Elfabrio

Saudi Arabia · access guide

Elfabrio (pegunigalsidase alfa-iwxj) for a Saudi adult with Fabry disease: what the pathway looks like in 2026

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

A Saudi adult with Fabry disease walks into the conversation about Elfabrio with the deepest LSD infrastructure in the region directly available to them. KFSHRC Riyadh runs one of the busiest lysosomal storage disorder programmes in the MENA region, with the Department of Medical Genetics, adult metabolic medicine, cardiomyopathy programme, and nephrology service co-located. KFSH Jeddah and KAMC Riyadh both manage Fabry cases on the western and central Saudi sides. The diagnostic capacity is on Saudi soil; the multidisciplinary surveillance is on Saudi soil; the question is operational, not whether the country has the infrastructure.

This page is the first honest read you get on Elfabrio in Saudi Arabia, written by the team that would coordinate around your treatment plan if you decided you wanted operational support on the workup, the choice among the three ERTs, the SFDA filing where named-patient is needed, the NUPCO procurement loop for public-sector cases, the infusion centre, the antibody monitoring, and the long-term cost picture.

We will be specific about what Fabry disease is, what the diagnostic prerequisites are, where Elfabrio sits among the alternatives, what the Saudi regulatory pathway looks like in 2026, what it costs in SAR and US dollars, and what life looks like settling into chronic ERT under KFSHRC, KFSH Jeddah, KAMC, or one of the private Saudi tertiary centres.

What Fabry disease is, in plain terms

Fabry disease is an X-linked lysosomal storage disorder caused by pathogenic variants in the GLA gene. GLA normally produces alpha-galactosidase A, which lives inside lysosomes and breaks down globotriaosylceramide (Gb3). When GLA is faulty, Gb3 and related glycosphingolipids accumulate in lysosomes across vascular endothelium, kidney podocytes, cardiomyocytes, and dorsal root ganglia neurons. The accumulation causes the multisystemic disease.

Classic Fabry presents in childhood with burning hand and foot pain (acroparesthesias), heat and cold intolerance, reduced sweating, gastrointestinal pain, and the characteristic angiokeratomas. Renal failure, hypertrophic cardiomyopathy, and cerebrovascular events emerge in adulthood. Untreated life expectancy in classic Fabry males is reduced by approximately 20 years.

Later-onset and variant phenotypes present in middle adulthood with predominantly cardiac or predominantly renal involvement. Many such patients are identified during the workup for unexplained left ventricular hypertrophy at adult cardiology clinics, or for unexplained chronic kidney disease at adult nephrology clinics.

Female heterozygotes can be severely affected because of X-inactivation. The term "carrier" undersells the clinical reality; many Saudi female Fabry patients have significant disease and benefit from ERT. KFSHRC genetics service has explicit experience with female Fabry phenotyping.

The X-linked inheritance pattern means mothers can transmit to sons and daughters; fathers transmit to all daughters and to no sons. The cascade-testing conversation for first-degree relatives is part of standard care. In Saudi Arabia, where consanguineous-marriage patterns concentrate recessive disease, the family-screening conversation is especially load-bearing, even though Fabry itself is X-linked rather than autosomal recessive.

The diagnostic prerequisite that has to be in place

You cannot start Elfabrio without confirmed Fabry disease. The workup has two prongs:

Enzyme assay. Alpha-galactosidase A activity measured in leukocytes or dried blood spot. Diagnostic in classic-Fabry males (low or absent activity). Not reliable in female heterozygotes, who often have normal enzyme activity because of X-inactivation patterns.

GLA gene sequencing. Identifies the pathogenic variant. Confirmatory in males; primary diagnostic tool in females. Also informs the migalastat question: roughly 35 to 50 percent of GLA variants are amenable to the oral pharmacological chaperone, and amenability testing should be done before assuming ERT is the only option.

Supporting biomarkers: plasma or urine lyso-Gb3, elevated in Fabry, baseline biomarker for treatment monitoring.

Baseline organ assessment at diagnosis: echocardiogram with strain imaging, cardiac MRI with T1 mapping where available, eGFR, albuminuria, urine protein/creatinine ratio, audiology, ophthalmology (cornea verticillata is Fabry-specific), neurological screen including small fibre neuropathy assessment, and brain MRI for white matter lesions and silent infarcts.

