Elevidys

United Kingdom · access guide

Elevidys access in the United Kingdom: the Specials Licence pathway

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

Quick orientation

Elevidys (delandistrogene moxeparvovec, formerly SRP-9001) is Sarepta Therapeutics' one-time intravenous AAVrh74 gene therapy for Duchenne muscular dystrophy (DMD). The US FDA initially granted accelerated approval in June 2023 for ambulatory boys aged 4 to 5 years and converted to full approval in June 2024 with expansion to ambulatory and non-ambulatory DMD patients aged 4 years and older. The medicine delivers a micro-dystrophin transgene via AAVrh74 capsid to muscle tissue, with the aim of producing a functional shortened dystrophin protein.

As of mid-2026 Elevidys does not yet hold a UK marketing authorisation. The EMA's pathway through European centralised assessment is in progress with Sarepta's European partner Roche, and the MHRA reliance pathway may follow. NICE technology appraisal is the eventual NHS funding determinant. In the absence of UK marketing authorisation, UK families with DMD pursuing Elevidys depend on the Specials Licence pathway or, more commonly given the operational complexity, travel to a US authorised treatment centre.

Why UK DMD families pursue Elevidys

Duchenne muscular dystrophy is a fatal X-linked recessive disorder affecting approximately 1 in 5,000 male births. The UK has approximately 2,500 boys living with DMD. The current UK standard of care includes corticosteroids (prednisolone or deflazacort), ataluren (Translarna) for nonsense-mutation DMD subset patients, and supportive cardiac and respiratory care. Elevidys offers a one-time gene-replacement strategy that, in clinical trials, has shown functional improvement on motor function endpoints in younger ambulatory patients, with the expansion to older and non-ambulatory patients reflecting the FDA's June 2024 full approval.

UK families pursue Elevidys when their child meets the age criteria (typically 4 years and older), the genetic eligibility (DMD gene mutation profile that does not have anti-AAVrh74 antibodies), and the family has the funding and travel capacity to pursue treatment at a US authorised treatment centre or, occasionally, through a UK Specials Licence route.

The Specials Licence pathway for Elevidys in the UK

Without a UK marketing authorisation, Elevidys access for UK patients requires the Specials Licence pathway under the Human Medicines Regulations 2012 or treatment at a US authorised treatment centre. The Specials route requires a UK-licensed consultant paediatric neurologist's clinical decision and a UK Specials Licence holder with import authorisation. In practice the operational complexity of importing AAVrh74 gene therapy, the cardiac and liver function monitoring protocol, and the corticosteroid pre-medication regimen typically routes UK families to US authorised treatment centres rather than to UK delivery for Elevidys at this stage.

The US authorised treatment centres for Elevidys include a growing network of paediatric neuromuscular centres across the United States, with leading sites at Nationwide Children's Hospital Columbus (where the Sarepta clinical programme was developed), Children's Hospital of Philadelphia, Boston Children's Hospital, Cincinnati Children's Hospital Medical Center, Children's Hospital Los Angeles, Texas Children's Hospital, Children's National Washington DC, the Lucile Packard Children's Hospital Stanford, and a wider network of designated centres.

Where Elevidys is delivered

Elevidys delivery requires a paediatric neuromuscular centre experienced with AAV gene therapy infusion, the corticosteroid pre-medication and tapering regimen, the cardiac and liver function monitoring protocol, and the post-infusion intensive care window. For UK patients pursuing Elevidys the operational realities favour treatment at a US authorised treatment centre. Travel and accommodation logistics for the typical 2 to 4 week pre-treatment workup and post-infusion monitoring window are part of the operational plan.

If a UK consultant elects to deliver Elevidys within a UK paediatric neuromuscular centre under the Specials framework, Great Ormond Street Hospital, Evelina London, Birmingham Children's Hospital, Manchester Children's, Sheffield Children's, Royal Hospital for Children Glasgow, and Bristol Royal Hospital for Children hold the relevant cell and gene therapy infrastructure, subject to Sarepta's authorised treatment centre designation policy and any UK supply chain arrangements with Roche.

Real cost picture for Elevidys

Elevidys's US list price is approximately USD 3.2 million for the one-time infusion, making it among the highest-priced medicines globally. At 0.79 GBP to 1 USD the product converts to roughly GBP 2.53 million. The full US treatment cost stack adds the infusion admission, the pre-medication and tapering oral steroid regimen (typically 6 to 8 weeks of high-dose corticosteroids), the cardiac and liver function monitoring (echocardiogram, troponin, liver function panel), and the longitudinal motor function follow-up. For a US-based Elevidys case the all-in cost typically lands in the USD 3.4 million to USD 3.7 million range (approximately GBP 2.69 million to GBP 2.92 million).

