American Journal of Biomedical Science & Research

www.biomedgrid.com

ISSN: 2642-1747

Mini Review

The Challenge to Cure Duchenne Muscle Dystrophy in Childhood

Stefan Bittmann[1,2]*, Elisabeth Luchter[1], Elena Moschüring Alieva[1]

[1]*Department of Pediatrics, Ped Mind Institute, Department of Pediatrics, Hindenburgring 4, D-48599 Gronau, Germany*

[2]*Honorary Professor (Hon.Prof.), School of Medicine, Shangluo Vocational and Technical College, Shangluo, 726000, Shaanxi, China*

***Corresponding author:** Stefan Bittmann, Department of Pediatrics, Ped Mind Institute, Department of Pediatrics, Hindenburgring 4, D-48599 Gronau, Germany and Honorary Professor (Hon.Prof.), School of Medicine, Shangluo Vocational and Technical College, Shangluo, 726000, Shaanxi, China.

To Cite This article: *Stefan Bittmann*, Elisabeth Luchter, Elena Moschüring Alieva. The Challenge to Cure Duchenne Muscle Dystrophy in Childhood. Am J Biomed Sci & Res. 2026 29(5) AJBSR.MS.ID.003847,*

DOI: 10.34297/AJBSR.2026.29.003847

Received: January 09, 2026; **Published:** January 21, 2026

Abstract

Duchenne Muscular Dystrophy (DMD) is an X-linked recessive inherited muscle disease that manifests in early childhood and rapidly leads to muscle weakness, muscle atrophy, and wheelchair dependence. It is the most common form of progressive muscular dystrophies in childhood. The disease affects one in 3,500 male newborns and manifests between the first and sixth year of life. Girls usually do not develop DMD because a gene defect on one X chromosome can be compensated for by the second X chromosome. Duchenne muscular dystrophy is inherited in an X-linked recessive manner. A mutation in the dystrophin gene leads to the absence of dystrophin in muscle cells. In approximately 60-70% of cases, this is due to a deletion, in 35% to a point mutation, and in about 5% to a duplication. Most often exons 45 to 55 are involved with a peak on exon 51. This is an important aspect for exone skipping approaches. In histopathological examination, muscle fibers are degenerated and necrotic. Cell nuclei are centrally located. Endomysium and perimysium are fibrosed. The decrease in muscle tissue is compensated for by an increase in adipose tissue and connective tissue. Recent research focus on interesting new therapy options like exone skipping approaches, vector-based gene transfer and CRISP-Cas9 technology to repair the mutation on the genetic level and nanocarrier or magnetic microroboter delivery approaches to bring a functional dystrophin gene in right place.

Keywords: Duchenne, Muscular atrophy, Children, Treatment, Exone skipping, Gene therapy, CRISP Cas9, Nanocarrier, Magnetic microroboter

Introduction

Duchenne muscular dystrophy, also known as Duchenne muscular dystrophy or DMD, is the most common muscular genetic disease in childhood. Duchenne muscular dystrophy was described by Guillaume-Benjamin Duchenne in Paris in the 19th century [1]. It occurs at a frequency of about 1:3600 to 1:6000 [2-9]. The gene locus is Xp21.2 [2-32]. Various mutations of the Dystrophin gene are found, here in special 60% deletions, 5% duplications, 35% point and nonsense mutations [2-32]. The type of mutation is crucial for the individual course of the disease and the outcome. The dystrophin gene includes 79 exons and 78 introns with an overall production of an mRNA of 14000 nucleotides. The gene has 7 promoters at 5`end: 3 of 7 promoters produce full-length dystrophin isoforms: Dp427m (muscle), Dp427c (brain) and Dp427p (cerebellar Purkinje cells). 4 of 7 promoters produce shorter dystrophin isoforms: Dp260 (retina), Dp140 (brain) and Dp116 (Schwann cells, cardiac cells). 4 major functional domains do exist: the N-terminal, rod domain, a cysteine-rich domain and a C-terminal domain. A severe impairment of muscle function, dysregulation of muscle fiber stability during contraction, disruption of several signaling pathways, segmental myofiber fibrosis, membrane leakage, chronic inflammation with unbalanced polarization of M1 and M2 macrophages and nevertheless, compromised myogenesis are prominent features of the disease. Muscle fiber weakness. Outflow of CK and LDH out of muscle cells, well-known markers of DMD; influx of intracellular Ca2+ concentration. Then mitochondrial dysfunction. Major causes of death are cardiac abnormalities, cardiomyocytes dystrophin absence results in membrane instability and mitochondrial dysfunction. Laboratory findings include high transaminases, very high CK, pathological electroneurography, NCV prolonged and an early detection of respiratory loss syndrome. Due to its X-linked