KFSHRC Riyadh runs the enzyme assay, the gene sequencing, the lyso-Gb3 biomarker assay, and the multidisciplinary baseline organ assessment in-house. KFSH Jeddah and KAMC Riyadh route to KFSHRC or to international Fabry reference laboratories where needed. For amenability testing the standard is to send the genotype to one of the international Fabry reference laboratories; the GLA-variant-to-amenability mapping is a curated database that the geneticist accesses on a per-patient basis.

A clinical rationale letter from your treating geneticist documents the diagnosis (enzyme plus genetic), the amenability status for migalastat, the baseline organ-involvement picture, the recommended ERT choice and the reasoning, and the long-term monitoring schedule.

Where Elfabrio sits among the alternatives

Elfabrio is the third commercial ERT for Fabry disease. The choice among them is the treating geneticist's, based on patient-specific factors. We are not here to tell you one is better than another. We will be specific about the menu:

Agalsidase alfa (Replagal, Takeda): 0.2 mg/kg every 2 weeks IV, approximately 40 minute infusion, CHO-cell-derived. Not FDA approved (available in the EU and in MENA). Historically lower infusion-reaction rate.

Agalsidase beta (Fabrazyme, Sanofi): 1 mg/kg every 2 weeks IV, CHO-cell-derived. FDA and EMA approved. Long track record. Anti-drug antibody development common.

Elfabrio (pegunigalsidase alfa-iwxj, Chiesi/Protalix): 1 mg/kg every 2 weeks IV, plant-cell-expressed in tobacco cell culture, PEG-modified. FDA approved May 2023 (adults). EMA approved May 2023 (age 8+). The PEGylation is intended to extend plasma half-life and reduce immunogenicity.

Migalastat (Galafold, Amicus): oral pharmacological chaperone, 123 mg every other day. Adults with amenable GLA mutations only. Where amenable, the alternative to lifelong IV ERT.

The BALANCE trial demonstrated non-inferiority of Elfabrio to agalsidase beta on annualised eGFR slope over 24 months. The BRIDGE trial showed stable or improved renal function in patients switching from Replagal to Elfabrio over 12 months. The choice in any given patient is a clinical judgement taking into account antibody status, infusion-reaction history, prior treatment response, family preference, and local supply.

The Saudi regulatory pathway: how it actually works in 2026

The Saudi Food and Drug Authority (SFDA) is the federal authority. Where Elfabrio holds formal Saudi registration, standard prescription and import procurement applies through the qualified-centre hospital pharmacy. Where formal registration is not yet in place, the SFDA named-patient mechanism is the realistic pathway, filed by the hospital's import pharmacy on the treating geneticist's behalf. [VERIFY: SFDA Elfabrio 2026 registration status]

For public-sector cases, the National Unified Procurement Company (NUPCO) handles centralised purchasing for MoH facilities and several major specialty hospitals. NUPCO procurement loop coordination is part of the operational picture for KFSHRC, KFSH Jeddah, KAMC Riyadh, King Fahad Medical City, and other Saudi public-sector tertiary centres. Private-sector cases at Dr Sulaiman Al Habib network, Saudi German Hospital, or Al Mouwasat work through commercial distribution.

For Saudi nationals, the Council of Cooperative Health Insurance (CCHI) regulates insurance coverage and the rare-disease pathway is workable for adult LSD cases at the major tertiary centres. CHI coverage of recent-FDA-approval biologics like Elfabrio is uneven; the geneticist's letter documenting why Elfabrio rather than Fabrazyme is the recommended choice is the gating step.

In our experience coordinating Fabry ERT cases in Saudi Arabia, the timeline from filing to first infusion runs three to eight weeks depending on whether NUPCO procurement is in the loop and whether antibody-status documentation (for switches from Fabrazyme or Replagal) is needed.

The realistic Saudi infrastructure for adult Fabry disease ERT: - **KFSHRC Riyadh.** Department of Medical Genetics with the deepest LSD programme in the region. Adult metabolic medicine, cardiomyopathy programme, nephrology, infusion unit with anaphylaxis-management capability. The Saudi anchor for adult Fabry. - **KFSH Jeddah.** Genetics and adult metabolic infrastructure for western Saudi patients. Coordinates with KFSHRC Riyadh for the most complex cases. - **KAMC Riyadh (King Abdulaziz Medical City, National Guard).** Adult internal medicine, genetics referral, cardiology and nephrology depth. - **King Fahad Medical City, Riyadh.** Cardiology and nephrology for the Fabry cardiac and renal phenotypes; genetics referral. - **KKUH Riyadh (King Khalid University Hospital).** Adult metabolic and genetics; more limited LSD-specific volume. - **Dr Sulaiman Al Habib network.** Private-sector adult Fabry care across multiple Saudi cities; pulls in KFSHRC genetics consultation for complex cases. - **Saudi German Hospital Riyadh and Jeddah.** Private-sector adult internal medicine and infusion-suite capability.

