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Application of Personalized Medicine in the Treatment of Duchenne Muscular Dystrophy (DMD)

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Abstract: *Duchenne muscular dystrophy (DMD) is a severe X-linked neuromuscular disease due to pathogenic variants in the DMD gene, leading to progressive skeletal and cardiac muscle degeneration. DMD is a genetically heterogeneous disorder, and the treatment has moved from supportive to a mutation-driven individualized approach to medicine. Precision interventions involve antisense oligonucleotides (ASOs, exon skipping), nonsense readthrough therapeutics, adeno-associated virus (AAV) mediated micro-dystrophin replacement, genomics editing and monitoring, and patient-derived model systems for drug testing. Personalized medicine in DMD not only involves choosing the right drug, but also selecting the right patient, the right mutation-targeted approach, and the right time of intervention. The field is also growing with the use of AI and bioinformatics, enhancing the interpretation of mutations, disease prediction and trial stratification. This review highlights the pathogenesis, prevalence and molecular basis of DMD and critically analyses the impact of personalized medicine on the DMD treatment, emphasizing translation, limitations and future treatment directions.*

Keywords: Duchenne muscular dystrophy, personalized medicine, precision therapy, dystrophin, exon skipping, gene therapy, CRISPR, biomarker, bioinformatics, AI

I. INTRODUCTION

DMD is one of the most significant model disease for personalized medicine as the clinical phenotype is directly determined by the molecular lesion, while the latter has a high degree of heterogeneity. DMD does not represent a single therapy target, but a family of disease phenotypes with a common final pathway: dystrophin deficiency and progressive muscle failure [1, 5]. That fact is underscoring the growing importance of mutation specific treatments.

What is important in DMD is not just that the muscle loses strength, it is a loss of a muscle structural protein that is responsible for the stability of the sarcolemma, and for preventing damage from muscle contraction. With the absence of dystrophin, the muscle fibre goes through a cycle of injury, an overload of calcium, an inflammatory response, and fibrotic replacement [1] [3]. This process involves skeletal muscle, myocardium and respiratory muscles; the disease thus becomes multisystemic and inexorably progressive [1, 6].

There are three reasons why personalized medicine is key in DMD. The DMD gene is very large, very prone to mutations, and mutations result in deletions, duplications, nonsense variants, splice defects and small insertions and deletions, none of which is uniformly responsive to the same therapy [4, 5]. Second, several currently available treatments are mutation-restricted, in which case the eligibility for treatment is dependent on precise molecular diagnosis [7]–[12]. Third, the timing for intervention is critical: precision therapies are more effective at an earlier stage before irreversible fibrosis and cardiopulmonary decline occurs [1], [6], [22].

As such, there is growing recognition that DMD is a precision neuromuscular disorder and not a one-size-fits-all muscular dystrophy. This change is particularly significant in countries like India where delayed diagnosis, under-

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ascertainment and restricted availability of molecular treatments continue to be an obstacle to individualized treatment [20, 21].

