Casgevy

Qatar · access guide

Casgevy (exagamglogene autotemcel) for a Qatari family: what the pathway looks like in 2026

By Reserve Meds clinical & regulatory team. Last reviewed 2026-05-20.

Sidra Medicine in Doha established a dedicated Gene Therapy Center for Pediatric Rare Diseases in 2025. Sidra was the first hospital outside the United States, and the fifth globally, to administer Elevidys gene therapy for Duchenne muscular dystrophy. As of 2026, Sidra has Qatar Ministry of Public Health authorisation to administer Casgevy for sickle cell disease and transfusion-dependent beta-thalassemia in patients aged 12 and older.

For a Qatari family considering Casgevy, the conversation is unusual in MENA because the gene-therapy infrastructure sits inside Qatar, not at the end of a cross-border journey. Sidra's Gene Therapy Center has the multidisciplinary capacity to run the full pathway: haematology, genetics, BMT, peri-transplant intensive care, and one-year follow-up.

This page is meant to be the first honest read you get on Casgevy in Qatar, written by the team that would coordinate the documentation and second-opinion layer around your child's case if your treating haematologist has raised this with you, or you have raised it with them.

We will be specific about who Casgevy is currently approved for, what the workup at Sidra decides, what the journey actually looks like, what it costs in QAR and US dollars, where Reserve Meds fits given that the operational centre is already in Doha, and what life looks like in the year after treatment.

What Casgevy actually is, in plain terms

Casgevy is the first approved CRISPR/Cas9 gene-edited cell therapy in medicine. It is given as a one-time treatment, but the operational reality is closer to a bone marrow transplant than a one-hour infusion.

Your child's own hematopoietic stem cells are mobilised out of the bone marrow into the blood, harvested through apheresis sessions, shipped to Vertex's manufacturing facility, edited using CRISPR/Cas9 at the erythroid-specific enhancer region of the BCL11A gene, and returned. The edited cells, once reinfused after myeloablative conditioning, reactivate fetal haemoglobin production. For sickle cell disease, fetal haemoglobin reduces sickling. For transfusion-dependent beta-thalassemia, it removes the requirement for chronic transfusions.

The edit is permanent. It does not cross to germline cells. Your child's future children will not inherit the edit. The change is hereditary only at the haematopoietic stem cell line, in your child's own bone marrow.

What Casgevy is not is a treatment that can be given outpatient. Conditioning is myeloablative. The patient is admitted for the conditioning week, the infusion, and four to six weeks of recovery during pancytopenia and engraftment. Outpatient follow-up is monthly for the first year.

Who is currently a candidate, and who is not

The current MOPH-approved indication, aligned with FDA and EMA, is age 12 and older. Your child must have either:

- **Sickle cell disease with a history of recurrent vaso-occlusive crises**, severe enough that the disease meaningfully interferes with their life, or - **Transfusion-dependent beta-thalassemia**, defined by a sustained regular transfusion requirement.

The Sidra Medicine workup will confirm the diagnosis, the severity criteria, and whether your child is a candidate for myeloablative conditioning. Cardiac, pulmonary, hepatic, and renal function must be adequate. For TDT patients in particular, iron overload from years of transfusion needs to be assessed and managed.

If your child is under 12, Vertex is preparing 2026 submissions to expand the approved age range, but the current indication does not include younger patients. We will not pretend otherwise. Reach out anyway. We can discuss monitoring and supportive care that fits between now and a potential future eligibility window.

If your child has SCD that has not produced recurrent VOCs, the case for Casgevy is harder. Sidra and the wider international community typically look for documented VOC history before approving. We are honest about this.

The Qatar pathway in 2026

Casgevy is formally approved by Qatar's Ministry of Public Health, Department of Pharmacy and Drug Control, for patients aged 12 and older with SCD or TDT. Qatar is among the first countries globally to authorise the therapy.