recessive inheritance, almost only boys are affected [2-32]. However, female carriers of this gene can also show symptoms, such as in the cardiac area, and occasional monitoring for cardiomyopathy using echocardiography or cardiac MRI as well as ECG is recommended for these women from the age of 25-30. Duchenne muscular dystrophy begins in early childhood with weakness of the pelvic and thigh muscles and progresses rapidly. With optimized therapy including home ventilation and the use of corticosteroids in early childhood, the prognosis has improved in recent years, and life expectancy has also increased due to improved medical care. Just a few years ago, most patients died in adolescence. Today, reaching the third decade of life is common. Histopathological image of a cross-section through the calf muscle of a patient with Duchenne muscular dystrophy. Duchenne muscular dystrophy is a genetically determined synthesis disorder of the muscle structural protein dystrophin. It is not produced in this form of the disease. In contrast, in the milder form of muscular dystrophy, Becker-Kiener dystrophin is produced in a shortened but partially functional variant. The lack of dystrophin leads to the degeneration of muscle fibers over time and their replacement by fat or connective tissue. About 1/3 of cases are de novo mutations, and only 2/3 are inherited from the unaffected mother (carrier). The course of the disease varies in severity, but early childhood development is usually normal. Depending on the severity of the disease, mild muscle weakness of the legs may be noticeable from the age of 3 to 5, leading to frequent stumbling and falling. As the disease progresses, climbing stairs may only be possible with the help of a railing. Muscle weakness of the pelvic and thigh muscles can lead to a waddling gait (Trendelenburg sign) and make it difficult to rise from sitting or lying down. Children support themselves on their thighs when rising (Gowers maneuver) or use walls and furniture for support. In severe forms, from the age of 5 to 7, climbing stairs and getting up from sitting or lying down may only be possible with the help of others, as the disease also affects the muscles of the shoulders and arms. Between the ages of 7 and 12, lifting the arms to a horizontal position is hardly possible. Many children at this age already rely on a wheelchair but can still care for themselves to some extent. In severe cases, complete dependency on care may occur from the age of 18. Due to muscle wasting, painful joint deformities and bone deformations occur. Characteristic of Duchenne type are the so-called “gnome” or “ball calves.” They are caused by fat deposits in the connective tissue-restructured calf muscles (pseudohypertrophy). When the shoulder girdle muscles break down, protruding shoulder blades (“scapulae alatae”), also known as angel wings, occur. Weakness of the respiratory muscles makes coughing difficult, especially during airway infections, significantly reducing life expectancy. Although the heart muscle is usually affected by the disease process, increased heart rate and other rhythm changes or impairment of heart function rarely cause subjective symptoms. The life expectancy of patients is about 40 years, but some patients may die before puberty. In 50-95% of cases, a lateral curvature of the spine (scoliosis) also develops, usually when walking ability is lost, typically between 10 and 14 years of age. In about 85% of affected children, scoliosis initially progresses with an average increase in the Cobb angle, which describes the extent of the misalignment, by 2.1° per month. This also impairs lung function, and forced vital capacity decreases by about 4% for every 10° increase in scoliosis. It is possible to halt the progression and reduce lung volume by performing a long-segment spinal fusion spondylodesis. However, the procedure carries risks, and its significance is not clear. A Scottish retrospective study examined the complication rate in 26 posterior spondylodesis surgeries. On average, the boys were 14.2 years old, the surgery lasted an average of 260 minutes, the average postoperative stay in the intensive care unit was five days, and the hospital stay was fifteen days. Complications were significantly more common than in comparable surgeries for other underlying conditions and occurred in nearly 40 per cent of patients, including four cases of acute liver damage and five deep wound infections. The suspicion of muscular dystrophy arises when an unusual, symmetrically developed muscle weakness is observed in childhood. The onset of functional disorders, the course, and the occurrence of similar disorders in the family, especially in male relatives of the mother, are of particular interest in the medical history. During the physical examination, general abnormalities such as posture, mobility, and breathing are assessed. The neurological examination tests the function of nerves and muscles. Laboratory values provide important clues - the concentration of transaminases is often elevated, but the concentration of creatine kinase, a muscle enzyme, is significantly increased, often exceeding 10-100 times the normal range. However, an elevated creatine kinase level does not replace further diagnostics, as there may be other causes. Additional tests include electroneurography, which determines nerve conduction velocity, and electromyography, which helps differentiate between primary muscle disease and motor nerve disease as the cause of weakness. MRI and ultrasound are used as imaging techniques. Structural muscle changes can be assessed without stressing the patient. Muscle biopsy allows examination of the muscle under a light or electron microscope, enabling a detailed study of muscle metabolism. Genetic testing confirms the diagnosis and allows for precise classification by type. This genetic diagnosis is important because it provides information on possible therapies. Not all therapies are suitable for all Duchenne patients. Many deaths in DMD patients are due to a decline in respiratory function. Early detection of respiratory function loss in DMD is crucial. In the early stages, declining respiratory function is asymptomatic. In cases of impaired respiratory function, measurements usually decrease by around 5% per year. DMD patients with respiratory function limitations are at increased risk for sometimes severe and life-threatening respiratory infections. To prevent respiratory infections due to accumulated mucus systems can be used to assist coughing. This involves directing air into the lungs via automatic airflow and quickly suctioning it out, making coughing much more efficient. Current therapeutic options cannot sufficiently influence the progressive decline in respiratory function in DMD. Regular monitoring of respiratory function increases the chances of slowing progression through early interventions. The increasing loss of muscle strength irreversibly reduces respiratory function and is responsible for many deaths in DMD.

