MADISON NEUROLOGY LLC

608 403 0000

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

# Vyondys 53®

## Golodirsen

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

| | |
|---|---|
| **GENERIC NAME** | golodirsen |
| **BRAND** | VYONDYS 53® |
| **MEDICATION CLASS** | Antisense oligonucleotide (phosphorodiamidate morpholino oligomer, exon 53 skipping) |
| **PRESENTATION** | 100 mg/2 mL (50 mg/mL) single-dose vial — dilute in 0.9% NaCl |
| **ROUTE** | IV infusion over 35–60 minutes, once weekly |
| **US INDICATION (FDA)** | Duchenne muscular dystrophy in patients with a CONFIRMED mutation amenable to exon 53 skipping (accelerated approval based on dystrophin increase; continued approval may depend on confirmatory trials). |

**Class context.**
Class point for exon-skipping PMOs: renal toxicity signals in nonclinical/clinical data drive the monitoring schedule, and serum creatinine UNDERESTIMATES renal impairment in DMD (low muscle mass) — cystatin C is the preferred marker.

## Section 1 — Pre-Treatment Checklist

### A. Required screening labs

☐ Genetic confirmation of a DMD mutation amenable to EXON 53 skipping — REQUIRED; this defines the labeled population (verify the report names exon 53 amenability explicitly).

☐ Renal baseline: serum creatinine, CYSTATIN C (preferred GFR marker in DMD), BUN, and urine dipstick.

☐ Document the current corticosteroid regimen and baseline motor function (North Star / timed tests as age-appropriate).

### B. Vaccinations

☐ No label restrictions; keep routine pediatric vaccinations current per primary care.

### C. Clinical assessments & counseling

- ☐ Baseline motor assessment for response documentation.
- ☐ Plan reliable WEEKLY access — adherence is the main real-world failure mode; confirm transport and scheduling before the first dose.
- ☐ Hypersensitivity counseling (rash, fever, respiratory symptoms).

### D. Exclusion / hold criteria — do not start

| CRITERION | RATIONALE / REQUIRED ACTION |
|---|---|
| Mutation not amenable to exon 53 skipping | Not the labeled population — do not dose. |
| Serious hypersensitivity to golodirsen | Do not administer. |

## Section 2 — Order Set

### Premedication

- ☐ None required; premedicate after a prior hypersensitivity event per institutional practice.

### Dosing schedule

| | |
|---|---|
| **Standard dose** | 30 mg/kg IV once weekly, infused over 35–60 minutes. |

### Standing orders — every infusion

- ☐ Monitor for hypersensitivity during and after each infusion.
- ☐ Track weight at intervals — dosing is weight-based; growing children change dose.

### Missed or delayed dose

Give a missed weekly dose as soon as possible and resume the weekly schedule.

## Section 3 — Follow-Up Protocol

| ITEM | FREQUENCY / TIMING | ACTION / THRESHOLD |
|---|---|---|
| Renal function — cystatin C, creatinine, BUN, urine dipstick | Baseline, then periodically per label during treatment | Any deterioration → hold and evaluate before continuing. |
| Motor function testing | Every 6–12 months | Response documentation for continuation. |
| Hypersensitivity review | Every infusion | Anaphylaxis → discontinue. |

## Section 4 — Pause vs. Stop Criteria

### A. Pause or delay (resume when resolved)

| CRITERION | REQUIRED ACTION |
|---|---|
| Renal signal (rising cystatin C/creatinine, new proteinuria) | Hold and evaluate before continuing. |

### B. Discontinue permanently

| CRITERION | REQUIRED ACTION |
|---|---|
| Anaphylaxis or serious hypersensitivity | Discontinue. |
| Progressive renal toxicity attributed to therapy | Discontinue; specialist review. |

## Section 5 — Notes & Practical Points

- ☐ Accelerated approval: families should understand approval rested on a dystrophin surrogate endpoint, with confirmatory benefit trials ongoing.
- ☐ Never substitute across exon-skipping products — each matches a specific mutation class.

**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.