Zolgensma

United Kingdom · access guide

Zolgensma access in the United Kingdom: the Specials Licence pathway

Last reviewed 2026-05-16 by Reserve Meds clinical and regulatory team.

Quick orientation

Zolgensma (onasemnogene abeparvovec) is a one-time intravenous gene therapy from Novartis Gene Therapies for spinal muscular atrophy (SMA). The medicine carries a UK marketing authorisation granted on the basis of EMA approval and a UK reliance route, and NICE issued a positive technology appraisal in March 2021 (TA755) recommending Zolgensma for SMA type 1 within an NHS England-Novartis managed access agreement. Eligibility under the NICE recommendation centres on pre-symptomatic infants identified through screening, SMA type 1 infants under 12 months of age, and case-by-case clinical review for older infants and children. Treatment is delivered at a small number of UK paediatric neurology centres equipped for AAV9 gene therapy administration.

Two scenarios drive UK families to pursue Zolgensma through alternative routes: a child sitting outside the strict NICE-recommended criteria (older than 12 months, weight above the typical dosing band, or with disease progression already established), and families pursuing additional clinical support during the manufacture and treatment window. Reserve Meds coordinates US-side sourcing and the operational chain for these complex paediatric cases.

Why UK families pursue Zolgensma outside the standard route

The UK has approximately 60 to 70 new SMA cases per year, weighted heavily toward SMA type 1 (the most severe, with onset typically before 6 months and historically fatal before 2 years without disease-modifying therapy). The standard of care has shifted dramatically with the arrival of three disease-modifying therapies: Zolgensma (one-time AAV9 gene therapy), Spinraza (nusinersen, intrathecal antisense oligonucleotide), and Evrysdi (risdiplam, oral small molecule). NICE has recommended each for specific patient populations.

The friction points for Zolgensma specifically are the age and weight ceiling, the patient screening for AAV9 neutralising antibodies (a contraindication), and the centre slot scheduling. The UK SMA newborn screening pilot is expanding, but a child diagnosed after symptom onset may be racing the disease progression. Families look for parallel routes when the NHS scheduling timeline does not match the disease timeline.

The Specials Licence pathway for Zolgensma in the UK

Zolgensma holds a UK marketing authorisation, so the standard NHS route through NICE TA755 and the Novartis managed access agreement is the principal pathway. The Specials route applies when the patient sits outside the NICE-recommended criteria but the treating paediatric neurology consultant clinically supports the case.

The supply chain is tightly controlled by Novartis Gene Therapies. The medicine is dose-banded by weight, manufactured at the Novartis facility, quality-released, and shipped frozen on dry ice to the treating centre. The Specials notification supports the chain-of-custody record for the import shipment.

Where Zolgensma is delivered in the UK

The NHS specialist paediatric neuromuscular centres delivering Zolgensma include Great Ormond Street Hospital London (the highest-volume UK paediatric SMA centre), Evelina London Children's Hospital, Birmingham Children's Hospital, Manchester Children's Hospital (Royal Manchester Children's Hospital), Sheffield Children's Hospital, Royal Hospital for Children Glasgow, and Bristol Royal Hospital for Children. Zolgensma infusion takes place in an inpatient setting because of the steroid pre-medication regimen, the AAV9 infusion procedure, the post-infusion liver function monitoring requirement, and the systemic inflammatory response monitoring window.

Steroid pre-medication (typically oral prednisolone starting 24 hours before infusion and continuing for at least 8 weeks with taper) is part of the Zolgensma protocol because of the documented risk of hepatotoxicity. Weekly liver function monitoring during the first month and then twice-monthly for at least 3 months is standard.

Real cost picture for Zolgensma in the UK

Zolgensma's US list price is approximately USD 2.125 million for the one-time infusion, the highest single-dose medicine list price in the world. At 0.79 GBP to 1 USD the product converts to roughly GBP 1.68 million. NHS contracted pricing under the NICE TA755 managed access agreement reflects a confidential commercial discount. The published research suggests the agreed UK net price is materially below US list, though the exact figure is commercially confidential.