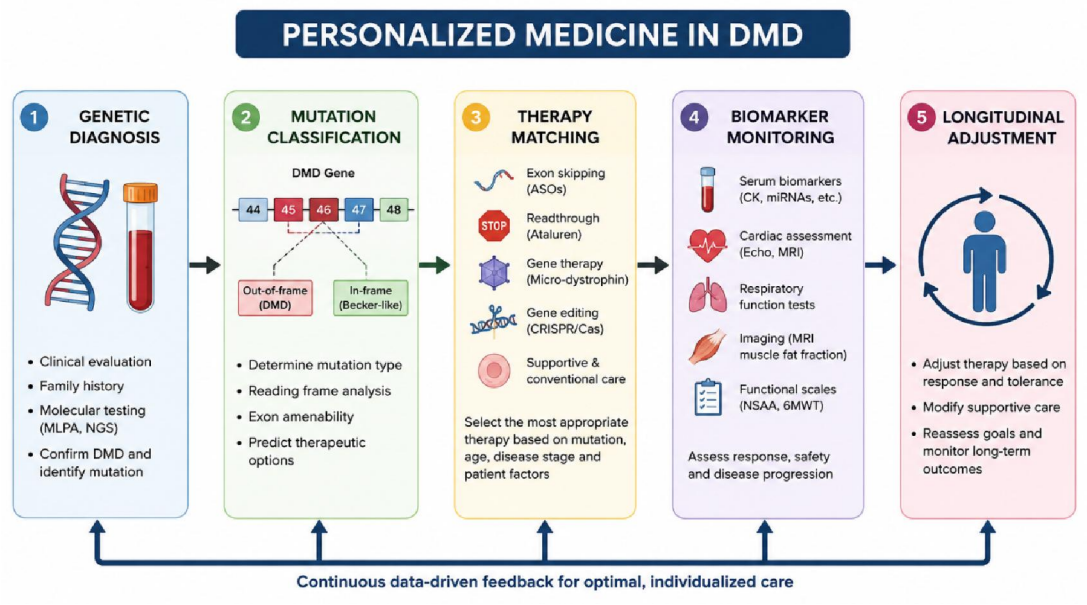

Figure 1: Conceptual framework of personalized medicine in DMD

II. DMD: DISEASE OVERVIEW AND PATHOGENESIS

DMD is an X-linked recessive dystrophinopathy due to loss-of-function mutations in the DMD gene on Xp21.2 that encodes dystrophin, a large cytoskeletal protein that is important for maintaining the stability of muscle membranes [1, 3, 4]. Disease occurs mainly in boys and female carriers may sometimes have symptoms if there is a skewed X-inactivation or disease may be a manifestation of carrier status [6].

Clinically, the onset of DMD is generally in early childhood, when there are delays in motor milestones, running difficulties, frequent falls, proximal muscle weakness, pseudohypertrophy of the calves and a positive Gowers' sign [1, 6]. As the disease progresses, children become unable to walk independently, suffer from scoliosis, respiratory insufficiency, dilated cardiomyopathy and ultimately die from the cardiopulmonary complications if disease modification is not sufficient [1], [22].

Pathogenetically, dystrophin is an important component of the dystrophin-glycoprotein complex that connects the actin cytoskeleton to the extracellular matrix. Without this, the muscle fibres become mechanically weak and each contraction causes micro-tears or openings in the sarcolemma and an uptake of calcium ions [3,4]. Calcium dysregulation triggers the activation of calpains and other proteolytic pathways, triggering damage to mitochondria, elevated R.O.S. levels and necrosis [1]. Chronic damage invokes inflammatory cells, triggers fibro-adipogenic pathways and ultimately leads to fibrotic and fatty replacement of functional muscle tissue [1, 6].

This is because DMD is a progressive disease despite the underlying mutation being static. The mutation is permanent and the downstream injury-response cycles exacerbate tissue injury. That is, a purely symptomatic approach is inadequate biologically because the target is not weakness alone, but the molecular cascade that produces weakness [1, [22]].

DMD mutation leads to lack of dystrophin, which causes the sarcolemmal fragility, allowing for calcium entry, initiating oxidative stress, leading to inflammation, fibrosis and subsequent muscle degeneration.

The disease is also cardiac because cardiac cells called cardiomyocytes depend on dystrophin to maintain their mechanical ability. Consequently, the progression of cardiomyopathy can be asymptomatic even with seemingly

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normal skeletal muscle management [1] [22]. Neurocognitive and behavioral symptoms have also been reported, consistent with the general pattern of dystrophin expression in the CNS [6]. Hence DMD should not be viewed as a solely skeletal myopathy.

Table 1: Major pathobiological events in DMD

Pathobiological event	Consequence	Relevance to personalized therapy
Dystrophin loss	Sarcolemmal fragility	Requires mutation-specific correction
Calcium overload	Protease activation, mitochondrial injury	Targets for disease-modifying adjuncts
Chronic inflammation	Ongoing muscle necrosis	Supports anti-inflammatory background therapy
Fibrosis and fat infiltration	Irreversible tissue replacement	Reduces response ceiling for late treatment
Cardiac involvement	Cardiomyopathy and arrhythmia	Requires early surveillance and individualized care

III. PREVALENCE OF DMD: GLOBAL AND INDIAN PERSPECTIVES

Globally, DMD is the most common severe muscular dystrophy in males. Meta-analysis indicates a pooled prevalence of ~7.1 per 100,000 males and a pooled birth prevalence of ~19.8 per 100,000 live male births [2]. Estimates of incidence of the conventional method are around 1 in 3,500 to 5,000 live male births; these rates vary with ascertainment methods, geographic location, and availability of diagnostic testing [1, 2]. The apparent burden is more stringent in places where better genetic diagnosis and longer survival means that there are more people living who have an affected heart or lungs at any time point [1, 22].

