

## Casgevy

Bahrain · access guide

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

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

Bahrain has one of the highest documented sickle cell disease carrier rates in the world. Public-health surveys over the past two decades have repeatedly shown that the disease carries deep multi-generational presence in Bahraini families, with particularly high concentration in some communities. Transfusion-dependent beta-thalassemia is similarly part of the country's haematology landscape. For a Bahraini family that has lived with SCD or TDT across siblings or generations, Casgevy is the first potentially curative one-time therapy approved anywhere in medicine, and Bahrain's National Health Regulatory Authority is one of the regulators that has acted on it.

This page is meant to be the first honest read you get on Casgevy in Bahrain, written by the team that would coordinate around your child's case if you decided to go forward. We assume 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, where the workup happens in Bahrain, where the actual infusion occurs (Bahrain does not have an in-country Casgevy administration centre as of 2026; Sidra Medicine in Doha is the closest with deep documented experience), what it costs in BHD and US dollars, how MoH treatment-abroad funding may interact with the case, and what life looks like in the year after.

## 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

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The NHRA, FDA, and EMA approved indication 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 work-up 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 what monitoring and supportive care fit 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. Most international centres and the regional cross-border programmes look for documented VOC history before approving. We are honest about this.

## The Bahrain pathway in 2026

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Bahrain's National Health Regulatory Authority published the Gene Therapy Products Registration and Control Regulations in 2019. The kingdom has had a formal advanced therapy medicinal products framework for over five years. As of 2026, the regulatory pathway is mature; the operational barrier is that Bahrain does not have an in-country Casgevy administration centre. The infusion routes to a regional or international Authorized Treatment Center.

The pathway for a Bahraini family runs:

- **Workup in Bahrain.** King Hamad University Hospital (KHUH) and Salmaniya Medical Complex (SMC) are the natural intake points for the haematology and pre-transplant assessment. Bahrain's specialist paediatric haematology services run the diagnostic confirmation, the severity evaluation, and the initial fitness-for-transplant assessment. Bahrain Specialist Hospital, Royal Bahrain Hospital, and American Mission Hospital provide additional private-sector paediatric and tertiary support where useful. - **Referral to an administration centre.** The most operationally simple cross-border option is **Sidra Medicine in Doha**, a 90-minute flight from Manama, with Qatar MOPH authorisation for Casgevy administration in age 12+ SCD and TDT, and the deepest documented paediatric gene-therapy programme in MENA. Alternative pathways include **Yas Clinic Hospital in Abu Dhabi** (which administered the UAE's first Casgevy case in April 2026), and Vertex's US and European Authorized Treatment Center network. - **Cross-border regulatory and procurement layer.** The receiving centre's import pharmacy handles the Vertex procurement under their own regulator. Bahrain MoH treatment-abroad funding documentation, when applicable for Bahraini nationals, runs in parallel. - **Mobilization, apheresis, manufacturing wait, conditioning, infusion, recovery.** At the receiving centre, over the standard four-to-six-month treatment arc. - **Return to Bahrain for long-term follow-up.** Monthly haematology visits transition back to KHUH or SMC after the high-acuity recovery period, in coordination with the administration centre's transplant team.

## The workup that decides eligibility

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Several results need to land before the transplant pathway opens, whether at Sidra or another administration centre.

**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. The receiving centre's fertility preservation team leads it. We support the family with information and logistics.

**Psychosocial assessment** for the inpatient stay and the long recovery, including the cross-border component.

A clinical rationale letter from your treating haematologist documents the indication, severity, prior treatment history, and the transplant plan. For Bahraini nationals seeking MoH treatment-abroad funding, this letter is also part of the funding application.

## The cost conversation, in the form a Bahraini family needs

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Casgevy's product list price in 2026 sits at approximately USD 2.2 million, or roughly BHD 828,000, 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 for cases routed cross-border or paid cash typically runs USD 2.8 to 3.5 million, or BHD 1.05 to 1.32 million.

For Bahraini nationals with MoH treatment-abroad funding, public funding may underwrite much of the cross-border cost. The funding application is case-by-case and the documentation packet needs the right structure. We can support that side.

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 at the receiving centre, supportive care, monitoring labs, travel and accommodation for the family during the lengthy stay, 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.

Expatriate insurance coverage for one-time gene therapies via AXA Gulf, Bahrain National Insurance, GIG Bahrain, and others is typically subject to prior authorisation; approval is uncommon. We provide the documentation packet that increases approval likelihood.

## The year after

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The first four to six weeks inpatient at the administration centre 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 the administration centre for the early high-acuity phase, transitioning back to Bahrain (KHUH or SMC) for the bulk of the first-year monitoring once the patient is stable. 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.

Practical implications for a Bahraini family: a substantial portion of a year of normal life is reorganised around the treatment. The family typically relocates to the administration centre's city for the inpatient and recovery period. School attendance for adolescent patients will be interrupted. We coordinate 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 Bahraini family

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Reserve Meds is a US-based concierge coordinator for cross-border specialty medicine. For a Bahraini family pursuing Casgevy, our scope depends on which administration centre you choose and what funding pathway applies.

**For Bahraini-national families with MoH treatment-abroad funding routing to Sidra Medicine or another regional centre:** we are most useful as a documentation, second-opinion, and family-logistics layer. Sidra and the receiving centre's teams cover operational coordination. We help with second-opinion clinical reviews, MoH funding documentation, family travel and accommodation logistics, and continuity of care back to KHUH or SMC.

**For families pursuing cross-border Casgevy at Sidra Medicine or Yas Clinic without public funding:** cross-border documentation, qualified-centre liaison, named case-lead coordination, family travel and accommodation logistics for the lengthy stay, and the cross-border financial structure.

**For families pursuing international Casgevy** (US or Europe Vertex Authorized Treatment Center): the standard Reserve Meds scope. Regulatory documentation, qualified-centre liaison, named case-lead coordination from intake through one-year follow-up, travel and accommodation logistics, 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, KHUH, SMC, Yas Clinic Hospital, or any other treatment centre. Clinical decisions stay with your treating haematologist and the receiving centre's transplant team.

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

## **A note for families weighing this**

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Sickle cell disease and transfusion-dependent beta-thalassemia have long histories in many Bahraini 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 same broad framing holds across the Sunni and Shia communities; the underlying principle of preserving life and function is shared. 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. The four-to-six-month manufacturing waiting period after cell collection means the total treatment arc is closer to a year. We are honest about that.

## **What to do if you want to start**

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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 Bahraini families, that conversation will frame Sidra Medicine in Doha as the operationally simplest cross-border option, with Yas Clinic Abu Dhabi or international Vertex Authorized Treatment Centers as alternatives.

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 Bahrain 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.

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### **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.

Reserve Meds is in pre-launch. Published timelines and cost ranges are indicative, not guarantees.

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