For a private case the all-in cost extends to the inpatient infusion admission, the AAV9 neutralising antibody screen, the cardiac and liver baseline workup, the prednisolone tapering regimen, and the multi-month follow-up. Private UK Zolgensma cases typically land in the GBP 1.8 million to GBP 2.0 million range. UK private medical insurance does not typically fund Zolgensma; the principal funder is either NHS commissioning, family fundraising, or specific manufacturer compassionate-use access.

Typical timeline for Zolgensma in the UK

Zolgensma's timing pressure is the disease itself. Once SMA type 1 is diagnosed, motor function loss is rapid; the earlier the treatment, the better the outcome. The realistic UK timeline for an NHS-routed case from referral to infusion is approximately 8 to 16 weeks, with the AAV9 antibody screen, the centre slot, and the steroid pre-medication run-in as the critical-path items. The Specials route does not materially shorten this because the rate-limiting step is the clinical preparation, not the paperwork.

The Novartis manufacture cycle for Zolgensma is not patient-specific (the medicine is an off-the-shelf AAV9 vector dosed by weight band), so manufacture lead time is shorter than for autologous cell therapies. End-to-end timelines of 4 to 8 weeks from final eligibility confirmation to infusion are achievable in expedited scenarios.

What your UK paediatric neurologist needs to provide

The treating UK consultant paediatric neurologist (GMC-registered, working within a specialist paediatric neuromuscular centre) is the prescribing physician of record. The clinical packet typically includes the SMA genetic confirmation (SMN1 gene homozygous deletion or point mutation with SMN2 copy number), the age and weight (weight is the dosing variable; dose bands are defined up to approximately 21 kg with newer data on higher weights), the AAV9 neutralising antibody screen (a positive screen above titre threshold is a contraindication and routes to alternative SMA disease-modifying therapy), baseline cardiac function (echocardiogram and ECG), liver function panel, platelet count, and the documented disease severity and motor function assessment.

Steroid pre-medication and the post-infusion monitoring plan with liver function tests, troponin, platelets, and clinical follow-up at the specialist centre complete the standard of care.

Common questions about Zolgensma in the UK

Will the NHS fund Zolgensma for my child? If your child meets the NICE TA755 criteria (typically SMA type 1, under 12 months, AAV9 antibody-negative, and meeting the clinical eligibility framework), NHS England funds through the managed access agreement. The NHS Specialised Commissioning route is the standard.

What if my child is older than 12 months? NICE eligibility is restricted to specific age and clinical criteria. Older children may be considered case by case through Individual Funding Request to NHS England, although the bar is high. Private supply via the Specials framework or manufacturer compassionate-use access are the parallel routes.

What about the SMA newborn screening programme? The UK National Screening Committee is reviewing SMA newborn screening, and a regional pilot in Thames Valley and parts of Oxford ran. Newborn screening enables pre-symptomatic identification, which gives the best clinical outcomes with Zolgensma. The expansion of routine newborn SMA screening is the strategic direction of travel.

What if my child has AAV9 antibodies? A positive AAV9 neutralising antibody titre above the threshold is a contraindication to Zolgensma. The alternative SMA disease-modifying therapies (Spinraza, Evrysdi) are the appropriate routes for these patients.

Can my child have both Zolgensma and Spinraza or Evrysdi? Combination therapy is a topic of active clinical research and not part of the standard NICE recommendation. Some specialist centres consider sequenced or combination therapy on a case-by-case basis under research protocols.

What about families fundraising for international treatment? Several UK families have raised funds for Zolgensma access in earlier years when the medicine was not yet NICE-recommended. The 2021 NICE recommendation and managed access agreement materially reduced the need for international fundraising for in-scope patients. Out-of-scope patients (older children, complex cases) may still consider international centres including US and European options.

Where Reserve Meds fits in Zolgensma cases

Reserve Meds is a US-based concierge coordinator. For Zolgensma the NHS commissioning route through NICE TA755 is the principal access pathway. Our role is most relevant for out-of-scope cases pursuing parallel routes, families exploring US authorised treatment centres for complex circumstances, and families wanting independent operational support across the multi-week pre-treatment, infusion, and follow-up window. We do not replace the paediatric neurology consultant or the UK specialist centre.