However, the actual prevalence is difficult to define in India due to lack of nationally bio-registered population databases and the accessibility of confirmatory testing. There are significant numbers of patients and a trend towards delayed diagnosis, as suggested by available hospital-based studies. For instance, in a tertiary care series from India, the clinically significant diagnostic delay and the proportion of cases with advanced presentation in the hospital were observed [20, 21]. The primary issue is not the lack of detection, but under-detection; that is, many children are diagnosed only after the loss of function has already started, and that is when the value of mutation-specific intervention is reduced.

Whereas in personalized medicine prevalence is important because it marks the size of the population that can be treated, diagnostic delay is even more important because precision therapies are more effective prior to irreversible muscle remodeling [1, 6]. In India, the problem of presentation is that it comes late, molecular testing is not widely available, and cost of treatment is high, resulting in the availability of treatment being far less than the theoretical level [20] and [21].

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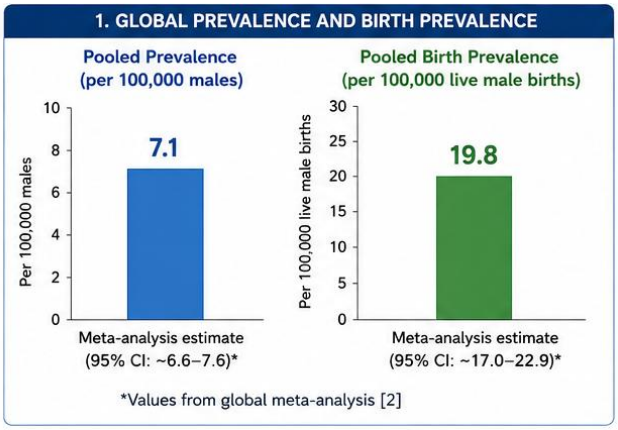

Figure 2: Global DMD Prevalance

IV. WHY DMD IS A MAJOR DISEASE OF CONCERN

DMD is a serious problem because it is fatal, pediatric, progressive and costly. It leads to early childhood disability, chronic dependency, frequent hospitalizations and severe psychosocial burden for families [1, 22]. The disease also leaves a long-lasting therapeutic tail: even if a mutation-specific intervention is available, patients are still in need of ongoing medical care to conditions such as the respiratory system, the heart, the joints, nutrition and rehabilitation.

The cardiac phenotype is especially important. As dystrophin is absent in cardiomyocytes, it contributes to fibrosis, arrhythmias and dilated cardiomyopathy, which may be the biggest cause of morbidity and mortality [1], [22]. Respiratory muscle failure is also significant since, if left untreated and unattended, a hypoventilation state during sleep will progress to ventilatory failure [22]. DMD is not only a muscle disease, but a progressive failure of many systems.

It is a rare disease and its scientific and clinical value is that it is a prototype of precision medicine in a rare disorder. The DMD can be divided into a number of therapeutic subcategories based on exon deletions, nonsense variants or editing targets [5], [7] – [12]. In this respect, it has turned out to be a testing ground for the rest of the field of personalized therapeutics.

V. GENETIC BASIS AND MOLECULAR MECHANISM OF DMD

One of the largest genes in the human genome is the DMD gene, which encodes dystrophin, a large structural protein consisting of several domains, such as N terminal actin binding domain, central rod domain, cysteine rich domain and C terminal domain [3, 4]. It is a large organism that is susceptible to mutations. Mutations can be inherited from carrier mothers, occur de novo, and range from exon deletions and duplications to nonsense mutations, splicing defects and small insertions and deletions [1, 5, 6].

The most important genotype–phenotype correlation is that out-of-frame mutations typically prevent dystrophin production, leading to DMD, whereas in-frame mutations allow for the production of a truncated but partially functional dystrophin and are more commonly associated with Becker muscular dystrophy [5, 6]. This is the molecular rationale behind exon-skipping therapy. In certain cases, the reading frame is not restored when specific exons are skipped in pre-mRNA processing, but instead a Becker-like dystrophin transcript is created [7]–[12].