Therapy Options

Conservative Treatment

Ergotherapy, physiotherapy and orthopaedic approaches are conservative management options for the involved children at first [7,11].

Drug Applications

Treatment by corticosteroids like Deflazacort (EMFLAZA) or Vamoloron (VBP-15)-steroid analogon are helpful medical drug to get a relief for muscular functional delay and inflammation. Vamorolon has anti-inflammatory benefits, a better safety profile and takes care for the bone mineral density in children. Givinostat, a histone deacetylase inhibitor had a positive outcome of PHASE III EPIDYS trial and was approved in the United States for children with DMD [5]. Cardioprotective agents include ACE inhibitors, beta blockers and angiotensin receptor blockers.

Approved Gene Therapy Options to Date

Gene therapy approaches include ELIVIDYS, delandistrogene moxeparvovec, a AAV vector with truncated dystrophin. It is a new and first gene therapy option in the US for boys aged 4-5 years despite no functional improvement of motoric function has been found. Recently, liver failure in three patients have been described. The data are too early for long term application of this new gene therapy option.

Genetic Restoration Strategies for Dystrophin Expression in Muscle and Heart

Antisense Oligonucleotide (ASO) mediated Exon Skipping Approach

In Duchenne muscle dystrophy, treatment with ASO (usually exon 45, 51, 53), are highly personalized for specific patients with specific mutations [22-32]. Skipping defective parts of the dystrophin gene with the insertion of microdystrophin to stabilize muscle fibers is the point of research. The process restores the reading frame in the dystrophin mRNA, leading to the production of a truncated partially functional protein. Most ASOs belong to phosphorodiametate morpholino oligomers (PMO) that induce exon skipping and restore the reading frame. There are 4 different ASOs available for different mutations: Eteplirsen (Exondys 51, Sarepta Therapeutics) is approved in the USA in 2025 and targets exon 51 by skipping. Golodirsen (Vyondys 53, SRP-4053) uses exon 53 skipping, Casimersen (Amondys-45, SRP-4045) induces exon 45 skipping. Viltolarsen (Viltepso) skips exon 53 and is approved in Japan since March 2020. Improvement in the 6-minute walk test as a measure of motor function is not performed, but higher dystrophin levels are observed. Multiple injections are necessary to achieve the maximum cumulative antisense effect with restoration of dystrophin expression, but not in cardiac tissue. Overall, ASO therapy restores a shorter dystrophin with modest efficacy, requiring continuous dosage due to the short lifespan of ASOs. There is no functional impact on cardiac tissue. Paradoxically, there is increased functional activity with further deterioration of heart function.

Conjugation of ASO with Nanoparticles, Peptides and Polymers (PPMO)

3 PPMOs have been evaluated: Vesleteplirsen (SRP-5051) targets exon 51 connected with a linear peptide.