For a Qatari family or a Qatar-resident expatriate family pursuing Casgevy, the practical pathway runs almost entirely through Sidra Medicine:

- **Referral**. Your treating haematologist refers to Sidra Medicine's Gene Therapy Center for Pediatric Rare Diseases. For Qatari children already in the Sidra paediatric haematology service, the referral is internal. - **Sidra workup**. Sidra's multidisciplinary team, spanning haematology, genetics, cardiology, BMT, and rehabilitation, completes the candidate assessment. Two to four weeks typically. - **MOPH coordination and Vertex procurement**. Sidra's import pharmacy and MOPH Department of Pharmacy and Drug Control handle the regulatory and procurement layer. Because Casgevy is on the MOPH approved list, this is standard prescription and import, not named-patient. - **Mobilization and apheresis**. G-CSF and plerixafor mobilize hematopoietic stem cells; apheresis harvests them at Sidra over multiple sessions. - **Vertex manufacturing**. The harvested cells go to Vertex's manufacturing facility for CRISPR editing. The waiting period between cell collection and reinfusion is typically four to six months. - **Conditioning**. Inpatient busulfan-based myeloablative conditioning, approximately a week before the infusion, at Sidra. - **Infusion**. Single inpatient infusion of the edited cells at Sidra. - **Recovery**. Four to six weeks inpatient during pancytopenia and engraftment. - **Follow-up**. Monthly at Sidra for the first year, then less frequent. Long-term haematology monitoring.

For Qatari nationals being treated under the public health system at Sidra, much of the cost can be underwritten. Direct consultation with the Sidra patient navigator is the right path to confirm.

For expatriate Qatari residents, mandatory private insurance arrangements vary. Specialty drug coverage for one-time gene therapies is typically subject to prior authorisation; approval is uncommon outside specific MOPH-coordinated frameworks. We provide the documentation packet that maximises the chance of approval.

The workup that decides eligibility

The Sidra Gene Therapy Center workup typically covers:

Confirmed diagnosis with detailed phenotype, documented VOC history (for SCD), transfusion history (for TDT), prior hydroxyurea response (for SCD), and iron-chelation history. Your haematologist's records typically cover this.

Bone marrow assessment including cytogenetics.

Cardiac function (echocardiogram, cardiac MRI for TDT patients with iron-overload concerns).

Pulmonary function.

Hepatic function including assessment of any prior hepatitis, iron overload, or transfusion-related hepatic effects.

Renal function.

Iron overload assessment for TDT patients (T2-star cardiac MRI, liver iron quantification).

Infectious disease screening, CMV serology, immunisation review.

Fertility preservation counselling. Myeloablative conditioning typically causes permanent infertility. For adolescents, gamete preservation needs to be discussed before conditioning starts. This is a culturally sensitive conversation. We do not pretend it is anything other than serious. Sidra's fertility preservation team will lead it. We support the family with information and logistics where useful.

Psychosocial assessment for the inpatient stay and the long recovery.

A clinical rationale letter from your treating haematologist documents the indication, severity, prior treatment history, and the transplant plan.

The cost conversation, in the form a Qatari family needs

Casgevy's product list price in 2026 sits at approximately USD 2.2 million, or roughly QAR 8.0 million, for the cell-therapy product itself. That is the manufacturer's price. The full cost of care, including pre-treatment workup, mobilisation, apheresis, the four-to-six-month manufacturing waiting period, conditioning, the inpatient transplant admission, supportive care, and the first year of monitoring, adds substantially. Total cost of care typically runs USD 2.8 to 3.5 million, or QAR 10.2 to 12.7 million.

For Qatari-national families being treated at Sidra under public health system coverage, the financial framing is often very different from cross-border cash-pay. Direct consultation with the Sidra patient navigator is the right path. Reserve Meds will not speculate about the Qatari public-system financial structure on a public page.

For expatriate residents and self-pay families, the standard cash-pay-with-documentation pattern applies. We separate every line in the quote: cell-therapy product, mobilisation drugs, apheresis sessions, conditioning drugs, inpatient admission, supportive care, monitoring labs, our coordination fee. We do not put a markup on the manufacturer's drug price. Our coordination fee is disclosed in writing before any funds move.