The mechanism of dystrophin deficiency is that it destabilizes the dystrophin-glycoprotein complex allowing the sarcolemma to become vulnerable to damage during contraction [1, 3]. Protease activation, mitochondrial stress, ROS accumulation and repeated myofiber necrosis is brought about by calcium influx [1]. This is an ongoing injury response which causes inflammation and fibrosis, leading to a progressive loss of regenerative capacity in the muscle [1, 6]. Early diagnosis is very important because if fibrosis is advanced, even if function is restored, it may only be partially restored.

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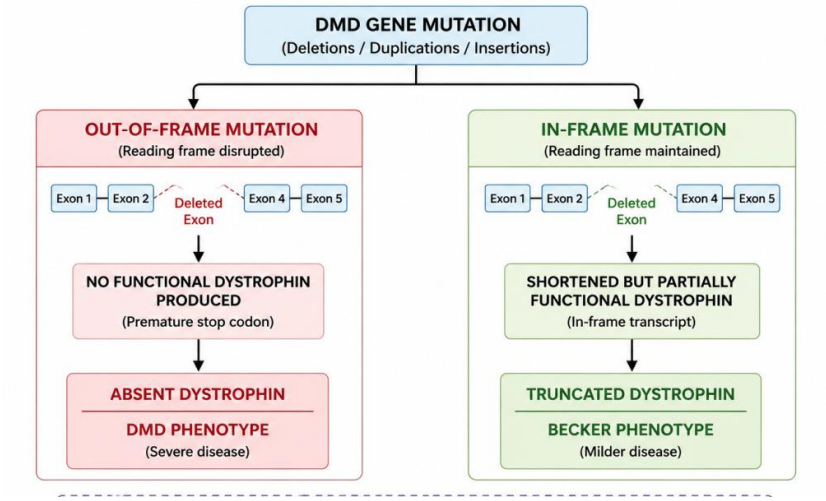

Figure 3: Reading frame Principle in DMD

Table 2: Mutation classes and precision therapy relevance

Mutation class	Approximate therapeutic relevance	Example precision strategy
Exon deletions	Often exon-skippable	Exon-skipping AONs
Nonsense mutations	Readthrough amenable	Ataluren
Duplications	May be editing-relevant	CRISPR-based correction
Splice-site defects	RNA-level correction possible	Splice modulation / editing
Complex variants	Variable	iPSC-based testing and bioinformatics-guided selection

VI. PRECISION DIAGNOSTICS AS THE FOUNDATION OF PERSONALIZED MEDICINE

Accurate molecular diagnosis is the first step to personalized treatment. If no specific mutation is characterized, there is no way to choose mutation specific therapy [6]. The multiplex ligation-dependent probe amplification (MLPA) technique is very valuable to detect exon deletions and duplications, and next generation sequencing (NGS) is crucial for point mutations, nonsense variants and splice defects [6]. Many workflows are not mutually exclusive, but rather complement each other to the benefit of both MLPA and NGS.

Biomarker-guided medicine is also increasingly important. Diagnosis, monitoring and therapeutic response assessment is facilitated by measuring serum creatine kinase, circulating proteins, microRNAs, and imaging-derived metrics [18]. Biomarkers have relevance in DMD because many therapies give only partial restoration of dystrophin, and clinicians must have outcome measures that are more sensitive than a simple clinical observation. Baseline biomarker profile stratification can also help identify who will benefit most from a particular intervention [18].

Precision care with imaging, particularly quantitative MRI, is able to detect fat replacement and the fibrotic burden before they become evident functionally [17, 18]. In clinical terms, biomarker and image data are used to define the therapeutic window and to ensure that treatment is not delayed when there is little salvageable muscle remaining.

VII. PERSONALIZED MEDICINE IN DMD TREATMENT

A. Conventional treatment as the foundation

Although genomic medicine is available, conventional care cannot be dispensed with. The use of glucocorticoids such as prednisone and deflazacort has been the standard of care due to a reduction in the rate of functional decline and a delay in loss of ambulatory function, but they have not been shown to reverse the initial mutation [1, 22].

Background interventions required include cardiac medications, respiratory support, physiotherapy, orthopedic management and nutritional care.

