0380

Ewa Romanowicz
Independent Public Healthcare Institution in Wyszków, ul. Komisji Edukacji Narodowej 1, 07–200 Wyszków, Poland
ORCID ID: 0009-0004-6612-3174

Aleksandra Kołdyj
District Medical Centre in Grójec, ul. Piotra Skargi 10, 05-600 Grójec, Poland
ORCID ID: 0009-0002-5695-608X

Agnieszka Ozdarska
Independent Public Healthcare Institution in Wyszków, ul. Komisji Edukacji Narodowej 1, 07–200 Wyszków, Poland
ORCID ID: 0009-0008-7083-2140

Adrian Krzysztof Biernat
Specialist Provincial Hospital in Ciechanów, ul. Powstańców Wielkopolskich 2, 06-400 Ciechanów, Poland
ORCID ID: 0009-0007-6734-0447

Marcin Lampart
St. John Paul II Independent Public Western Specialist Hospital, ul. Daleka 11, 05-825 Grodzisk Mazowiecki, Poland
ORCID ID: 0009-0005-8485-850X

Anna Rupińska
Independent Public Outpatient Healthcare Group – Warsaw-Ochota, ul. Szczęśliwicka 36, 02-353 Warsaw, Poland
ORCID ID: 0009-0001-8567-5925

Zuzanna Jasińska
Warsaw Southern Hospital, ul. Rotmistrza Witolda Pileckiego 99, 02-781 Warsaw, Poland
ORCID ID: 0009-0001-9448-782X

Kamila Krzewska
Primary Care Clinic "Zdrowa Rodzina", ul. Generała Tadeusza Pełczyńskiego 22J, 01-471 Warsaw, Poland
ORCID ID: 0009-0002-5672-3796

ABSTRACT

Introduction: Duchenne muscular dystrophy (DMD) is a severe X-linked neuromuscular disorder characterized by progressive muscle degeneration due to mutations in the dystrophin gene. Current treatments primarily focus on symptom management. Delandistrogene moxeparvovec is a gene therapy designed to deliver a shortened, functional version of the dystrophin gene, potentially modifying disease progression.
Aim of the study: To evaluate the clinical efficacy and safety of delandistrogene moxeparvovec in improving skeletal muscle function and physical performance in patients with DMD based on data from completed clinical trials.
Methodology: The analysis includes four published clinical trials involving ambulatory boys aged 4–8 years with confirmed dystrophin mutations. Primary outcomes focused on changes in the North Star Ambulatory Assessment (NSAA), while secondary endpoints included time to rise from the floor and the 10-meter walk/run test. Study designs ranged from open-label to randomized, double-blind trials. Adverse events and regulatory outcomes were also considered.
Conclusions: Delandistrogene moxeparvovec demonstrated variable improvements in motor function, with more pronounced benefits in younger age groups. Some trials showed statistically significant results, while others failed to meet primary endpoints. Adverse events, including serious complications, were reported. The therapy received FDA approval in 2023 and 2024 for defined patient subgroups, though some decisions were made despite inconclusive efficacy data.

KEYWORDS

Duchenne Muscular Dystrophy, Delandistrogene Moxeparvovec, Gene Therapy, Skeletal Muscle Function, Genetic Disorders, Dystrophin

