Casgevy access in Pakistan

A patient-first guide to accessing Casgevy (exagamglogene autotemcel) for sickle cell disease and transfusion-dependent beta-thalassemia for Pakistani families, with travel-to-treatment coordination through qualified international Authorized Treatment Centers.

Quick orientation

Casgevy is the first CRISPR/Cas9 gene-edited cell therapy approved anywhere in the world, indicated for sickle cell disease (SCD) and transfusion-dependent beta-thalassemia (TDT) in patients aged 12 and older. Pakistan carries one of the highest documented burdens of inherited hemoglobinopathies anywhere in the world, with thalassemia trait estimated at 5 to 7 percent of the national population and sickle cell disease concentrated in Sindh and Balochistan, both driven by autosomal recessive inheritance and the country's elevated consanguinity rate. Casgevy is not registered with the Drug Regulatory Authority of Pakistan (DRAP), and certified Authorized Treatment Centers do not yet exist in Pakistan, so access for Pakistani families is a travel-to-treatment engagement through the United States, the European Union, the United Kingdom, or Saudi Arabia. Reserve Meds coordinates the US-side and international logistics, the qualified-center introduction, and the multi-country family funding workflow. Reserved for you.

Why patients in Pakistan need Casgevy via NPP

The thalassemia and sickle cell burden in Pakistan is structural. Beta-thalassemia trait carrier rates of 5 to 7 percent translate into thousands of transfusion-dependent children identified each year, and the prevalence of SCD among Sindhi, Baloch, and Makrani populations is well-documented. Families typically navigate lifelong red cell transfusion programs, iron chelation, and the steady accumulation of transfusion-related complications. For those with severe phenotypes, the case for a one-time potentially disease-modifying gene therapy is clinically compelling, particularly for adolescents who have already accumulated significant transfusion burden or recurrent vaso-occlusive crisis history.

The access gap for Casgevy in Pakistan is not a "registered but unstocked" problem. It is a deeper structural gap. Casgevy has not been filed with DRAP, the certified Authorized Treatment Center network that Vertex Pharmaceuticals qualifies for cell-therapy delivery does not include any Pakistani institution, and the cryogenic vapor-phase liquid nitrogen handling plus busulfan myeloablative conditioning plus cell-therapy infusion suite infrastructure that the protocol demands is not currently available in Pakistan at the scale and qualification level the manufacturer requires. Aga Khan University Hospital (AKUH) and Shaukat Khanum Memorial Cancer Hospital and Research Centre (SKMCH&RC) operate strong bone marrow transplant programs, but neither holds Vertex's Casgevy ATC qualification at this time.

The practical access route, therefore, is cross-border travel-to-treatment. The patient and family travel to a qualified center in the United States, the United Kingdom, the European Union, or Saudi Arabia, where Casgevy is registered and where the institutional infrastructure exists. Reserve Meds orchestrates the introductions, the documentation, and the supply-side logistics. The clinical decision stays with the family and the treating cell-therapy team.

The DRAP Special Permission pathway for Casgevy

DRAP regulates the import of medicines through the Quality Assurance and Laboratory Testing (QA&LT) Division's Import and Export Section, with the Drug Registration Board overseeing new product registration. For unregistered medicines required for a specific patient, DRAP issues a Special Permission, also called the No Objection Certificate (NOC) for Personal Use Import, filed through the Online Import and Export System (OIES) portal. For most named-patient drug imports into Pakistan, this is the relevant pathway.

For Casgevy specifically, the DRAP filing typically does not target the cell product itself, because the manufactured edited cells are returned directly to the patient at the treating center abroad rather than imported as a stand-alone pharmaceutical into Pakistan. Where the DRAP pathway becomes relevant is for ancillary materials, for return-to-Pakistan follow-up medicines, or where a Pakistani patient is treated abroad and continues post-engraftment monitoring at home. The dominant access architecture is travel-to-treatment, with the regulatory layer concentrated at the destination jurisdiction (FDA for the US, MHRA for the UK, EMA for the EU, SFDA for Saudi Arabia).