The year after

The first four to six weeks inpatient at Sidra are the highest-acuity period. The patient is functionally immunocompromised during the engraftment window. Infection prophylaxis, transfusion support, and intensive monitoring run the daily care.

After discharge, the patient is on a structured outpatient follow-up: monthly haematology visits at Sidra for the first year, with declining frequency thereafter. Transfusion requirement typically falls off within months for TDT patients who achieve engraftment, and VOC frequency typically falls off for SCD patients.

Long-term, lifelong haematology surveillance is standard. The vector is non-integrating from a genomic-insertion standpoint, but long-term monitoring is standard for any one-time gene therapy. Long-term data accumulation is ongoing globally; Sidra's Elevidys cohort has been published in peer-reviewed literature, and the Casgevy cohort will be similarly documented over time.

Practical implications for a Qatari family: a substantial portion of a year of normal life is reorganised around the treatment. School attendance for adolescent patients will be interrupted for the inpatient and recovery period. Sidra coordinates with the school on tutoring or remote-learning support as needed. Siblings, parents, and the extended family network typically reorganise their schedules around the inpatient admission.

What Reserve Meds does for a Qatari family

Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. For a Qatari family pursuing Casgevy, the operational centre is already in-country at Sidra Medicine. Our role is different from the cross-border cases we coordinate elsewhere.

For families being treated at Sidra under MOPH or public-system coverage: Sidra runs the operational coordination end to end. Reserve Meds is most useful as a documentation and international second-opinion concierge layer. We can help with international second-opinion clinical reviews from US Vertex Authorized Treatment Center transplant specialists, prior-authorisation documentation for private insurance overlays, and translation of medical records.

For expatriate Qatar-resident families whose insurance pathway needs a fuller documentation packet, or whose family is split across countries and needs the cross-border logistics layer, we operate at the standard scope.

For families considering international Casgevy (US or Europe Vertex Authorized Treatment Center) rather than Sidra: standard Reserve Meds scope. Regulatory documentation, qualified-centre liaison, named case-lead coordination from intake through one-year follow-up, travel and accommodation logistics for the lengthy stay, and the cross-border financial structure.

Reserve Meds is not your child's prescriber. We do not practise medicine. We do not manufacture Casgevy. We do not own or operate Sidra Medicine, Hamad Medical Corporation, or any other treatment centre. Clinical decisions stay with your treating haematologist and the Sidra transplant team.

We work cash-pay where applicable. Our coordination fee is disclosed in writing.

A note for families weighing this

Sickle cell disease and transfusion-dependent beta-thalassemia have long histories in many Qatari and resident MENA families. We assume you and your wider family have lived with this for years already, often across multiple affected family members. A potentially curative one-time therapy is a different kind of decision than the chronic-care adjustments you have made. We are not trying to push that decision. The right consultation pace is the one your treating haematologist and your family set together.

For Muslim families thinking through the religious-ethical dimension, the Islamic bioethics consensus on disease-modifying therapies that preserve life and function is broadly permissive, including for gene-editing therapies that do not alter the germline. The fertility-preservation conversation has its own religious-ethical layer; families typically consult both their treating clinician and their religious advisor before committing. We will not pressure either conversation.

Families typically take between two and six weeks from first call to readiness for the formal workup. Given Sidra's in-country presence, the timeline can be more compressed than for cross-border cases. The four-to-six-month manufacturing waiting period after cell collection means the total treatment arc is still close to a year. We are honest about that.

What to do if you want to start

If your child meets the basic eligibility (age 12 or older, SCD with recurrent VOCs or TDT), the first concrete step is a call with our case-lead so we can confirm the right pathway for your family. For most Qatari and Qatar-resident families, that will be Sidra Medicine; for some, it will be international referral.

If your child is under 12, has SCD without recurrent VOCs, or is in a situation where Casgevy is not currently the answer, reach out anyway. We can discuss timing, supportive care, and alternative options including Lyfgenia for eligible SCD patients.

Most families reach us first on WhatsApp, which we hold open during Qatar business hours and on weekends for active cases.

Start your child's case on the portal, or open a WhatsApp conversation with the case-lead and we will take it from there.

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.

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