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Conventional therapy is not mutation-specific, but stage-specific and toxicity-aware personalization. Steroids regimen, dosage and monitoring should be based on age, ambulatory status, growth, bone health, behavioral effects, and cardiac risk [1] [22]. That is, even "treatment" needs to be tailored to the individual.

B. Exon-skipping therapy

The most well defined case of mutation-targeted therapy in DMD is known as exon-skipping antisense oligonucleotides [7]– [11]. The therapy is based on the idea that restoration of the open reading frame can be achieved by the deletion of a selected exon from the pre-mRNA, so that a shortened but still functional dystrophin will be synthesized. This approach is highly individualised (only for patients with mutations that are compatible with the drug).

Eteplirsen is designed for mutations that can be targeted in exon 51 [7, 8]. Both golodirsen and viltolarsen target exon 53 [9], [10]. Casimersen targets exon 45 [11]. These therapies are not generally substitutable, and are only available to patients depending on the mutation profile of that patient. This is what makes DMD a textbook case of precision medicine.

The restrictions are significant as well. Exon skipping (partial restoration of dystrophin) is only effective at low levels, and needs to be repeated [7]–[11]. Therapeutic response can also vary based on stage of disease, degree of fibrosis and muscle delivery to the affected muscle, particularly the heart. Therefore, the clinical benefits of the same genotype cannot be assumed to be the same.

C. Nonsense readthrough therapy

Ataluren is formulated for nonsens mutations that result in a premature stop codon. It induces ribosomal readthrough, which can result in functional dystrophin [12]. Another type of personalized medicine and another strategy for the treatment of mutations. It is dependent of the context of the variants, stability of the transcripts and disease stage, and it can only be applied to a specific group of patients [12].

D. Gene replacement therapy

The most important advance is micro-dystrophin gene therapy which tries to correct the lack of dystrophin function through a compact AAV delivered construct [13, 14].

Study of rAAVrh74 systemically delivered to humans (landmark).The feasibility and clinical foundation of MHCK7.micro-dystrophin were generated [13] and were the basis for later approval pathways. In 2024 FDA expanded the use of Elevidys (delandistrogene moxeparvovec-rokl) to ambulatory and non-ambulatory patients 4 years of age or older with a confirmed DMD mutation [14]. That approval was a significant step towards precision genomic therapy for DMD.

Gene therapy is also an example of the potential limitations of precision medicine. It is not equally accessible to all patients, it can be limited by immune reactions and liver toxicity, and it does not replace the complete-length dystrophin protein [13, 14]. The therapy is therefore transformative, but not in the strict sense a cure.

E. Gene editing with CRISPR/Cas systems

The most conceptually robust personalized approach is for the mutation that causes the disease to be corrected on the DNA level (CRISPR based correction) [15]. These involve methods of exon excision, frame restoration, and specific correction of pathogenic variants. Main advantages of gene editing is the long-lasting benefit achieved by a single intervention; main disadvantages are off-target effects, delivery difficulties, immunogenicity, and the ethical issues [15, 16].

The current reviews focus not only on the fact that CRISPR is moving from models into novel therapeutics but that there are significant translational challenges to overcome [15, 16]. Edition will likely be required to complement strong delivery systems and early intervention to overcome the downstream burden of fibrosis and cardiomyopathy in DMD.

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F. Cell-based and patient-derived model systems

Patient-derived systems such as induced pluripotent stem cell (iPSC) models are becoming more and more relevant for personalized drug testing and mechanistic studies. They enable modelling of dystrophin deficiency, splicing behaviour, and model response to candidate therapies at the level of the patient, and are powerful tools for precision preclinical work [16, 18]. In a future personalized workflow, muscle cells derived from iPSC could help determine if a mutation-specific drug is likely to be effective prior to administering it to the patient.

G. Biomarker-guided monitoring and imaging-based personalization

Most of the DMD therapies are partially, and later on, effective, therefore the use of biomarkers to assess the therapy is required [18]. Monitor response, optimize timing and detect early non-responders through serum and imaging markers. In particular, Quantitative MRI and radiomics have shown to be important tools since they can be used to measure fat infiltration and muscle composition before any apparent clinical degeneration happens [17, 18].