Where the Pakistan-side documentation matters most is patient and family preparation. The clinical justification letter from the treating Pakistani physician confirms diagnosis with ICD-10 coding (D57.x for SCD or D56.x for TDT), severity with documented vaso-occlusive crisis history or transfusion dependence, prior therapies attempted (hydroxyurea, voxelotor where available, chronic transfusion, iron chelation) with outcomes, and the clinical rationale for one-time gene-edited cell therapy. The Pakistan Medical and Dental Council (PMDC) licensing verification accompanies the letter. The Pakistani treating team's referral package then travels with the patient to the destination institution.

Regulatory timelines for any DRAP-side filing for routine cases at a major institution run four to eight weeks; complex cases extend to ten to sixteen weeks. The operational clinical timeline at the destination institution (apheresis through manufacturing through conditioning through engraftment) is six months to one year. Mandatory pre-treatment fertility preservation counseling is documented as part of the patient's pre-engagement education at the destination center; the busulfan conditioning regimen carries a high risk of permanent infertility, and the fertility discussion is not optional.

Where Casgevy gets dispensed for Pakistani patients

Within Pakistan, the institutions that handle international referrals and pre- and post-treatment workup most fluently are concentrated in Karachi, Lahore, and Islamabad. Aga Khan University Hospital (AKUH) in Karachi, Shaukat Khanum Memorial Cancer Hospital and Research Centre (SKMCH&RC) in Lahore, and the Indus Hospital and Health Network are the primary referral centers for hematologic gene therapy candidates. Liaquat National Hospital in Karachi, the Pakistan Kidney and Liver Institute (PKLI) in Lahore, the Combined Military Hospitals (CMH) network, the Children's Hospital and Institute of Child Health in Lahore for pediatric cases, and Shifa International Hospital in Islamabad round out the institutional map.

The actual Casgevy delivery takes place at the destination institution abroad. Vertex's qualified Authorized Treatment Center network includes US academic medical centers (Boston Children's Hospital, Memorial Sloan Kettering, City of Hope, Sarah Cannon Research Institute and others), UK NHS designated centers, EU centers in Italy and Germany among others, and in Saudi Arabia primarily King Faisal Specialist Hospital and Research Centre (KFSH&RC). For Pakistani families with relatives in the Gulf, Saudi Arabia is often the closest qualified destination and the most

operationally practical, including for language and cultural fit. For families pursuing the US route, the visa process and the multi-month treatment stay become part of the planning conversation.

Real cost picture for Casgevy for Pakistani patients

The Casgevy cost structure is dominated by the manufactured cell product itself. US wholesale acquisition cost (WAC) is approximately USD 2.2 million per patient for the single one-time infusion (Vertex stated list price at launch). At the current USD to PKR rate (approximately PKR 278 to 280 per USD on 8 to 9 May 2026), the cell product alone converts to roughly PKR 612 to 616 million. This figure covers the cell product only. It does not include apheresis, manufacturing logistics, busulfan conditioning hospitalization, the cell-therapy infusion suite, post-infusion inpatient stay with transfusion and antimicrobial support, fertility preservation, or long-term follow-up.

All-in delivered cost at a qualified destination center is materially higher than the cell-product WAC. Patient and caregiver travel costs (visa, international airfare, multi-month accommodation near the destination institution, in-country transport, food) add a meaningful line item, particularly for the US route. International logistics for the manufactured cell product (cryogenic vapor-phase liquid nitrogen shippers) are absorbed within the treating institution's commercial arrangement with Vertex and do not become a separate line item for the family. The Reserve Meds concierge coordination fee is a separate line item.

Because the Pakistani Rupee has been volatile and inflation rose to 10.9 percent in April 2026, Reserve Meds quotes in USD and accepts wire transfers from any USD-accessible source. Pakistani families regularly pool funding across overseas relatives in Saudi Arabia, the UAE, th