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1. Introduction

Duchenne muscular dystrophy (DMD) is the most common and one of the most severe forms of muscular dystrophy, inherited in an X-linked recessive manner (Bez Batti Angulski et al., 2023). The disease results from mutations in the dystrophin (DMD) gene, leading to progressive damage of striated skeletal and cardiac muscle tissue (Shih et al., 2020). Dystrophin is a key component of the sarcolemmal protein complex, which stabilizes and protects muscle fibers during contraction. It functions as a structural bridge between the myofibrillar cytoskeleton and the extracellular matrix. The absence or dysfunction of dystrophin gradually compromises sarcolemmal integrity, resulting in progressive muscle fiber degeneration and pathological fat accumulation (Aldharee, 2024). Clinically, DMD is characterized by early symptom onset, typically around 2–3 years of age. By the age of 12, most patients become wheelchair-dependent, and by approximately 20 years of age, assisted ventilation becomes necessary. The average life expectancy ranges from 20 to 40 years, with respiratory or cardiac failure being the most common causes of death (Duan et al., 2021). The diagnostic process for DMD usually begins in early childhood, prompted by hallmark clinical signs such as a positive Gowers' sign, progressive muscle weakness, and motor clumsiness. Muscle atrophy typically begins in the proximal limb muscles and progresses to distal groups. A distinctive feature of DMD is calf pseudohypertrophy caused by fibrotic tissue deposition and fat infiltration. As the disease progresses, children often develop toe-walking due to shortening of the plantar flexor muscles. Less frequently, diagnosis is preceded by the detection of developmental delay or elevated serum enzyme levels, such as alanine aminotransferase (ALT), aspartate aminotransferase (AST), lactate dehydrogenase (LDH), or creatine kinase (CK). Elevated ALT, AST, or LDH may be misattributed to hepatic pathology, potentially delaying the correct diagnosis of DMD (Birnkrant et al., 2018; Patterson et al., 2023). Conventional DMD therapies have focused on symptom management and delaying disease progression through glucocorticoids, physical rehabilitation, and support of respiratory and cardiovascular function (Trucco et al., 2020). However, these treatments do not target the underlying genetic

defect and offer only partial clinical benefit. In recent years, gene therapy has emerged as a promising treatment avenue, aiming to introduce functional copies of the dystrophin gene or its truncated variants such as micro-dystrophin—capable of partially restoring protein function (Mendell et al., 2023). Delandistrogene moxeparvovec gene therapy (Elevidys, SRP-9001) is a gene therapy currently undergoing clinical evaluation. Its mechanism involves delivering a gene encoding micro-dystrophin to the patient's muscle cells via a viral vector, enabling synthesis of a functional protein and potentially improving muscle structure and function (Mendell, Sahenk, et al., 2024).

2. Aim of The Study

This review aims to assess the therapeutic potential of delandistrogene moxeparvovec (Elevidys) gene therapy in the treatment of Duchenne muscular dystrophy (DMD). As previously outlined, DMD is a progressive genetic disorder that causes degeneration of skeletal, respiratory, and cardiac muscles, significantly reducing lifespan and quality of life. Despite the availability of symptomatic treatments, conventional therapies do not modify the molecular progression of the disease. Delandistrogene moxeparvovec, the first approved gene therapy for DMD, offers the possibility of delivering a functional version of the dystrophin gene, thereby opening new therapeutic prospects. This review presents the current understanding of the mechanism of action of this therapy, its impact on motor function and clinical outcomes, and discusses potential limitations and challenges associated with its application. The primary goal is to determine whether delandistrogene moxeparvovec represents a transformative therapeutic intervention that alters the natural course of DMD and improves patient quality of life.

3. Methodology

This review is based on an analysis of the available scientific literature concerning Elevidys gene therapy and its application in the treatment of Duchenne muscular dystrophy. Literature searches were primarily conducted using the PubMed database, employing a combination of the following keywords: "Duchenne muscular dystrophy", "gene therapy", "Elevidys", "delandistrogene moxeparvovec", "microdystrophin", "AAV gene delivery", "functional outcomes", "motor function", "quality of life", and "clinical trial." Studies published in peer-reviewed journals between 2010 and 2025 were included, with particular emphasis on clinical trials conducted between 2022 and 2025. Included in the analysis were phase 1–3 clinical trials, systematic reviews, and expert articles focusing on the mechanism of action, safety, and efficacy of Elevidys. Special attention was given to the therapy's effects on functional metrics (e.g., North Star Ambulatory Assessment), biomarkers of dystrophin expression, and quality of life indicators for patients and their families. Selection criteria for the literature included recency, scientific relevance, methodological clarity, and data reliability.