For Saudi patients who need a multi-day cardiac or renal workup, KFSHRC Riyadh remains the regional default. Reserve Meds coordinates the in-Saudi pathway and the cross-border pattern (typically not needed for Saudi patients given KFSHRC depth, but occasionally relevant for second-opinion or family-research-registry purposes).

The access pathway in Saudi Arabia: step by step

1. Diagnostic confirmation (enzyme assay plus GLA sequencing) on Saudi soil; migalastat amenability check via international reference laboratory if not yet done. 2. Clinical geneticist consultation at KFSHRC Riyadh, KFSH Jeddah, KAMC, or KFMC with the documentation packet from Reserve Meds. 3. Baseline multidisciplinary organ assessment: cardiac (echo, MRI with T1 mapping), renal (eGFR, albuminuria, biopsy in selected cases), neurological, audiology, ophthalmology. 4. ERT choice decision with the treating geneticist. Antibody testing if switching from another ERT. 5. SFDA filing through the hospital's import pharmacy (or NUPCO procurement loop for public-sector cases) with Reserve Meds providing the documentation packet. 6. First Elfabrio infusion at the qualified Saudi centre under geneticist or metabolic specialist supervision. Pre-medication titrated based on infusion-reaction history; anaphylaxis-management capability on site. 7. Stable every-2-week infusion routine established over the next 2 to 3 months; infusion duration shortens from approximately 3 hours to approximately 1.5 hours as tolerance is established. 8. Ongoing surveillance: lyso-Gb3 and Gb3 biomarkers at intervals, anti-drug antibody titre at intervals, eGFR every 3 months, echocardiogram annually or more often, neurological reassessment, family cascade testing follow-up.

The cost conversation, in the form a Saudi family needs

The 2026 indicative annual list price of Elfabrio is approximately USD 350,000 to USD 400,000 per year for an average-weight adult at 1 mg/kg every-2-weeks dosing, or approximately SAR 1.31 million to SAR 1.50 million per year. Over a multi-decade therapy course, cumulative drug cost can sit between USD 10 million and USD 20 million, before counting cardiac valve replacement, dialysis or renal transplantation, and other supportive care.

For Saudi nationals being treated at KFSHRC, KFSH Jeddah, KAMC, or KFMC under the public system, much of the cost is underwritten through the government health funding pathways and the NUPCO procurement loop handles the supply side. The rare-disease pathway through CCHI applies for private-sector patients with eligible insurance plans. Patient out-of-pocket for Saudi nationals at the major tertiary public centres is typically substantially lower than the private-sector or expatriate-resident cost picture.

For expatriate residents, the cost picture is typically a mix of insurance coverage, employer support where applicable, and family-pay. We separate every line in the intake quote: drug per infusion, infusion-suite charges, premedication, antibody and biomarker monitoring labs, cardiac and renal surveillance, our coordination fee. Nothing is bundled. We do not put a markup on the manufacturer's drug price.

Insurance pre-authorisation in the Saudi private sector for Elfabrio specifically (rather than the older Fabry ERTs) often requires the geneticist's letter documenting why Elfabrio is the recommended choice. We supply the insurer with the documentation packet at no charge. We do not process the claim or guarantee coverage.

What to monitor on Elfabrio

The surveillance schedule is built around the multisystemic nature of Fabry disease and the known safety considerations of Elfabrio:

- **Lyso-Gb3 and Gb3 biomarkers** at baseline and at 6-month intervals as biochemical efficacy markers. - **Anti-drug antibody (IgG ADA) titre** at intervals. ADAs develop in approximately 50 percent of Elfabrio patients; mostly without effect on efficacy, but persistent high-titre neutralising antibodies prompt clinical reassessment. - **eGFR and albuminuria** every 3 months. Urine protein/creatinine ratio at the same cadence. Renal stability is the primary long-term efficacy endpoint. - **Echocardiogram** annually, more often based on cardiac phenotype. Strain imaging and LV mass tracking. Cardiac MRI with T1 mapping at intervals. - **Neurological reassessment** annually. Pain diary review. Brain MRI at intervals. - **Audiology and ophthalmology** annually. - **Infusion-associated reaction surveillance** at every infusion. Most reactions are mild to moderate and respond to slowing the infusion and additional premedication; hypersensitivity and rare anaphylaxis remain on the differential. - **Membranous glomerulonephritis surveillance** through urine protein monitoring (rare but documented Fabry-ERT class consideration).

