

Optimizing the Role of CORTICOSTEROIDS

Within the Evolving

DMD TREATMENT LANDSCAPE



RAPID RECAP

Selecting a Corticosteroid

Drug	Pros	Cons
Prednisone/ Prednisolone ^{1,2}	• Standard of care • Slows progression of muscle weakness • Prolongs ambulation by 2–3 years • Improves pulmonary function • Best outcomes when started early (ages 2–4)	• Excessive weight gain, cushingoid appearance • Behavioral changes • Growth stunting, delayed puberty • Osteoporosis, increased fractures • Adrenal insufficiency
Deflazacort ¹⁻³	• Similar or greater efficacy than prednisone • Delays loss of ambulation • Lower risk of weight gain vs prednisone • Reduced scoliosis risk	• Cataracts • Behavioral issues • Mixed evidence on growth/bone health: some studies report more delay/fractures, others fewer effects
Vamorolone ^{2,4}	• Muscle-function improvements comparable to classic corticosteroids • Growth trajectories preserved; minimal growth stunting compared to prednisone/deflazacort • Mineralocorticoid receptor antagonism may help cardioprotection	• Cannot be used for stress dosing • Long-term and real-world data are still being collected

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Dosing Strategies¹⁻⁴

- Steroids should be started before the child reaches the “plateau phase” and loses any strength – this is generally around age 4–5 years
- Studies confirm the benefits of starting glucocorticoids in younger children, before significant physical decline
- The dose may change throughout a patient’s lifespan depending on their emerging needs
 - Steroids should NEVER be stopped abruptly
 - Dose adjustments may be needed for significant side effects

Prednisone
0.75 mg/kg/day

Deflazacort
0.9 mg/kg/day

Vamorolone
6 mg/kg/day

- Alternate regimens
 - Weekend dosing
 - Different doses for patients with hepatic impairment
- Maximum dose:
 - Vamorolone has a maximum daily dosage of 300 mg for patients weighing more than 50 kg
 - Generally, doses of twice-weekly steroids can go up to 250 mg/day of prednisone or 300 mg/day of deflazacort

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Combination Therapy:

Corticosteroids for DMD are used in combination with:⁵⁻⁷

Gene transfer therapy

Exon-skipping therapy

Givinostat

Key Takeaways

Initiation & Timing: Start corticosteroids at ~4-5 years old, before plateau; discuss at diagnosis.^{1,2}

Monitoring & Adjustment: Track growth, puberty, bone health, and behavior; adjust dose for tolerability without abrupt discontinuation.^{1,3}

Gene Therapy Readiness: Use prednisone/prednisolone peri infusion (1 mg/kg/day, max 60 mg; escalate to 2 mg/kg/day if liver toxicity).⁶

Combination Therapy Coordination: Continue stable steroids during exon skipping or givinostat; ensure dosing is stable before adding new agents.⁷

Patient & Family Counseling: Advise on side effects, stress dosing, and steroid role alongside new therapies.^{1,2}

References:

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3. EMFLAZA (Deflazacort) [package insert]. South Plainfield, NJ: PTC Therapeutics; 2021
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