4. Current State of Knowledge

4.1 Molecular Mechanisms of DMD and the Role of Muscle Function in Daily Living

Duchenne muscular dystrophy is caused by mutations in the DMD gene, which, as previously discussed, result in the absence of the muscle-specific dystrophin isoform (Dp427m). Mutations in the DMD gene may also lead to Becker muscular dystrophy (BMD), a milder disorder characterized by later onset and slower progression (Mercuri et al., 2019). Thousands of DMD gene mutations have been identified in patients with Duchenne and Becker muscular dystrophies. In both disorders, deletions account for approximately 60–70% of mutations. In DMD, duplications represent 5–15% of cases, while point mutations, small deletions, or insertions make up about 20%; the distribution in BMD is slightly different. These mutations commonly cluster in so-called hotspot regions, including exons 45–55 and 3–9 for DMD . Rarely, larger genomic rearrangements such as translocations between the X chromosome and autosomes occur, causing DMD in both males and females (Nevin et al., 1986). The DMD gene encodes the full-length dystrophin isoform Dp427m found in muscle, as well as other full-length isoforms- Dp427c and Dp427p present in cortical neurons and Purkinje cells of the cerebellum, respectively. In addition to these, shorter dystrophin isoforms are expressed in various tissues (Chelly et al., 1990; Górecki et al., 1992). Dystrophin forms a complex with associated proteins collectively known as the dystrophin-associated protein complex (DAPC) which links the sarcolemma (muscle cell membrane) to the cytoskeleton and extracellular matrix (Gao & McNally, 2015). The absence of dystrophin leads to the disintegration of the DAPC, disrupting the connection between the cytoskeleton, sarcolemma, and extracellular matrix. The sarcolemma becomes susceptible to mechanical damage during muscle contractions, facilitating the leakage of muscle enzymes into the bloodstream, such as creatine kinase (Aartsma-Rus & van Putten, 2014). Dystrophin is also involved in nitric oxide production and vasodilation during exercise. In DMD, these processes are impaired,

leading to muscle ischemia during exertion (Sander et al., 2000). Moreover, muscles in DMD produce increased levels of reactive oxygen and nitrogen species, while mitochondrial damage and inflammatory cells exacerbate oxidative stress. Microdamage to the plasma membrane results in abnormal calcium influx, leading to mitochondrial dysfunction and activation of proteolytic enzymes that degrade muscle tissue. Endoplasmic reticulum activity is also disrupted by elevated sarcolipin levels. Dystrophin deficiency impairs satellite cell division and their ability to repair muscle damage, resulting in defective muscle regeneration and replacement of myocytes with fat and fibrotic tissue. Autophagy is also inhibited, leading to the accumulation of damaged organelles and proteins, and further muscle degeneration. Early in the disease, M1 macrophages and T lymphocytes infiltrate muscle tissue, exacerbating damage; at later stages, M2 macrophages predominate, promoting regeneration and fibrosis (Duan et al., 2021). In cardiac muscle, dystrophin interacts with specific protective proteins, which may explain the delayed onset of cardiomyopathy in DMD patients (Klietsch et al., 1993). Dystrophin is also expressed in specific brain structures, including the amygdala, hippocampus, and Purkinje cells. Its deficiency is associated with cognitive impairment and behavioral disturbances in approximately one-third of individuals with DMD (Thangarajh et al., 2019).

4.2 Current Standard Treatments and Their Limitations

At diagnosis, boys with Duchenne muscular dystrophy typically show substantial muscle mass loss, and available treatments - primarily physiotherapy and glucocorticoids-can only slow disease progression (Birnkrant et al., 2018). Glucocorticoids suppress the immune system and help prolong ambulation. Deflazacort was approved by the U.S. Food and Drug Administration (FDA) for DMD treatment in 2017. Clinical studies have shown that daily deflazacort use is associated with improved functional outcomes, particularly in older patients and in advanced disease stages (McDonald et al., 2023). Although only deflazacort has received FDA approval, prednisone and prednisolone are also widely used (Zelikovich et al., 2022). Other anti-inflammatory agents have been tested in clinical trials. One such drug is vamorolone, approved in 2023 by both the FDA and the European Medicines Agency (EMA). It effectively inhibits the pro-inflammatory NF-κB pathway without adversely affecting bone remodeling biomarkers, thereby not increasing the risk of osteoporosis or fractures (Guglieri et al., 2022). Promising results have also been reported for givinostat, a histone deacetylase inhibitor that regulates gene expression in muscle and satellite cells by modulating dysregulated nitric oxide signaling. Givinostat received FDA approval in March 2024 and, in April 2025, EMA issued a conditional recommendation for its use alongside corticosteroids in ambulatory DMD patients aged six years and older in the European Union (EU) (EMA, 2025b; Syed et al., 2025).