ENTR-601-044, developed by Entrada Therapeutics, targets exon 44 with a cyclic peptide but seem to have potential of toxicity. AB-Transferrin receptor 1, evaluated to date in EXPLORE44-Trial, targets exon 44 by exon skipping. BMN 351, developed by BioMarin, targets exon 51 by skipping.

Vector-mediated Gene Therapy and Split Intein Approaches

DMD has the largest known gene, an 11.2 kb mRNA coding sequence, making it impossible to load onto adeno-associated viral vectors, which can only carry 4.7 kb. By removing the central rod domain, AAV vector transfer is possible allowing delivery of microdystrophin. Recent research focus on multi vector delivery approaches to deliver the whole dystrophin gene in the cells by more than one vector. New future option is the multiple application of AAV-gene transfer viral vectors. Triple inteins are small bacterial polypeptides, which usage of two separate protein fragments, called exteins, which will be expressed from two different viral vectors, that full dystrophin can be delivered.

Microdystrophin-Gene Therapy (AAV micro Dys, 1/3 of full dystrophin) and CRISP-Cas 9 Therapy

Trials with several small dystrophins carried by viral vectors have been experimentally tested (12). Significant advancements in muscle-specific CK8e and MHCK7 promoters, providing robust expression in skeletal and cardiac muscle cells, especially AAV9-CK8e-microdystrophin that is better for cardiac cells. FDA approved this therapy in 2024, with improvement in the 10-meter walk and time to rise from the floor. Several limitations with potential activation of immune responses due to AAV capsid proteins, unmethylated CpGs and cytotoxic T-lymphocyte immune response plays a crucial role in liver toxity, myositis and myocarditis, microangiopathy and renal failure. A strong type 1 interferon reaction (strong IFN 1 response) was recently described. Using CRISP-CAS 9 therapy have a well-known danger for immune reaction to bacterial CAS enzymes. The presence of antibodies against AAV with binding antibodies in 100 per cent and neutralizing antibodies in 30-50 per cent was recently described. Patients with high levels of neutralizing antibodies were removed from trial. Muscle tissue includes 40 per cent of body mass and makes a high dosage of AAV necessary. Trial are planned to first remove different antibodies, then introducing a gene therapy by AAV-vector system (Phase 1, Sarepta Therapeutics). The next generation micro Dys gene therapy includes minimizing immunogenicity by removing H1 from AAV. H1 carries a dominant immunogenic epitope associated with serious fulminant side effects. Main advantage compared to ASO based skipping is that CRSIP Cas9 functions directly at the genome level.

Offering one-time treatment, uses CAS9 nuclease to split double strang near mutational region. It induces therefore alternative splicing. Novel approaches are modified CAS9 fully devoided by nuclease activity for specific repair of point mutations. Moreover, the usage of inactive CAS9, "dead CAS9" with mutated HNH domain is under research. Base editing can correct maximum 6-point mutations. CRISP CAS capacity to accelerate generation of new animal models are focus of recent research.

