



Zolgensma in Egypt

The EDA named-patient coordination pathway — indicative 2026

RARE DISEASE · AAV9 GENE THERAPY FOR SMA

The clinical situation

Zolgensma (onasemnogene abeparvovec-xioi) is a one-time AAV9-based gene therapy for paediatric spinal muscular atrophy (biallelic SMN1 mutations) in patients under two years of age. Manufactured by Novartis. Administered as a single weight-dosed intravenous infusion. Prednisolone is initiated one day before infusion and tapered over approximately two months. Transaminase, platelet, and troponin monitoring are required weekly for the first month then on schedule.

The pathway, 5 steps

- 1 Consultation & SMN1 genotyping.** Your paediatric neurology team confirms biallelic SMN1 mutation, weight, age, and anti-AAV9 antibody status (titre <1:50 required).
- 2 Baseline assessment.** Transaminases, platelets, troponin-I, CHOP-INTEND or HINE-2 motor milestone scoring, and infection screen.
- 3 EDA named-patient application.** Clinical rationale, genotype report, weight, and infusion-centre attestation filed under personal-use import.
- 4 US-side sourcing & cold-chain logistics.** Reserve Meds coordinates the dose through our US-licensed DSCSA-compliant specialty wholesale partner; deep-frozen cold-chain to the infusion centre.
- 5 Infusion & post-dose monitoring.** Paediatric team infuses at a qualified centre; weekly labs month 1, then tapered per FDA labelling.

Indicative economics

Reference US cash-pay range: USD 2,100,000–2,250,000 as a one-time product cost (drug-only reference; Reserve Meds provides a transparent delivered quote at intake).

Indicative first-dose timing

6–10 weeks from a complete EDA application to infusion, once cohort intake opens. Indicative — not guaranteed.

Reserve Meds's role

Sourcing. US-licensed specialty wholesale partner, DSCSA chain-of-custody. **Documentation.** Regulatory package for your paediatric neurology team and EDA review, keyed to SMN1 genotype and age/weight. **Logistics.** Deep-frozen cold-chain shipment with importer-of-record handling to the infusion centre. **Concierge case lead.** Named point of contact for the family and care team. **We are a coordinator** — not the prescriber, not the infusion centre. All clinical decisions remain with your treating paediatric neurologist.

COMPOSITE EXAMPLE · PRE-LAUNCH WAITLIST

Join the Zolgensma × Egypt first-cohort waitlist. *Our concierge reaches out as we open intake.*

reservemed.com/access-guides/zolgensma-egypt.html



SCAN TO JOIN

Reserve Meds · US-based concierge for cross-border specialty medicine. We are a coordinator; we are not the prescriber and not the dispensing pharmacy. All clinical decisions remain with the treating physician. Not medical advice.

Reserve Meds is in pre-launch. Service availability is limited to our first cohort; all timelines published are indicative, not guarantees. Composite case examples only.

reservemed.com · hello@reservemed.com · WhatsApp concierge: +1 516 528 3120 · Reviewed 2026-04-23 by Reserve Meds clinical & regulatory team