Elfabrio

Abu Dhabi · access guide

Elfabrio (pegunigalsidase alfa-iwxj) for an Abu Dhabi-based adult with Fabry disease: what the pathway looks like in 2026

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

An Abu Dhabi-resident adult with Fabry disease has the conversation about Elfabrio in the emirate that carries the deepest UAE genetics and adult metabolic medicine infrastructure. The Center for Genetic Diseases at SKMC, the adult metabolic team at SSMC, the genetics service at Tawam Al Ain, and the cardiology institute at Cleveland Clinic Abu Dhabi together form the operational anchor for adult Fabry care on the UAE side. For patients with the cardiac phenotype (hypertrophic cardiomyopathy presentation), CCAD's cardiomyopathy programme is well-suited; for patients with the renal phenotype, SKMC and SSMC nephrology depth carries the day.

This page is the first honest read on Elfabrio in Abu Dhabi, 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 Fabry ERTs, the EDE filing and DoH Abu Dhabi emirate-level layer, the in-emirate MDT alignment, and the long-term cost picture.

What Fabry disease is, in plain terms

Fabry disease is an X-linked lysosomal storage disorder. Deficient alpha-galactosidase A activity allows globotriaosylceramide (Gb3) to 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, heat and cold intolerance, reduced sweating, gastrointestinal pain, and angiokeratomas. Renal failure, hypertrophic cardiomyopathy, and cerebrovascular events emerge in adulthood. Untreated life expectancy in classic males is reduced by approximately 20 years.

Later-onset and variant phenotypes present in middle adulthood with cardiac or renal involvement. Female heterozygotes can be severely affected because of X-inactivation. The "carrier" framing undersells the clinical reality for many women.

The X-linked inheritance pattern means cascade testing of first-degree relatives is part of standard care.

The diagnostic prerequisite that has to be in place

You cannot start Elfabrio without confirmed Fabry disease:

Enzyme assay. Alpha-galactosidase A activity in leukocytes or dried blood spot. Diagnostic in classic-Fabry males; not reliable in female heterozygotes.

GLA gene sequencing. Primary diagnostic tool in females; confirmatory in males. Informs the migalastat amenability question (roughly 35 to 50 percent of mutations are amenable; amenability is the gating decision before assuming ERT is the only option).

Supporting biomarker: lyso-Gb3.

Baseline organ assessment: echocardiogram with strain imaging, cardiac MRI with T1 mapping (CCAD has the depth), eGFR, albuminuria, audiology, ophthalmology (cornea verticillata), neurological screen including small fibre neuropathy assessment, brain MRI.

SKMC's Center for Genetic Diseases runs the enzyme assay, GLA sequencing, and the baseline multidisciplinary organ workup in-house. SSMC and Tawam Al Ain participate in the workup pathway. CCAD's cardiology institute handles the cardiac MRI and detailed cardiomyopathy phenotyping. Amenability testing for migalastat is routed to an international Fabry reference laboratory.

A clinical rationale letter from your treating geneticist or metabolic specialist 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

Three commercial Fabry ERTs plus an oral alternative for amenable mutations:

Agalsidase alfa (Replagal, Takeda): 0.2 mg/kg q2w IV, ~40 min infusion, CHO-derived. **Agalsidase beta (Fabrazyme, Sanofi):** 1 mg/kg q2w IV, CHO-derived. **Elfabrio (Chiesi/Protalix):** 1 mg/kg q2w IV, plant-cell-expressed, PEG-modified, FDA approved May 2023 (adults). EMA approved May 2023 (age 8+). **Migalastat (Galafold, Amicus):** oral pharmacological chaperone for amenable GLA mutations.

The BALANCE trial showed 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 treating geneticist makes the call based on patient-specific factors including antibody status, infusion-reaction history, prior response, and supply.

The Abu Dhabi regulatory pathway: how it actually works in 2026

The Emirates Drug Establishment (federal) plus the Department of Health Abu Dhabi (emirate-level) form the Abu Dhabi pathway. Where Elfabrio holds formal UAE registration, standard prescription and import procurement applies. Where not, the EDE named-patient mechanism is filed by the hospital's import pharmacy on the treating geneticist's behalf, with DoH Abu Dhabi layering its own approval. [VERIFY: EDE Elfabrio 2026 registration status]

For Emirati nationals, Thiqa coverage routes through the DoH Abu Dhabi rare-disease pathway. SEHA-network hospitals (SKMC, SSMC, Tawam, Al Ain Hospital, Mafraq) participate in the Thiqa rare-disease framework. Private-sector providers (CCAD, Burjeel network, NMC Royal, HealthPlus) coordinate with Daman and other commercial insurers.

In our experience coordinating recent-FDA-approval rare-disease ERTs in Abu Dhabi, timeline from filing to first infusion runs three to six weeks. For Elfabrio specifically, the variables are antibody-status documentation (if switching from Fabrazyme or Replagal) and the cardiology and nephrology baseline staging picture.