Mental-health screening. Fabry disease carries a meaningful psychosocial burden. Chronic neuropathic pain, progressive cardiac and renal disease, the X-linked inheritance pattern with family-planning weight, and the diagnostic-delay journey many patients have walked all contribute. Depression and anxiety are documented at substantially elevated rates in Fabry cohorts. PHQ-9 screening at baseline and at routine intervals is appropriate; C-SSRS screening where clinical concern arises. Psychiatry or clinical psychology referral should be a standing option in the MDT, not a crisis-only afterthought.

Religious-ethical considerations

Elfabrio is produced in plant cell culture (tobacco, Nicotiana tabacum) and chemically modified with PEG. It is not derived from animal tissue and not derived from human plasma. The plant-cell origin is operationally simpler from a halal framing perspective than mammalian-cell products in some interpretations, though the Sunni and Shia bioethics consensus on disease-modifying therapies for life- and function-preserving indications is broadly permissive regardless of cell-line origin. Families typically consult with their religious advisors before committing; we will not pressure that conversation. We will provide the technical information about the production method when asked.

For Saudi Fabry families weighing the cascade-testing conversation for first-degree relatives, the genetic counselling team at KFSHRC, KFSH Jeddah, or your treating centre is the right home for that conversation. In families where multiple generations are affected, the family-planning and reproductive-counselling conversations often run in parallel with the patient's own treatment-initiation conversation. Reserve Meds coordinates the operational logistics; the clinical conversation stays with your team.

When Elfabrio is not the right answer, or not the only answer

For patients with an amenable GLA mutation, oral migalastat is the alternative to lifelong every-2-week IV ERT. The amenability testing should be done before the ERT conversation closes.

For patients stable on Replagal or Fabrazyme with good clinical and biomarker response and no antibody-related issues, switching to Elfabrio is not automatic. The treating geneticist makes the call based on patient-specific factors.

For patients with very advanced cardiac or renal disease at diagnosis, the conversation includes whether ERT will meaningfully alter the trajectory or whether supportive care (cardiac valve replacement, dialysis, renal transplantation) is the more meaningful intervention. Your geneticist, cardiologist, and nephrologist will frame this candidly.

For patients pursuing emerging gene-therapy programmes for Fabry disease, we can talk through trial eligibility where it applies, but we will not present trial therapy as routinely available.

What Reserve Meds does, and what we do not do

Reserve Meds is a US-based concierge coordinator for cross-border and complex specialty medicine. For a Saudi adult pursuing Elfabrio, our scope is the diagnostic-confirmation pathway routing, the multidisciplinary team documentation packet, the SFDA filing (or NUPCO loop coordination for public-sector cases) in collaboration with your treating hospital's import pharmacy, the sourcing logistics from Chiesi's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the qualified Saudi centre, and named case-lead coordination from intake through the establishment of a stable every-2-week infusion routine.

Reserve Meds is not your prescriber. We do not practise medicine. We do not manufacture Elfabrio. We do not own or operate the infusion centre. We are not your insurer. Clinical decisions stay with your geneticist and the infusion centre team; we are the operational layer that turns those decisions into a coordinated case.

We work cash-pay where applicable. Our coordination fee is disclosed in writing. We will not start work without a signed engagement.

What to do if you want to start

The first concrete step is a call with our case-lead so we can confirm where you are in the diagnostic and clinical picture, and whether the right next move is the workup, the amenability check, the ERT choice conversation with your geneticist at KFSHRC or your treating centre, or the SFDA / NUPCO filing.

If you have been diagnosed with Fabry but have not yet seen a geneticist with Fabry-ERT experience, reach out anyway: we will help you align the MDT before any operational work begins.

Most patients reach us first on WhatsApp, which is the medium we hold open during Saudi business hours and on weekends for active cases.

Start your treatment plan 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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