Since nonsense mutations account for approximately 10–15% of DMD cases, ataluren has been used to treat this subgroup (Bladen et al., 2015). This compound enables ribosomes to "read through" premature stop codons, facilitating the production of a truncated but partially functional dystrophin protein (Mercuri et al., 2023). Clinical trial results indicated modest improvements in treated boys, leading to conditional EMA approval in 2014 and limited use in non-EU countries-excluding the United States, where it was never approved. Although some newer data suggested a potential delay in symptom progression, EMA concluded that the drug's impact on dystrophin production was minimal. Consequently, the agency recommended against renewing marketing authorization in both 2023 and January 2024. Finally, on March 28, 2025, the European Commission revoked ataluren’s conditional marketing authorization in the EU (Bello et al., 2025; EMA, 2025a).

Antisense oligonucleotides (ASOs) are synthetic nucleic acid fragments that modulate pre-mRNA splicing, promoting exon skipping in the dystrophin gene to produce a shortened yet functional dystrophin protein (Aoki & Wood, 2021). Four such drugs-eteplirsen, golodirsen, casimersen, and viltolarsen-received conditional approval in the U.S. between 2016 and 2021. Viltolarsen was also approved in Japan in 2020. None have been approved by the EMA. Although these therapies apply to only about 14% of DMD patients, they may improve muscle function despite modest increases in dystrophin levels (Roshmi & Yokota, 2021).

CRISPR/Cas9 technologies are currently under investigation as potential DMD treatments (Szwec et al., 2024). These methods enable dystrophin gene correction in animal models, restoring functional protein expression. Other experimental approaches aim to upregulate utrophin expression as a partial dystrophin substitute, although efficacy remains limited (Morin et al., 2023). This strategy only affects a subset of muscle nuclei, resulting in uneven tissue protection. Risks associated with AAV vector-based gene delivery—such as immune responses and off-target mutations- persist, though rAAV vectors continue to serve as the foundation for several approved gene therapies (Bennett, 2023; Bönnemann et al., 2023; Morin et al., 2023). Restoration of dystrophin function can be achieved by administering recombinant adeno-associated viral vectors (rAAV) carrying sequences encoding micro-dystrophin or micro-utrophin (Morin et al., 2023). These sequences are

based on functional variants of dystrophin observed in patients with the milder Becker muscular dystrophy (BMD) phenotype (Ramos et al., 2019). Alternatively, utrophin levels can be increased using molecules that modulate UTRN gene regulators or stabilize the utrophin-glycoprotein complex (UGC). Preliminary preclinical studies in mouse and dog models have demonstrated a high therapeutic potential of micro-dystrophin and micro-utrophin (μDys/μUtr). However, micro-dystrophin elicits a stronger immune response, which has been associated in some clinical trials with serious adverse events, such as immune-mediated myositis. On the other hand, while micro-utrophin has a more favorable safety profile, it exhibits lower mechanical efficacy and limited durability of expression in myocytes.

The first gene therapy to receive initial conditional approval from the FDA in 2023 for patients aged 4–5 years was delandistrogene moxeparvovec (Zaidman et al., 2024). Delandistrogene moxeparvovec (Elevidys) is a gene therapy developed as a treatment for DMD. It comprises a non-replicating recombinant AAV vector derived from serotype 74 isolated from rhesus macaques (rAAVrh74), carrying a micro-dystrophin transgene, leading to the production of a truncated micro-dystrophin protein (with a molecular weight of 138 kDa, compared to 427 kDa for full-length dystrophin in healthy muscle) (Mendell, Sahenk, et al., 2024). Gene replacement therapies under development for Duchenne muscular dystrophy (DMD) utilize truncated constructs of the DMD gene ("micro-dystrophins") containing only the most essential domains, as the wild-type DMD gene, consisting of 79 exons and encoding a 14 kb transcript, exceeds the packaging capacity of adeno-associated virus (AAV) vectors (approximately 5 kb) [38]. As previously mentioned, the delandistrogene moxeparvovec gene therapy received accelerated approval from the U.S. Food and Drug Administration on June 22, 2023, for ambulatory boys aged 4–5 years. Subsequently, on June 20, 2024, based on phase 3 clinical trial data, the indication was expanded, resulting in full approval for use in ambulatory boys with DMD aged 4 years and older, along with accelerated approval for use in non-ambulatory patients with DMD (Bhattacharyya et al., 2024; Zaidman et al., 2024).