Table 3: Personalized therapeutic approaches in DMD

Strategy	Molecular target	Precision requirement	Main objective
Corticosteroids	Downstream inflammation	Stage- and toxicity-based	Slow progression
Exon skipping	Pre-mRNA splicing	Mutation-specific exon amenability	Restore reading frame
Nonsense readthrough	Premature stop codon	Nonsense mutation	Continue translation
Micro-dystrophin gene therapy	Gene replacement	Broad eligibility, mutation confirmation	Partial functional replacement
CRISPR/Cas editing	Pathogenic DNA variant	Variant-specific	Permanent correction
iPSC-based testing	Patient biology	Individualized model	Predict response
Biomarkers/MRI	Disease burden and response	Longitudinal monitoring	Optimize timing and efficacy

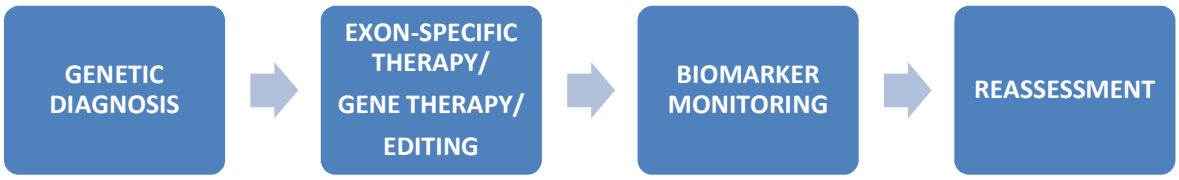

Figure 4: Precision-treatment ladder in DMD

VIII. FUTURE DIRECTIONS: AI, MACHINE LEARNING, AND BIOINFORMATICS

Complex genotype–phenotype relationships and multiple response dimensions are increasingly exploited by the use of AI and machine learning in DMD. Computational systems can be used to aid in variant interpretation, the prediction of progression and treatment stratification [17]. A 2025 radiomics study demonstrated that machine learning using MRI was more effective than clinical interpretation alone in identifying early DMD compared to Becker muscular dystrophy, further highlighting the clinical benefit of data-driven precision phenotyping [17].

Bioinformatics also aids in personalized treatment through annotation of variants, prediction of splicing effects, etc. and connection between genotype and therapeutic option. It is not a luxury in a disease where an exon can be a drug

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eligibility criterion, it is a "clinical enabler" – and that is what computational pipelines are. AI may also aid in predicting which children are at risk of losing ambulation early, who will benefit from exon skipping and who will have too much replacement fibrotic tissue to respond meaningfully [17, 18].

The integrated precision workflows of molecular diagnosis, imaging, biomarker profiling, computational prediction, and treatment matching will be the future of DMD management. That's the ultimate in personalized medicine for DMD.

IX. CHALLENGES AND TRANSLATIONAL LIMITATIONS

Although significant advances have been made, there are still several challenges to overcome. Mutation-specific therapies can only be used in subsets of patients, meaning that there is no specific drug for many patients [7]–[12]. Cost, delivery and safety concerns are obstacles to the success of gene therapy and genome editing [13]–[16]. Even the most effective molecular intervention may be less effective in case of advanced fibrosis, known cardiomyopathy, or respiratory deterioration [1], [22].

Access and cost are primary challenges in low and middle income countries like India. Molecular diagnosis may not be available or timely, and when it is known, the treatment may be inaccessible or unaffordable [20, 21]. This equates to a huge translation gap between science and practice.

Ethical aspects also come in to play, especially with regards to irreversible genome editing. The objective is not just to edit DNA, it is to edit DNA in a safe, sustainable and fair way. Personalized medicine in DMD will be successful only if it is not only biologically effective, but socially accessible as well.

X. CONCLUSION

Today, Duchenne muscular dystrophy (DMD) is one of the most promising examples of personalised medicine. The mode of action of the disease, the presence of mutations with different characteristics and the gradual stages of the disease all require personalized treatment. Traditional steroid and supportive therapy is still important, but the therapeutic options have broadened to include exon skipping, nonsense readthrough, gene replacement, genome editing, monitoring with biomarkers and computational stratification [1], [7]–[18].

The principle of mutation informed, stage aware and biomarker guided management of DMD is most critical. The field is shifting from a symptom-only approach to precision therapeutics to understand and target the molecular cause of the disease. While challenges to cost, access, safety and durability remain, DMD has emerged as a flagship disease that demonstrates personalized medicine and a model for the future of genomic therapeutics in rare disorders.

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