Combination of Exone Skipping and Gene Therapy

With the usage of U7 Small Nuclear Ribonucleoprotein (snRNP) cassette engineered the induction of exon skipping by AAV mediated delivery or by lentiviral modification of muscle progenitor cells was introduced in recent trials.

Discussion

Duchenne Muscular Dystrophy (DMD) is the most common of more than 30 chronic, genetically inherited muscle diseases grouped under the term "muscular dystrophy." In all of these conditions, progressively more functional muscle tissue is lost. Duchenne muscular dystrophy begins in early childhood and progresses slowly over years. Without proper treatment, it can lead to death in early adulthood by affecting the respiratory and cardiac muscles. Duchenne muscular dystrophy is rare but the most common form of muscular dystrophy, affecting about one in 3,500 newborn boys. The first signs of DMD appear between the ages of two and six. BMD typically manifests after the age of 7, often up to the age of 20, but in rare cases, symptoms may not appear until middle to late adulthood. DMD is caused by a genetic defect in the dystrophin gene, leading to the body's inability to produce dystrophin protein. Dystrophic skeletal muscle phenotypes can be horizontally transferred by faecal microbiome transplantation [8]. Dystrophin is +muscular dystrophy results in the gradual breakdown of muscle fibers. The genetic defect is inherited on the X chromosome. The dystrophin gene, defective in DMD and BMD, is located on the X chromosome, the sex chromosome. Women typically do not develop muscular dystrophy because they have two X chromosomes in their body cells. Even if one is defective, carrying the defective dystrophin gene, the healthy X chromosome usually takes over while the defective one remains inactive. However, in rare cases, functional inactivation of the healthy X chromosome can occur, leading to approximately 5-10% of female carriers developing symptoms of a mild form of muscular dystrophy or heart muscle disorder. Women carrying the mutated dystrophin gene can pass the defective X chromosome to their sons. Boys (or men) have one X chromosome from the mother and one Y chromosome - the male sex chromosome inherited from the father. Since there is no second X chromosome as a healthy "alternative," Duchenne muscular dystrophy always manifests in boys if they inherit the underlying genetic defect from their mother. The progressive muscle wasting in DMD primarily affects the pelvic and thigh muscles.

The aspects of "treatment" and "cure" have to be separated completely when treating paediatric patients with Duchenne muscular atrophy. Gene therapy approaches include ELIVIDYS, the first gene therapeutic option approved in the US, showed serious side effects in 3 patients, that overall, too fast approvals of these gene therapeutical should be closer evaluated for their benefit and their potential to produce serious side effects.

Moreover, vector-based gene transfers are related to serious side effects the more vectors are used [9,10,13-14-18,20-21]. Therefore, split intein approaches are at risk to develop more immunologic side effects then delivered by one vector. One vector cannot be the only part to deliver the whole dystrophin gene, because it is too big. From my viewpoint, the best way to treat these children is an ASO-based exone skipping of the involved exone to produce a smaller amount of dystrophin gene and later a CRISP-Cas9 based gene therapy with repairing the individual mutation [3,4,11,19]. Another interesting curing aspect is the delivery of a functioning gene by transcytotic transportation of size-controlled nanocarriers into dystrophic skeletal muscle or microroboters in the cells where it is necessary [2]. Research efforts to treat or cure the disease are extraordinary and extensive and based on different therapeutic options.

Ethics Approval and Consent to Participate

Not applicable.

Acknowledgment

Not applicable.

Funding

None.

Conflict of Interest

The authors declare no conflict of interest.

