Expanding DMD Treatment Horizons: NEW EVIDENCE

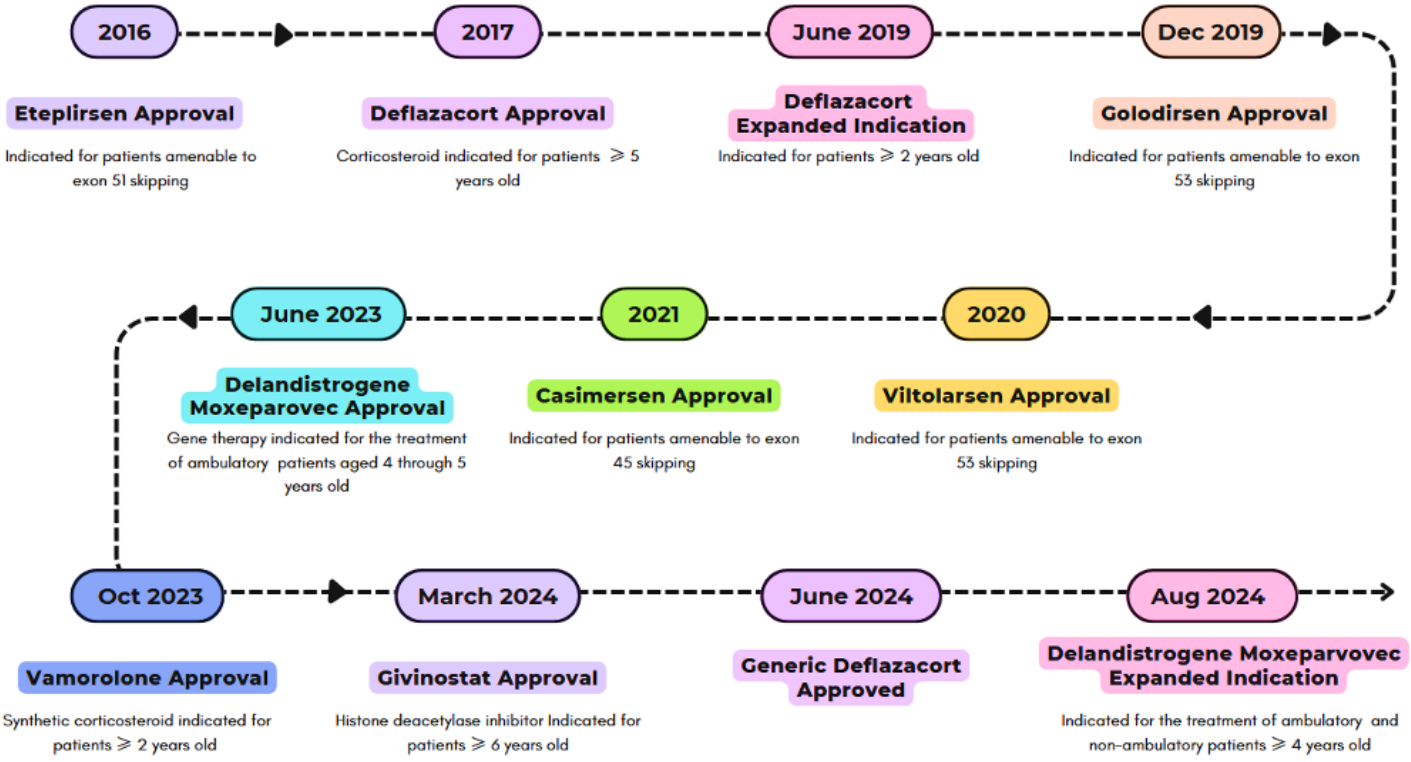
RAPID RECAP

Learning Objectives

After participating in this activity, learners should be better able to:

- Evaluate real-world, case-based scenarios for patients with DMD to help determine therapeutic candidacy across the age spectrum
- Assess the latest data from clinical studies on DMD treatments to help inform clinical decision-making
- Describe best practices for the ongoing monitoring of patients of various ages who are receiving treatment for DMD

Overview of DMD Therapies[1]



