Casgevy

United Kingdom · access guide

Casgevy access in the United Kingdom: the Specials Licence pathway

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

Quick orientation

Casgevy (exagamglogene autotemcel) is the first CRISPR-Cas9 gene-edited cell therapy ever approved anywhere in the world. The MHRA granted Casgevy a conditional marketing authorisation on 16 November 2023, making the United Kingdom the first country globally to authorise the medicine. The MHRA approval covers two indications: severe sickle cell disease with recurrent vaso-occlusive crises in patients aged 12 years and older, and transfusion-dependent beta-thalassaemia in patients aged 12 years and older. NICE issued positive guidance in August 2024 recommending Casgevy for severe sickle cell disease through NHS England's Innovative Medicines Fund, and a separate positive recommendation for transfusion-dependent beta-thalassaemia. UK patients with severe sickle cell disease now have an NHS-funded path to a potentially curative one-time therapy.

Despite this strong UK position, access is gated by centre capacity. There are seven NHS specialist haemoglobinopathy centres able to deliver Casgevy in adult and paediatric form, and slot capacity is the rate-limiting factor. Reserve Meds coordinates US-side sourcing and cell therapy operational support for private patients and for UK patients seeking parallel routes.

Why UK sickle cell and thalassaemia patients pursue Casgevy

The UK has approximately 17,000 people living with sickle cell disease and around 1,000 living with transfusion-dependent beta-thalassaemia, with significant concentration in London, the West Midlands, Manchester, and West Yorkshire. For severe sickle cell disease the only previously curative option was an allogeneic stem cell transplant with a matched donor, available to a small minority of patients. Casgevy changes the equation by offering a one-time autologous gene-edited cell therapy that increases foetal haemoglobin and reduces or eliminates vaso-occlusive crises.

NHS England commissioning began rolling out Casgevy through the Innovative Medicines Fund in late 2024 and through 2025. The seven specialist centres are building cell therapy capacity. Patients facing a 12 to 24 month wait for an NHS slot may pursue private routes or international cell therapy centre options in parallel.

The Specials Licence pathway for Casgevy in the UK

Casgevy holds a UK marketing authorisation, so the standard NHS England commissioning route through the Innovative Medicines Fund is the principal access pathway. The Specials route remains relevant in two scenarios: paediatric or adult patients sitting outside the NICE-recommended criteria where their haematologist judges clinical appropriateness, and private patients pursuing the therapy outside the NHS queue.

For Vertex Pharmaceuticals and CRISPR Therapeutics, the manufacturers, Casgevy is supplied through a tightly controlled specialty distribution channel. The cell therapy supply chain requires CD34+ haematopoietic stem cell collection by apheresis (typically after plerixafor mobilisation, not G-CSF because of vaso-occlusive risk in sickle cell), shipment of the apheresis product to the Vertex manufacturing facility for CRISPR editing of the BCL11A erythroid enhancer, quality release, and cryopreserved return to the treating centre for myeloablative conditioning (typically busulfan) and infusion. The Specials documentation supports the import notification and chain-of-custody record for the cell therapy product return shipment.

Where Casgevy is delivered in the UK

The seven NHS England specialist haemoglobinopathy centres designated to deliver Casgevy include King's College Hospital London (the highest-volume sickle cell centre in the UK), University College London Hospitals, Manchester Royal Infirmary, the Royal Hospital for Sick Children at Glasgow (paediatric), Birmingham Children's Hospital, Sheffield Teaching Hospitals, and Newcastle upon Tyne Hospitals NHS Foundation Trust. Great Ormond Street Hospital and Birmingham Children's Hospital are the principal paediatric Casgevy centres. King's College Hospital London delivered the world's first NHS Casgevy patient infusion in early 2025.

For private patients HCA Healthcare UK at University College Hospital and The Royal Marsden Private Care can support specific elements of cell therapy delivery, but the apheresis-manufacture-conditioning-infusion-monitoring chain is overwhelmingly delivered through the NHS specialist haemoglobinopathy centres for both NHS and private cases. Casgevy is not a community pharmacy or homecare medicine; it is an intrinsically inpatient cell therapy.

Real cost picture for Casgevy in the UK

Casgevy's US list price is approximately USD 2.2 million for the one-time infusion. At 0.79 GBP to 1 USD the product converts to roughly GBP 1.74 million. NHS contracted pricing under the Innovative Medicines Fund managed access agreement is subject to a confidential commercial discount, the terms of which are not public.

The full cost stack for a private Casgevy case extends to the apheresis with plerixafor mobilisation, the myeloablative busulfan conditioning, the inpatient transplant unit admission (typically 4 to 6 weeks), the long-term haematology and chimerism monitoring, and the fertility preservation that is recommended before busulfan exposure. For a private UK Casgevy case the all-in cost typically lands in the GBP 1.85 million to GBP 2.1 million range, with the cell therapy product representing the dominant share. UK private medical insurance does not typically fund Casgevy on standard policies; self-funding or NHS routing are the operative options.