The Yellow Card pharmacovigilance pathway for Zolgensma

The MHRA Yellow Card scheme is the UK's national pharmacovigilance reporting system. Healthcare professionals, patients, and carers can report suspected adverse drug reactions, medical device incidents, defective medicines, and counterfeit medicines through the scheme. For specialty medicines accessed through the Specials Licence pathway, Yellow Card reporting is the operational mechanism that connects the UK clinical experience back to the global pharmacovigilance dataset that the MHRA, FDA, EMA, and other regulators rely on.

For Zolgensma specifically, Reserve Meds coordinates the pharmacovigilance reporting chain in three ways. First, the prescribing UK consultant or the dispensing pharmacy submits any suspected adverse reactions through the Yellow Card scheme as standard practice. Second, the manufacturer's UK pharmacovigilance contact receives the case report through the standard regulatory channel and connects the case to the global safety database. Third, where the patient's clinical follow-up continues across markets, Reserve Meds provides the documentation continuity that lets the patient's consultants and the manufacturer's safety team coordinate across borders.

The MHRA also operates the Black Triangle (inverted black triangle) safety monitoring scheme for medicines that are under additional monitoring (typically newer medicines or medicines for which additional safety data are being collected). The Yellow Card scheme works the same way for Black Triangle medicines but with heightened attention to reporting.

UK consumer protection and patient rights for Zolgensma

UK patients accessing specialty medicines through private pharmacy supply have the same consumer protections that apply to any UK regulated medicine purchase. The Care Quality Commission regulates private healthcare providers in England; Healthcare Improvement Scotland, Healthcare Inspectorate Wales, and the Regulation and Quality Improvement Authority in Northern Ireland are the parallel regulators in the devolved nations. The General Pharmaceutical Council regulates pharmacy professionals and registered pharmacy premises. The General Medical Council regulates doctors. The Nursing and Midwifery Council regulates nurses, including specialist nurses involved in cell therapy and homecare administration.

For UK patients accessing Zolgensma, the relevant protections include the prescribing consultant's professional duty under GMC Good Medical Practice, the dispensing pharmacist's professional standards under General Pharmaceutical Council standards, the homecare provider's regulatory framework (where applicable), and the manufacturer's UK pharmacovigilance obligations. Reserve Meds operates as a US-based coordinator and is subject to US regulatory frameworks for our US-side operations; we do not replace or substitute for UK consumer protections, which the UK clinical and pharmacy chain provides directly.

Special considerations for international UK residents and dual-citizen families

The UK is home to a substantial population of international residents, dual-citizen families, and patients who spend significant time across multiple markets. For Zolgensma cross-border continuity of care across the UK, the United States, the Gulf, India, and other markets is a recurring operational pattern. Reserve Meds is structured to support this cross-market reality with a single coordinator who understands the regulatory frameworks across the relevant jurisdictions, the documentation portability across markets, and the operational connection back to the UK clinical team during periods of UK residence.

UK patients who spend time in the United States may also pursue treatment at a US authorised treatment centre when this is clinically or operationally preferable. Reserve Meds provides the US-side liaison, the documentation packet for the US treatment centre, and the operational support across the UK-US clinical handover both at the start and on return to the UK for long-term follow-up.

Where to read more about Zolgensma and the UK Specials pathway

Reserve Meds publishes detailed reference material across the regulatory pathways, country specifics, and condition-specific access guides. For the regulatory framework underlying the UK route to Zolgensma, the named-patient pathway overview covers the international framework and the United Kingdom country deep-dive covers the MHRA Specials Licence, NICE technology appraisal, NHS England Specialised Commissioning, and the dispensing infrastructure in detail. The MHRA's own guidance on the supply of unlicensed medicinal products (often called the MHRA Guidance Note 14) provides the formal regulatory framing for prescribers and pharmacists. The General Pharmaceutical Council's standards on the dispensing of unlicensed medicines provide the pharmacy practice framework.

For UK patient information on the NHS-funded pathway, the National Institute for Health and Care Excellence (NICE) publishes the relevant technology appraisal guidance, and NHS England Specialised Commissioning publishes the corresponding clinical commissioning policy. Patients can search the NICE website for the specific technology appraisal that applies to their medicine and indication.

Reserve Meds's role

US-based concierge coordinator for cross-border specialty medicine. We are not the prescriber, not the dispensing pharmacy, and not the manufacturer. All clinical decisions remain with your treating physician.

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