4.3 Effect of Elevidys Gene Therapy on Skeletal Muscle Function and Physical Performance in Patients with DMD

To date, results from three completed clinical trials and the first completed cohort (cohort 1) of the ongoing ENDEAVOR phase 1b study investigating delandistrogene moxeparvovec have been published. Their primary aim has been to assess the therapy's impact on motor function and physical performance in boys with DMD, particularly within the 4–8 year age range. Two additional studies are ongoing. Across all trials, the primary measure of therapeutic efficacy was the North Star Ambulatory Assessment (NSAA), which includes 17 tasks evaluating gait and gross motor function. Each task is scored on a three-point scale: 0 indicates inability to perform the task, 1 indicates performance with difficulty or assistance, and 2 indicates independent completion. The scale assesses activities such as rising from the floor, walking, and running. Secondary outcomes were also analyzed, including time to rise from the floor (TTR) and the 10-meter walk/run test (10MWR). In healthy children, the maximum score of 34 is typically achieved by age 4. In patients with DMD, improvement is observed until approximately age 6, followed by a gradual annual decline averaging 3.7 points after age 7. The minimally clinically important difference (MCID) on the NSAA is estimated to range between 2.3 and 3.5 points, depending on the calculation method (Ayyar Gupta et al., 2023).

The first study (NCT03375164) was a open-label, phase 1/2a, nonrandomized controlled trial involving four boys aged 4 to under 8 years with confirmed dystrophin gene mutations. The trial assessed the safety and tolerability of a single intravenous dose of delandistrogene moxeparvovec. Treatment was preceded by a dose of prednisone administered the day before infusion and continued for at least 30 days. After one year of follow-up, improvement in motor function was observed; however, the lack of a control group classifies this study as providing insufficient evidence of efficacy (Class IV). A subsequent publication included a comparison with an external control group matched via propensity score weighting, estimating a mean NSAA difference of 9.4 points in favor of gene therapy after four years (95% CI: 2.02–16.78), though this still constitutes Class III evidence (Mendell, Sahenk, et al., 2024).

The second clinical trial (NCT03769116) was a randomized, double-blind, placebo-controlled, phase 2 study conducted at two centers, divided into two 48-week parts. A total of 41 boys aged 4 to under 8 years with mutations involving exons 18–58 were enrolled. Participants were randomly assigned to receive either the gene therapy or placebo, with crossover in the second part of the study. All patients received prednisone for at least 60 days prior to therapy. The primary endpoint was the level of micro-dystrophin expression at week 12 post-treatment. This level, measured by Western blot, reached 23.82% of the physiological level in healthy individuals, indicating successful protein production. However, no statistically significant

improvement in NSAA scores was observed after 48 weeks across the full cohort-the mean difference between treated and control groups was 0.8 points (95% CI: –0.95 to 2.55), suggesting the result could be due to chance. Subgroup analysis by age revealed a significant improvement of 2.5 points (95% CI: 0.44–4.56) in children aged 4–5, while older children (6–7 years) showed a non-significant and slightly negative change (–0.7 points). After 96 weeks, the mean NSAA difference was 2.0 points (95% CI: –0.50 to 4.50), still not definitively significant. A similar result was obtained in the second part of the study when comparing with an external cohort—the LSM difference was 2.0 points (95% CI: 0.82–3.18), which this time reached statistical significance. As a randomized and double-blind study, this trial provides Class I evidence (Mendell et al., 2023).