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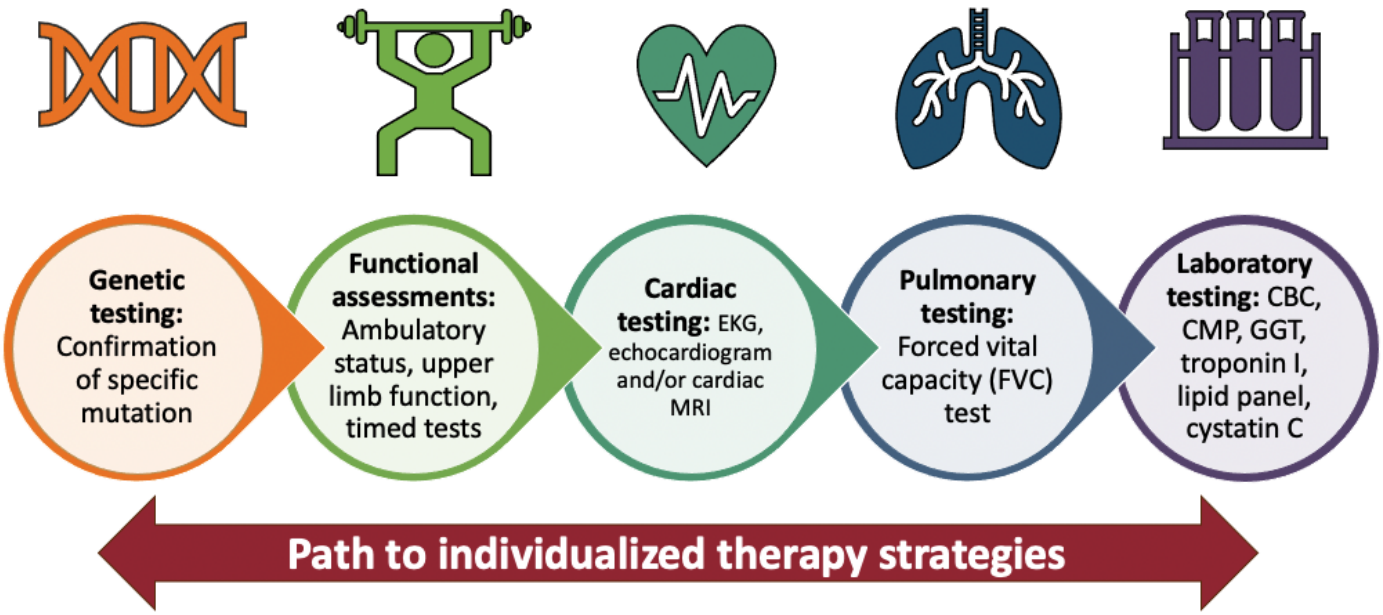
Baseline Patient Assessments for Determining Therapeutic Strategy[2]



CBC = complete blood count; CMP = comprehensive metabolic panel; EKG = electrocardiogram

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Comparison of FDA-Approved Therapies[3-5]

Therapeutic Approach	Pros	Cons
Gluco-corticoids	• Applicable to all patients with DMD, regardless of mutation • Prolonged time to loss of ambulation • Reduced requirement for scoliosis surgery • Improved cardiopulmonary function	• Weight gain • Changes in mood/behavior • Reduced bone health • Pubertal suppression • Adrenal insufficiency risk • Risk for cataracts • Frequent dosing (daily or intermittent)
Exon Skipping	• Prolonged time to loss of ambulation • Improved pulmonary function compared to natural history	• Requires frequent dosing intravenously • Only applicable to a subset of patients (mutation specific) • Requires monitoring of renal function • Low dystrophin protein production on biopsy
Gene Transfer Therapy	• Minimal genetic restrictions (exclusion of only deletions exons 8/9) • Significant microdystrophin protein production on biopsy • Improved functional outcomes • Single administration	• Risk for hepatotoxicity, myocarditis, immune mediated myositis, nausea/vomiting, thrombocytopenia, and complement activation • Subset of patients will be excluded from treatment due to antibody positivity for vector
HDAC Inhibitor Therapy	• Delays physical decline by approximately two years compared to standard care • Enhances muscle fiber regeneration, reduces inflammation, and decreases fibrosis • Adverse events were generally manageable	• Long-term efficacy across diverse patient populations remains unclear, particularly in older or non-ambulant patients • Requires laboratory monitoring • Might need to be used alongside other treatments to achieve optimal outcomes

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Key Takeaways

- Dystrophin-restoring therapies can serve as a foundational treatment for patients with DMD to be built upon
- Combining different therapies for DMD holds the potential to improve patient outcomes and reduce the overall disease burden by targeting multiple pathways associated with the disease spectrum

References

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3. Heydemann A, Siemionow M. A brief review of duchenne muscular dystrophy treatment options, with an emphasis on two novel strategies. *Biomedicines*. 2023;11(3):830.
4. Duvyzat (givinostat) [package insert]. Italfarmaco. March 2024.
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