MADISON NEUROLOGY LLC

608 403 0000

Neurology Care 7 days a Week. Because Your Brain Matters.

Elevidys®

Delandistrogene moxeparvovec-rokl

COMPLETE CLINICAL PROTOCOL: PRE-TREATMENT CHECKLIST • ORDER SET • FOLLOW-UP • HOLD/DISCONTINUE CRITERIA

GENERIC NAME	delandistrogene moxeparvovec-rokl
BRAND	ELEVIDYS®
MEDICATION CLASS	AAVrh74-based gene therapy — one-time micro-dystrophin gene transfer
PRESENTATION	Suspension for IV infusion supplied as a weight-specific kit of single-use vials
ROUTE	ONE-TIME IV infusion (approximately 1–2 hours)
US INDICATION (FDA)	Duchenne muscular dystrophy in patients at least 4 years of age with a confirmed DMD gene mutation — verify the CURRENT label for the exact age/ambulatory populations, which have been revised since first approval.

Class context.
Most practices deliver gene therapy in partnership with a designated treatment center — product handling, timing, and safety reporting are program-defined. Key labeled safety themes: acute serious liver injury (including failure), immune-mediated myositis (elevated risk with certain exon deletions), myocarditis (troponin surveillance), thrombocytopenia, and infusion-related reactions.

Section 1 — Pre-Treatment Checklist

A. Required screening labs

- ☐ Confirmed DMD gene mutation AND review against label exclusions: certain deletions (involving exons 8 and/or 9) carry a markedly increased risk of severe immune-mediated myositis — specialist review required; verify the current label.
- ☐ AAVrh74 total-binding ANTIBODY TITER — pre-existing immunity above the labeled threshold precludes dosing.
- ☐ Liver function: ALT, AST, GGT, total bilirubin — baseline; acute serious liver injury has occurred.
- ☐ Troponin-I — baseline (myocarditis signal).

☐ Platelet count — baseline (thrombocytopenia, including immune-mediated, has occurred).

☐ Active-infection review — defer dosing until fully resolved.

B. Vaccinations

☐ Review immunization status BEFORE the corticosteroid backbone begins; live vaccines follow corticosteroid-dose rules per immunization guidance.

C. Clinical assessments & counseling

☐ Specialist gene-therapy center coordination confirmed (dosing, preparation, pharmacovigilance reporting).

☐ Family education on the monitoring calendar — weekly labs for the first months are part of the therapy, not optional follow-up.

☐ Educate on immune-response symptoms: fever, vomiting, abdominal pain, jaundice, chest symptoms, edema, unusual bruising.

D. Exclusion / hold criteria — do not start

CRITERION	RATIONALE / REQUIRED ACTION
AAVrh74 antibody titer above the labeled threshold	Do not dose — neutralization risk.
Label-specified DMD exon deletions (e.g., involving exons 8 and/or 9)	Marked immune-mediated myositis risk — verify current label; specialist decision.
Active infection or significant baseline liver abnormality	Postpone and evaluate.

Section 2 — Order Set

Premedication

☐ CORTICOSTEROID BACKBONE (e.g., prednisone 1 mg/kg/day or equivalent) STARTING 1 DAY BEFORE the infusion and continued for weeks afterward with a response-guided taper — this is integral to the protocol, not an optional premedication.

Dosing schedule

One-time dose	1.33 × 10^14 vector genomes/kg IV as a single infusion (verify the current label's exact specification), over approximately 1–2 hours.
Observation	Monitor during the infusion and for several hours after; infusion-related reactions are managed by interruption/supportive care per severity.

Standing orders — every infusion

☐ Continue the corticosteroid backbone WITHOUT interruption; taper only per the treating specialist's response-guided plan.

☐ Pre-book the weekly lab draws (liver, troponin, platelets) BEFORE the infusion date is set.

Missed or delayed dose

Not applicable — one-time therapy. If the infusion is postponed, restart the pre-infusion steroid timing.

Section 3 — Follow-Up Protocol

ITEM	FREQUENCY / TIMING	ACTION / THRESHOLD
Liver function tests	Weekly for the first 3 months (or until clinically unremarkable), then per specialist	Rising transaminases → steroid adjustment/specialist management per the gene-therapy program.
Troponin-I	Weekly for the first month, then as indicated	Elevation → cardiology evaluation for myocarditis.
Platelet count	Frequently in the first weeks	Falling platelets → evaluate for immune-mediated thrombocytopenia.
Corticosteroid response/taper review	Specialist-guided	Do not taper on a fixed clock.
Motor function outcomes	Long-term per neuromuscular clinic	Program and registry reporting.

Section 4 — Pause vs. Stop Criteria

A. Pause or delay (resume when resolved)

CRITERION	REQUIRED ACTION
Active infection at the planned dosing date	Postpone until resolved.
Abnormal baseline labs (liver, troponin, platelets)	Postpone and evaluate.

B. Discontinue permanently

CRITERION	REQUIRED ACTION
Post-infusion immune-mediated liver injury or myocarditis	Specialist-directed immunosuppression escalation; report to the manufacturer program.

Section 5 — Notes & Practical Points

- ☐ The infusion is one day; the protocol is three months. The steroid backbone and weekly labs ARE the treatment.
- ☐ Verify the current label for eligible age and ambulatory status — this indication has evolved since first approval.

Prepared November 2026 by Madison Neurology LLC.

This document is an educational compilation and does not reproduce the complete prescribing information. Verify every element against the current FDA label and applicable REMS requirements before clinical use.

- Current U.S. prescribing information via NIH DailyMed — https://dailymed.nlm.nih.gov/dailymed/
- FDA Drugs@FDA database — https://www.accessdata.fda.gov/scripts/cder/daf/
- FDA drug safety and availability updates — https://www.fda.gov/drugs/drug-safety-and-availability

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Questions about infusion referrals: call 608 403 0000. Neurology Care 7 days a Week. Because Your Brain Matters.