The ENDEAVOR study is a two-part, open-label, phase 1b study of delandistrogene moxeparvovec. As of today, data from cohorts 2–7 of this study have not yet been published Cohort 1 of ENDEAVOR study comprising 20 ambulatory boys with DMD aged 4 to 8 years received a single intravenous dose of the therapy and corticosteroids for at least 60 days post-treatment. After one year, motor function was compared to a propensity score–matched control group (91 children). Results showed that treated boys improved by a mean of 3.2 NSAA points (95% CI: 1.59–4.81). Additionally, they were 1.2 seconds faster in the time to rise test (95% CI: –1.81 to –0.60) and 1 second faster in the 10-meter walk/run test (95% CI: –1.63 to –0.37). In a separate group of older, non-ambulatory boys (6 patients, mean age 15.26 years), upper limb function declined by –1.5 points after 52 weeks and –3.8 points after 104 weeks—indicating a slower decline than the natural disease course, which showed a –6.3 point decrease. Despite promising results, the lack of randomization categorizes this study as providing Class III, or moderate-quality, evidence(Zaidman et al., 2023).

The EMBARK trial (NCT05096221) is a phase 3, two-part, multinational, randomized, double-blind, placebo-controlled study involving 125 ambulatory DMD patients aged 4 to <8 years, with mutations in exons 18–44 and 46–79. Randomization was stratified by age and baseline NSAA score. Corticosteroid doses were comparable at baseline, but from weeks 2 to 12, the treatment group received higher doses. The primary endpoint—change in total NSAA score after 52 weeks-was not met (LSM difference 0.65; 95% CI: –0.45 to 1.75). No significant differences were observed across age or baseline NSAA subgroups. Secondary analyses showed small but significant improvements in functional tests: time to rise (TTR) and 10-meter walk (LSM differences –0.64 and –0.42 seconds, respectively). Step velocity and 4-stair climb time also favored the treatment group. The 100-meter walk/run test showed no difference. Patient-reported outcomes related to mobility and upper limb function were similar between groups. Nausea and vomiting occurred in 54% of treated participants and 0% in the placebo group, which may have compromised blinding integrity (Mendell et al., 2025). A comparison of the studies described is presented in Table 1.

Table 1. Clinical efficacy of Delandistrogene moxeparvovec gene therapy across selected trials, focusing on changes in motor function as measured by the North Star Ambulatory Assessment (NSAA).

Study (ID)	Study Characteristics	Number of Patients	Age of Patients	NSAA Change	Class of Evidence
NCT03375164	Phase 1/2a, open-label, nonrandomized controlled trial	4 boys	4–<8 years	+9.4 points (at 4 years vs. external group)	Class III evidence
NCT03769116	Phase 2, randomized, double-blind, placebo-controlled	41 boys	4–<8 years	+0.8 pts at 48 weeks (entire group, NS), +2.5 pts (age 4–5, IS), –0.7 pts (age 6–7, NS), +2.0 pts at 96 weeks (NS) vs. external group (IS)	Class I evidence
ENDEAVOR (NCT04626674)	Phase 1b, open-label	20 boys (cohort 1)	4–8 years, ambulatory	+3.2 pts after 1 year (vs. external group, IS)	Class III evidence
EMBARK (NCT05096221)	Phase 3, randomized, double-blind, placebo-controlled	125 boys	4–<8 years	+0.65 pts after 52 wks (NS)	Class I evidence

Based on clinical trial data, thirteen adverse events requiring treatment were identified, including vomiting, myocarditis, acute liver injury, and immune-mediated myositis (Zaidman et al., 2024). To mitigate the risk of immune response, corticosteroids are administered to patients one day prior to infusion and continued for at least 60 days thereafter. The most frequently observed adverse events (in ≥5% of patients) include nausea, vomiting, fever, thrombocytopenia, and transient elevations in liver enzymes, which typically resolve during steroid therapy. Less common but more serious complications include acute hepatitis and myocarditis. One death due to liver failure was reported in a patient treated outside of a clinical trial setting. In some patients with deletions involving exons 8 or 9, myositis occurred, leading to marked weakness and difficulty swallowing or breathing. Safety data for individuals with deletions spanning exons 1–17 are lacking, as this group was not included in key clinical studies (Mendell, Proud, et al., 2024).