Typical timeline for Casgevy in the UK

The Casgevy end-to-end timeline is set by the gene-editing manufacture cycle and the conditioning and recovery process, not by Specials paperwork. The realistic timeline runs as follows: Months 0 to 2 is the clinical eligibility assessment, fertility preservation counselling, baseline organ function workup, and centre slot booking. Months 2 to 3 is plerixafor mobilisation and CD34+ stem cell apheresis. Months 3 to 6 is the CRISPR cell editing manufacture cycle at Vertex (approximately 16 weeks). Month 6 is myeloablative busulfan conditioning and Casgevy infusion. Months 6 to 8 is inpatient transplant unit monitoring and early haematological recovery. Months 8 to 24 is the chimerism tracking and HbF response monitoring window. Years 2 to 15 is the post-marketing follow-up window.

What your UK haematology consultant needs to provide

The treating UK consultant haematologist (typically a haemoglobinopathy specialist within a designated specialist centre) is the prescribing physician of record. The clinical packet typically includes the haemoglobinopathy diagnosis and genotype (HbSS, HbSC, HbS-beta-thalassaemia, transfusion-dependent beta-thalassaemia subtype), the documented vaso-occlusive crisis history (typically requiring at least 2 severe crises per year over 2 years for sickle cell disease) or the transfusion dependence history, current organ function and any chronic complications (avascular necrosis, pulmonary hypertension, nephropathy, retinopathy, stroke history), fertility preservation discussion documentation, and baseline cardiac, pulmonary, hepatic, and renal function panels.

The MHRA Yellow Card scheme remains the active pharmacovigilance route for any suspected adverse reaction. Long-term gene therapy follow-up requirements include 15-year safety monitoring per regulatory expectations.

Common questions about Casgevy in the UK

Will the NHS fund Casgevy? Yes. NICE recommended Casgevy in August 2024 for severe sickle cell disease and for transfusion-dependent beta-thalassaemia. NHS England commissions through the Innovative Medicines Fund. The seven specialist haemoglobinopathy centres are the access points.

How long is the NHS waiting list? The published cell therapy slot wait time has run from 12 to 24 months at peak demand, though capacity is expanding. The fastest-routing scenario depends on geography, sickle cell disease severity, and the local centre's caseload.

What is the difference between Casgevy and Lyfgenia? Both are one-time autologous gene therapies for sickle cell disease but with different mechanisms. Casgevy uses CRISPR-Cas9 to edit the BCL11A erythroid enhancer and increase foetal haemoglobin. Lyfgenia uses a lentiviral vector to add a modified beta-globin gene. The US FDA approved both on the same day in December 2023; the UK MHRA approved Casgevy first (November 2023) and Lyfgenia has not yet received UK marketing authorisation as of late 2024.

Is Casgevy approved for paediatric patients? The UK marketing authorisation covers patients aged 12 years and older, the same as the FDA label. Paediatric cell therapy capacity at Great Ormond Street and Birmingham Children's covers the 12 to 17 year-old segment.

What are the major risks? The principal risks are those of the myeloablative busulfan conditioning regimen: infertility (mitigated by pre-treatment fertility preservation), severe and prolonged cytopenias with infection risk during the engraftment window, mucositis, and the broader risks of a stem cell transplant. The CRISPR gene-editing component carries theoretical long-term safety concerns that the 15-year post-marketing follow-up is designed to characterise. Patients should review the full Patient Information Leaflet with their consultant.

What if I am in Scotland, Wales, or Northern Ireland? Scotland has its own SMC pathway and the Royal Hospital for Sick Children Glasgow is a designated paediatric centre. Welsh patients are typically referred to English specialist haemoglobinopathy centres. Northern Ireland patients route through specialist centres in England.

Where Reserve Meds fits in Casgevy cases

Reserve Meds is a US-based concierge coordinator. For Casgevy specifically, the NHS England commissioning route through the Innovative Medicines Fund is the principal access pathway for UK patients. Our role is most relevant for private patients pursuing parallel routes, for patients seeking documentation support across complex multi-centre coordination, and for patients exploring the international US-based cell therapy options (Casgevy is delivered at approximately 50 US authorised treatment centres). We do not replace your haematology consultant or the UK specialist haemoglobinopathy centre; we provide operational support and a single named coordinator across what is intrinsically a complex care pathway.

The Yellow Card pharmacovigilance pathway for Casgevy

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 Casgevy 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 Casgevy

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 Casgevy, 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 Casgevy 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 Casgevy 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 Casgevy, 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.

Reserve Meds

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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