New Pathways and Possibilities in the Treatment of DMD

RAPID RECAP

Non-Gene Based Therapies for DMD[1]

Agent	Class	Indicated/Investigated Population	Target	Status
Givinostat	HDAC inhibitor	Patients ≥ 6 years old	Muscle growth and protection	Approved
Deflazacort	Corticosteroid	Patients ≥ 2 years old	Reduce inflammation	Approved
Vamorolone	Corticosteroid	Patients ≥ 2 years old	Reduce inflammation	Approved
CAP-1002	Cell therapy	Patients ≥ 10 years old	Muscle growth and protection	Phase 3
EDG-5506	Fast myosin ATPase inhibitor	Patients 6-17 years old	Muscle growth and protection/ heart function	Phase 2
Ifetroban	Thromboxane inhibitor	Patients ≥ 17 years old	Heart function	Phase 2
Satrilizumab	IL-6 mAb	Patients ≥ 8 to < 18 years old	Bone health	Phase 2
ATL1102	ASO for CD49d	Patients ≥ 10 to < 18 years old	Reduce inflammation	Phase 2
Rimeporide	NHE-1 inhibitor	Patients 6-14 years old	Regulate calcium balance	Phase 1/2
Canakinumab	IL1b mAb	Patients ≥ 2 years old	Reduce inflammation	Phase 1/2
SAT 3247	AAK inhibitor	Healthy volunteers and patients aged ≥ 18 to ≤ 40 years old	Protect muscle, promote regeneration	Phase 1

AAK = aurora A kinase; ASO = antisense oligonucleotide; CD = cluster of differentiation; HDAC = histone deacetylase; IL = interleukin; mAb = monoclonal antibody; NHE = sodium-hydrogen antiporter

Provided by The France Foundation

New Pathways and Possibilities in the Treatment of DMD

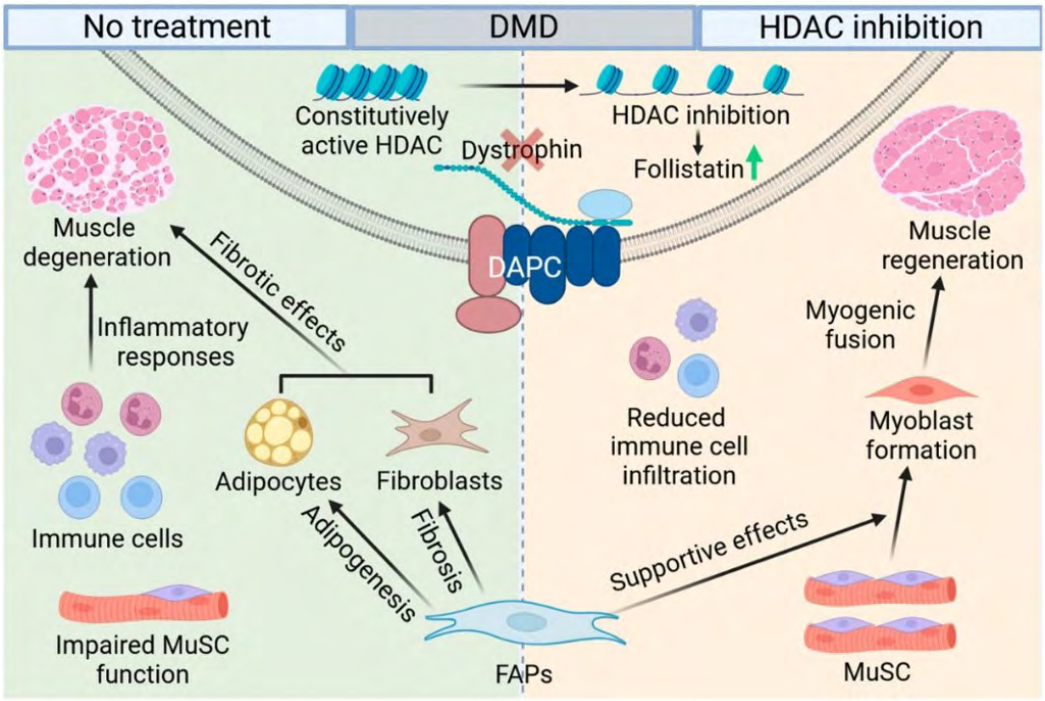
RAPID RECAP

Pathological Events Associated With HDAC Upregulation[2,3]:

- Activation of chronic inflammatory pathways
- Muscular atrophy and impairment of muscle repair mechanisms
- Fibrogenesis and adipogenesis

Impact of HDAC Inhibition[2-4]:

- Decreased inflammation
- Increased muscle fiber repair and regeneration
- Reduced fibrogenesis and adipogenesis



DAPC = dystrophin-associated protein complex; FAP = fibroadipogenic progenitors; MuSC = muscle stem cell

Provided by The France Foundation

New Pathways and Possibilities in the Treatment of DMD

RAPID RECAP

Considerations for Initiating Givinostat[4-7]:

- Givinostat is the first and currently only HDAC inhibitor approved for DMD
- Givinostat can be initiated in patients regardless of their eligibility for gene therapy or exon-skipping drugs
- Givinostat was tested in patients on corticosteroids, and patients should remain on stable corticosteroids throughout treatment
  - Coadministration does not require specific interval spacing, but periodic monitoring for overlapping adverse events is necessary
- Givinostat has the potential to complement gene therapy and exon-skipping therapy

Key Takeaways

- Corticosteroids are still the foundational therapy for DMD
- Multidisciplinary care is still needed to manage comorbidities associated with DMD
- As treatment options for DMD expand, combination approaches leveraging complementary mechanisms of action may offer broader disease control and improved outcomes

References

1. Parent Project Muscular Dystrophy. Accessed November 20, 2025. https://www.parentprojectmd.org/duchenne-drug-development-pipeline/
2. Aartsma-Rus A. *Front Cell Dev Biol*. 2025;12:1514898.
3. Consalvi S, et al. *Mol Med*. 2011;17(5-6):457-465.
4. Mercuri E, et al. *Lancet Neurol*. 2024;23(4):393-403.
5. DUVYZAT (givinostat) [prescribing information]. ITF Therapeutics; 2024
6. Anjum AF, et al. *Curr Ther Res Clin Exp*. 2025;102:100787.
7. Bizot F, et al. *Mol Ther Nucleic Acids*. 2022;30:606-620.

Provided by The France Foundation