References

1. Konagaya M (2025) [Duchenne de Boulogne: Pioneer of Neurology]. Brain Nerve 77(12): 1267-1274.
2. Liu X, Hicks MR, Feng Z, Jiang J, Li Y, et al. (2025) Transcytotic transportation of size-controlled nanocarriers into dystrophic skeletal muscle leads to therapeutic outcome in mice. Nat Commun 16(1): 11217.
3. Lyu G, Li P, Lang W (2025) A review of recent studies on CRISPR/Cas9-mediated genome editing in a variety of muscle-related genetic disorders. J Transl Med 23(1): 1381.
4. Aartsma Rus A (2025) Report of the multistakeholder drug development round table meeting of the World Duchenne Organization focusing on challenges for clinical development of therapies. J Neuromuscul Dis 4:22143602251399511.
5. Bertoldi M, Albamonte E, Bello L, D'amico A, Masson R, et al. (2025) Histone deacetylases in Duchenne muscular dystrophy: a role in the mechanism of disease and a target for inhibition. Clin Epigenetics 18(1): 9.
6. Karuppasamy M, English KG, Conner JR, Rorrer SN, Lopez MA, et al. (2025) Conditional Dmd ablation in muscle and brain causes profound effects on muscle function and neurobehavior. Commun Biol 8(1): 1736.
7. Zhao L, Pan S, Hu C, Shi Y, Li X (2025) From shadows to clarity: A new paradigm for comprehensive variant detection in undiagnosed dystrophinopathy using combined long-read and RNA sequencing. Comput Struct Biotechnol J 27: 5036-5044.

8. Butcher J, Gosse JT, Gobin J, Ravel-Chapuis A, Jasmin BJ, et al. (2025) Dystrophic Skeletal Muscle Phenotypes Can Be Horizontally Transferred via Fecal Microbiome Transplantations. FASEB J 39(23): e71281.

9. Osman MF, Ibrahim K, Gleeson C, Ibrahim H, Haque IU, et al. (2025) Real-world experience with gene therapy in Duchenne muscular dystrophy center readiness and patients safety: report from Qatar. Gene Ther.

10. Johnson EE, Reyes TR, Chamberlain JS, Ervasti JM, Tasfaout H (2025) Proteomics-based evaluation of AAV dystrophin gene therapy outcomes in mdx skeletal muscle. JCI Insight: e197759.

11. Mcleay L, Hardinge S, Minor KM, Friedenberg SG, Cullen JN, et al. (2025) Dystrophin-Deficient Muscular Dystrophy in a Family of Shiba Inu Dogs with a Complex Deletion Encompassing DMD Exon 5. Genes (Basel) 16(11): 1369.

12. Dias M, Dhuyvetter H, Byttebier E, Merckx C, De Bleecker JL, et al. (2025) Early Taurine Administration Decreases the Levels of Receptor-Interacting Serine/Threonine Protein Kinase 1 in the Duchenne Mouse Model mdx. Brain Sci 15(11): 1175.

13. Rostamitehrani Z, Javed R, Popplewell L (2025) Dystrophin Restorative and Compensatory Gene Addition Therapies for Duchenne Muscular Dystrophy: Could CRISPRa Provide a Realistic Alternative? Muscles 4(4): 52.

14. Benchetrit S, Chu C, Athanasiou D, Scherer S, de Andres Trelles F, et al. (2025) Paediatric medicines development for Duchenne muscular dystrophy: An EU regulatory perspective. J Neuromuscul Dis: 22143602251397736.

15. Biswas DD, Han NH, Hernández Rodríguez MY, Lee J, Abdelbarr SM, et al. (2026) AAV9 gene therapy to target respiratory insufficiency in the D2.mdx mouse model of duchenne muscular dystrophy. Respir Physiol Neurobiol 340: 104513.

16. Esper ME, Lin AYT, Bennett D, Rudnicki MA (2025) Intrinsic dysfunction in muscle stem cells lacking dystrophin begins during secondary myogenesis. Nat Commun 16(1): 10031.

17. Gallert SM, Fölsch M, Mavrommatis L, Kindler U, Schork K, et al. (2025) Proteomics of Duchenne Muscular Dystrophy Patient iPSC-Derived Skeletal Muscle Cells Reveal Differential Expression of Cytoskeletal and Extracellular Matrix Proteins. Cells 14(21): 1688.

18. Dowling P, Bouragba D, Negroni E, Trollet C, Zweyer M, et al. (2025) Proteomic Profiling of Myofiber Repair Annexins and Their Role in Duchenne Muscular Dystrophy. Proteomics: e70073.