Delandistrogene moxeparvovec received accelerated approval from the FDA on June 22, 2023, for ambulatory boys aged 4–5 years (Zaidman et al., 2024). Clinical, pharmacological, and statistical review teams considered that the data did not meet the criteria for accelerated approval, as micro-dystrophin expression was not deemed a sufficient surrogate endpoint; consequently, the decision was considered controversial. Subsequently, on June 20, 2024, the FDA granted full approval for ambulatory patients aged ≥4 years and accelerated approval for non-ambulatory patients aged ≥4 years. This decision was made despite negative recommendations from several review divisions (including clinical and biostatistical offices). It was concluded that data from the EMBARK (NCT05096221) and ENDEAVOR (NCT04626674) trials provided reliable evidence of efficacy, with the exception of patients with exon 8 and/or 9 deletions, in whom the use of the drug is contraindicated. The increase in micro-dystrophin levels in non-ambulatory patients was considered potentially predictive of clinical benefit, forming the basis for approval despite the absence of proven clinical efficacy in this subgroup. Ongoing trials aim to provide further data, including in boys with other mutations and in non-ambulatory patients. Many of these individuals accept an elevated risk due to the lack of alternative disease-modifying therapies (Crossnohere et al., 2022; Oskoui et al., 2025).

5. CONCLUSIONS

Duchenne muscular dystrophy (DMD) is a severe, progressive neuromuscular disorder of genetic origin, caused by mutations in the dystrophin gene. The absence of functional dystrophin protein leads to muscle fiber degeneration, chronic inflammation, and gradual deterioration of muscle strength and function. Symptoms typically emerge in early childhood, and despite advances in supportive care, DMD still results in premature death and remains a major therapeutic challenge.

Delandistrogene moxeparvovec is an investigational gene therapy designed to deliver a shortened version of the dystrophin gene (micro-dystrophin) to muscle cells using an adeno-associated viral (AAV) vector. Although earlier phase studies suggested improved micro-dystrophin expression and potential clinical benefit, the phase 3 EMBARK trial results were less promising. The primary endpoint-change in motor function assessed by the North Star Ambulatory Assessment (NSAA) after 52 weeks-did not demonstrate statistically or clinically significant improvement compared to placebo.

The NSAA is one of the most commonly used functional scales in boys with DMD, evaluating the ability to perform daily activities that require strength and coordination. While useful, this scale may have limited sensitivity in detecting subtle, slow functional changes, particularly in studies with short observation periods. Furthermore, minor differences in secondary endpoints in the EMBARK trial also failed to reach statistical significance.

Interpretation of the results is complicated by the use of corticosteroids—the standard of care in DMD—which independently improve muscle function, delay disease progression, and may mask or alter the response to gene therapy. Variability in dosing, duration, and type of steroid used represents a significant confounding factor that must be considered when analyzing treatment effects.

Ongoing studies are expected to provide more precise data regarding the long-term efficacy and safety of delandistrogene moxeparvovec. It is important to exercise caution in interpreting the current findings and to continue research efforts aimed at defining the true role of gene therapy in the treatment of DMD.

DISCLOSURE

Authors' contribution:
Conceptualization: Katarzyna Kozon, Agnieszka Floriańczyk
Methodology: Agnieszka Ozdarska, Adrian Krzysztof Biernat
Software: Anna Rupińska
Check: Adrian Krzysztof Biernat
Formal analysis: Agnieszka Floriańczyk, Ewa Romanowicz
Investigation: Marcin Lampart, Zuzanna Jasińska
Resources: Aleksandra Kołdyj, Kamila Krzewska
Data curation: Agnieszka Ozdarska
Writing -rough preparation: Katarzyna Kozon, Zuzanna Jasińska
Writing -review and editing: Ewa Romanowicz, Kamila Krzewska
Supervision: Katarzyna Kozon
Project administration: Aleksandra Kołdyj, Marcin Lampart
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