UK private medical insurance does not fund Elevidys. NHS Specialised Commissioning does not fund Elevidys in the UK at present (the medicine lacks UK marketing authorisation). Self-funding through family fundraising, manufacturer expanded access where available, or clinical trial enrolment are the operative funding routes.

Typical timeline for Elevidys

The Elevidys timeline runs as follows for a UK family travelling to a US authorised treatment centre: Months 0 to 1 is the clinical eligibility assessment in the UK with the paediatric neurologist, US treatment centre referral, AAVrh74 neutralising antibody titre screening, baseline cardiac and respiratory function workup, travel and visa logistics. Months 1 to 2 is the US centre admission, pre-treatment workup confirmation, corticosteroid pre-medication run-in, and Elevidys infusion. Months 2 to 4 is the post-infusion intensive monitoring window with cardiac, liver, and motor function assessments, and corticosteroid tapering. Long-term follow-up continues at the UK paediatric neurology centre with regular cardiac and motor function review.

What your UK paediatric neurologist needs to provide

The treating UK consultant paediatric neurologist supports the case through US authorised treatment centre referral. The clinical packet typically includes the DMD genetic confirmation (DMD gene mutation analysis, specifying exon deletion, duplication, or point mutation), the patient's age and weight, the AAVrh74 neutralising antibody titre, baseline cardiac function (echocardiogram with ejection fraction, ECG, BNP), baseline liver function panel, baseline motor function assessment (North Star Ambulatory Assessment, 4-stair climb, time-to-rise, 10-metre walk), pulmonary function (forced vital capacity), and the consultant's clinical letter supporting US treatment centre referral.

Long-term follow-up after return to the UK includes annual cardiac MRI and echocardiogram, ongoing motor function assessment, and the standard DMD multidisciplinary clinic schedule.

Common questions about Elevidys for UK families

Is Elevidys available on the NHS? Not at present. The medicine does not hold UK marketing authorisation as of mid-2026. NICE technology appraisal and NHS England commissioning will follow EMA and MHRA approval if and when granted.

What is the age range for Elevidys? The FDA full approval in June 2024 covers ambulatory and non-ambulatory DMD patients aged 4 years and older. There is no upper age limit in the FDA label, but clinical experience and trial data are concentrated in younger ambulatory boys.

What about the AAVrh74 antibody requirement? Elevidys requires a negative or below-threshold AAVrh74 neutralising antibody titre. AAV9-based gene therapies like Zolgensma use a different capsid and the antibody profile is not interchangeable. The AAVrh74 antibody screen is part of the eligibility workup.

What are the major risks? The principal risks include immune-mediated myositis (with creatine kinase elevation and weakness), myocarditis (with cardiac troponin elevation), hepatotoxicity (managed with the corticosteroid pre-medication and tapering regimen), and the broader risks of AAV gene therapy administration. The corticosteroid pre-medication and tapering is integral to the treatment protocol; families should expect 6 to 8 weeks of high-dose corticosteroid exposure with the associated short-term side effects.

Can a UK family travel to the US for Elevidys? Yes, in principle. The practical considerations include US authorised treatment centre acceptance, funding, the multi-week stay including pre-treatment workup and post-infusion monitoring, visa logistics, and long-term UK follow-up arrangements. Reserve Meds coordinates the US treatment centre liaison and logistics.

What about combination with other DMD therapies? Patients on background corticosteroids (prednisolone or deflazacort) continue these around the Elevidys infusion and tapering window. The interaction with ataluren (Translarna) for nonsense-mutation patients, with exon-skipping antisense oligonucleotides (eteplirsen, golodirsen, viltolarsen, casimersen), and with newer agents is a clinical decision based on the patient's specific mutation and treatment history.

Where Reserve Meds fits in Elevidys cases

Reserve Meds is a US-based concierge coordinator. For Elevidys the realistic UK access route is treatment at a US authorised treatment centre. Our role spans US treatment centre liaison, the international logistics for the patient and family across the multi-week pre-treatment, infusion, and monitoring window, and operational support to the UK paediatric neurology consultant before, during, and after the US treatment episode. We do not replace your UK consultant or the US treatment centre's clinical team.

The Yellow Card pharmacovigilance pathway for Elevidys

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 Elevidys 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 Elevidys

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 Elevidys, 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 Elevidys 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 Elevidys 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 Elevidys, 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.

reserved for you.

Composite case examples. This document is for general information only and does not constitute medical advice. Please consult your treating physician.
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