The realistic Abu Dhabi infrastructure for adult Fabry ERT: - **Sheikh Khalifa Medical City (SKMC), Abu Dhabi.** Center for Genetic Diseases, adult metabolic medicine, infusion-suite capability with anaphylaxis management. The UAE-side anchor. - **Sheikh Shakhbout Medical City (SSMC).** Rare-disease infrastructure, adult metabolic team, cardiology and nephrology depth. - **Tawam Hospital, Al Ain.** Genetics service; can coordinate adult Fabry alongside the longstanding paediatric metabolic programme. - **Cleveland Clinic Abu Dhabi.** Cardiology institute (well-suited to Fabry cardiac phenotype), nephrology, adult internal medicine, rare-disease pathway. - **Burjeel Medical City.** Metabolic clinic and infusion-suite capability. - **NMC Royal Khalifa City.** Adult internal medicine and infusion capability. - **HealthPlus / specialised private adult internal medicine clinics.** Refer in to SKMC, SSMC, or CCAD for the metabolic specialist consultation.

For Abu Dhabi patients who need the deepest LSD programme in the region, KFSHRC Riyadh remains the cross-border default for second opinions or research-registry enrolment; the in-emirate SKMC plus CCAD combination handles the vast majority of cases without cross-border referral.

The access pathway in Abu Dhabi: step by step

1. Diagnostic confirmation (enzyme assay plus GLA sequencing) at SKMC, SSMC, or Tawam; migalastat amenability check via international reference laboratory if not yet done. 2. Clinical geneticist or metabolic specialist consultation at SKMC, SSMC, Tawam, or CCAD with the documentation packet from Reserve Meds. 3. Baseline multidisciplinary organ assessment: cardiac (echo, MRI with T1 mapping at CCAD), renal (eGFR, albuminuria), neurological, audiology, ophthalmology. 4. ERT choice decision with the treating geneticist. Antibody testing if switching. 5. EDE plus DoH Abu Dhabi filing through the hospital's import pharmacy. Thiqa pre-authorisation for Emirati nationals; Daman or commercial insurer pre-authorisation for others. 6. First Elfabrio infusion at the qualified Abu Dhabi centre under geneticist or metabolic specialist supervision. Premedication titrated based on infusion-reaction history; anaphylaxis-management capability on site. 7. Stable every-2-week infusion routine over the next 2 to 3 months. Subsequent infusions can shorten from approximately 3 hours to approximately 1.5 hours as tolerance is established. 8. Ongoing surveillance: biomarkers and antibody titre at intervals, eGFR every 3 months, annual echocardiogram or more often based on cardiac phenotype, periodic geneticist follow-up.

The cost conversation, in the form an Abu Dhabi 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, or approximately AED 1.29 million to AED 1.47 million per year. Over a multi-decade therapy course, cumulative drug cost can reach USD 10 to 20 million, before counting cardiac and renal supportive care.

For Emirati nationals being treated at SKMC, SSMC, or Tawam under the SEHA-Thiqa public-sector framework, much of the cost is underwritten through the government health funding pathways and the DoH Abu Dhabi rare-disease pathway. For expatriate residents, the cost picture is typically a mix of insurance coverage (Daman, commercial), 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 labs, cardiac and renal surveillance, our coordination fee. Nothing is bundled.

Insurance pre-authorisation in Abu Dhabi for Elfabrio specifically often requires the geneticist's letter documenting why Elfabrio rather than Fabrazyme is the recommended choice. We supply the insurer with the documentation packet at no charge.

What to monitor on Elfabrio

- Lyso-Gb3 and Gb3 biomarkers at 6-month intervals. - Anti-drug antibody titre at intervals (ADAs in ~50 percent of patients; high-titre neutralising antibodies prompt reassessment). - eGFR and albuminuria every 3 months. - Echocardiogram annually, more often based on cardiac phenotype. Strain imaging and LV mass tracking. Cardiac MRI with T1 mapping at intervals at CCAD. - Neurological reassessment annually. - Audiology and ophthalmology annually. - Infusion-associated reaction surveillance at every infusion. - Membranous glomerulonephritis surveillance through urine protein monitoring.

Mental-health screening. Fabry disease carries a meaningful psychosocial burden. Chronic neuropathic pain, progressive cardiac and renal disease, X-linked family-planning weight, and the diagnostic-delay history many patients carry all contribute. PHQ-9 screening at baseline and at routine intervals is appropriate; C-SSRS where clinical concern arises. Psychiatry or clinical psychology referral should be a standing option in the multidisciplinary care plan, 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 Abu Dhabi Fabry families weighing the cascade-testing conversation for first-degree relatives, the genetic counselling team at SKMC's Center for Genetic Diseases is the right home for that conversation.

When Elfabrio is not the right answer

For patients with an amenable GLA mutation, oral migalastat is the alternative to lifelong IV ERT. 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.

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.

What Reserve Meds does, and what we do not do

Reserve Meds is a US-based concierge coordinator. For an Abu Dhabi-resident adult pursuing Elfabrio, our scope is the diagnostic-confirmation pathway routing, the multidisciplinary team documentation packet, the EDE plus DoH Abu Dhabi filing in collaboration with the hospital's import pharmacy, the sourcing logistics from Chiesi's authorised distribution through DSCSA-compliant chain of custody, cold-chain shipment to the qualified Abu Dhabi 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 own or operate the infusion centre. We are not your insurer. Clinical decisions stay with your geneticist and the infusion centre team.

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

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 diagnostic confirmation at SKMC, the metabolic specialist consultation, or the EDE filing.

Most patients reach us first on WhatsApp.

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.

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