19. van der Pijl EM, Pasteuning Vuhman S, Boertje van der Meulen J, Niks EH, Verschuuren JJGM, et al. (2025) Antisense-mediated exon skipping therapy improves neuromuscular junction deficits in a Duchenne muscular dystrophy mouse model. Neuromuscul Disord: 56-57.

20. Tomasini S, Monteleone E, Altieri A, Margiotta F, Dardmeh F, et al. (2025) microRNA-22 Inhibition Stimulates Mitochondrial Homeostasis and Intracellular Degradation Pathways to Prevent Muscle Wasting. Int J Mol Sci 26(20): 9900.

21. Tang A, Yokota T (2025) Brogidirsen and Exon 44 Skipping for Duchenne Muscular Dystrophy: Advances and Challenges in RNA-Based Therapy. Genes (Basel): 16(7): 777.

22. Hashimoto Y, Aoki Y (2025) Electrophysiological Evaluation in mdx52 Mouse Brain After Antisense-Mediated Exon 51 or 53 Skipping. Methods Mol Biol 2964: 277-282.

23. Shah MNA, Wilton Clark H, Haque F, Powell B, Sutanto LE, et al. (2025) DG9 boosts PMO nuclear uptake and exon skipping to restore dystrophic muscle and cardiac function. Nat Commun 16(1): 4477.

24. Phongsavanh M, Bizot F, Saoudi A, Gastaldi C, Le Coz O, et al. (2025) Valproic Acid Improves Antisense-Mediated Exon-Skipping Efficacy in mdx Mice. Int J Mol Sci 26(6): 2583.

25. Patel Y, Orogun L, Yocum N, Rodino Klapac LR, East L (2025) A Population Pharmacokinetic Model to Inform Extension of the Eteplirsen Dosing Regimen Across the Broad DMD Population. CPT Pharmacometrics Syst Pharmacol 14(5): 891-903.

26. Blitek M, Gastaldi C, Doisy M, Le Coz O, Tensorer T, et al. (2025) Combined 20-Hydroxyecdysone and Antisense-Mediated Exon Skipping Improve Functional Outcomes in a Mouse Model of Duchenne Muscular Dystrophy. Nucleic Acid Ther 35(2): 55-67.

27. Oppeneer T, Qi Y, Henshaw J, Larimore K, Melton A, et al. (2025) Targeting a Novel Site in Exon 51 with Antisense Oligonucleotides Induces Enhanced Exon Skipping in a Mouse Model of Duchenne Muscular Dystrophy. Nucleic Acid Ther 35(2): 68-80.

28. Chwalenia K, Wood MJA, Roberts TC (2025) Progress and prospects in antisense oligonucleotide-mediated exon skipping therapies for Duchenne muscular dystrophy. J Muscle Res Cell Motil 46(4): 293-300.

29. Oppeneer T, Qi Y, Henshaw J, Larimore K, Puoliväli J, et al. (2025) BMN 351-Induced Exon Skipping and Dystrophin Expression in Skeletal and Cardiac Muscle Lead to Preservation of Motor Function in a Mouse Model of Exon 51 Skip-Amenable Duchenne Muscular Dystrophy. Nucleic Acid Ther 35(2): 81-92.

30. Komaki H, Takeshita E, Kunitake K, Ishizuka T, Shimizu Motohashi Y, et al. (2025) Phase 1/2 trial of brogidirsen: Dual-targeting antisense oligonucleotides for exon 44 skipping in Duchenne muscular dystrophy. Cell Rep Med 6(1): 101901.

31. Doisy M, Vacca O, Fergus C, Gileadi T, Verhaeg M, et al. (2023) Networking to Optimize Dmd exon 53 Skipping in the Brain of mdx52 Mouse Model. Biomedicines 11(12): 3243.

32. Aartsma Rus A, De Waele L, Houwen Opstal S, Kirschner J, Krom YD, et al. (2023) The Dilemma of Choice for Duchenne Patients Eligible for Exon 51 Skipping The European Experience. J Neuromuscul Dis 10(3): 315-325.