Skyclarys access in Pakistan

How families in Pakistan access Skyclarys (omaveloxolone) for Friedreich's ataxia through the DRAP Special Permission Personal Use Import pathway.

1. Quick orientation

Skyclarys is the first and, as of May 2026, only disease-modifying therapy approved by the United States FDA for Friedreich's ataxia (FA) in adults and adolescents aged 16 years and older. It is an oral capsule developed originally by Reata Pharmaceuticals and now commercialized globally by Biogen following the 2023 acquisition. Skyclarys is not registered with the Drug Regulatory Authority of Pakistan (DRAP), and Pakistani families with genetically confirmed Friedreich's ataxia (biallelic GAA expansion in the FXN gene, or one expansion with a pathogenic FXN point mutation) reach for the medicine through the DRAP Special Permission Personal Use Import pathway. Reserve Meds coordinates the United States sourcing through the exclusive Biologics by McKesson channel, the regulatory documentation kit, and the international logistics into a Pakistani dispensing facility, while clinical decisions remain with your treating neurologist. Reserved for you.

2. Why Pakistan families need Skyclarys through the named-patient pathway

Friedreich's ataxia is ultra-rare, with a global prevalence of approximately one in 50,000 people. The Pakistani patient population, while small in absolute numbers, is real and is concentrated in families with the typical autosomal recessive inheritance pattern. Before the 2023 FDA approval, there was no disease-modifying therapy for FA, only supportive care. The arrival of the first such therapy created a sharp uptick in family-driven, cross-border demand, but Skyclarys has no DRAP registration in Pakistan and no Gulf or Middle East registration either. Patient advocacy networks such as the Friedreich's Ataxia Research Alliance and Ataxia UK actively educate families about the drug's existence, and diaspora connections in the United States, the United Kingdom, and the Gulf surface the option to Pakistani families.

For a Pakistani family pursuing Skyclarys, the access pattern is structural: the drug is not on the DRAP register at all, no local distributor stocks it, and even where private insurance through Adamjee, Jubilee, EFU, or State Life is in force, ultra-rare neurology imports are typically outside formulary. The DRAP Special Permission Personal Use Import framework is the documented lawful route, and Reserve Meds operates inside that framework as the United States-side coordinator while a PMDC-licensed neurologist at AKUH, Shifa International, or the Combined Military Hospitals network holds the clinical relationship.

3. The DRAP Special Permission pathway for Skyclarys

DRAP regulates the import of medicines through the Quality Assurance and Laboratory Testing Division's Import and Export Section. For unregistered medicines required by a specific patient, DRAP issues a Special Permission, also called the No Objection Certificate (NOC) for Personal Use Import. Applications are filed through DRAP's Online Import and Export System (OIES). For Skyclarys, the cell-specific clinical-justification angle is the genetic confirmation of FA via documented biallelic GAA expansion in the FXN gene (or one GAA expansion plus a pathogenic FXN point mutation), which is the diagnostic gate Reserve Meds requires before coordinating any shipment.

The application package typically includes the treating neurologist's clinical justification letter setting out the Friedreich's ataxia diagnosis, the molecular genetic report confirming the FXN GAA expansion status, the patient's age (label requires 16 years or older), the modified Friedreich Ataxia Rating Scale (mFARS) baseline if recorded, the baseline ALT, AST, and total bilirubin (the label specifies hepatic function monitoring), the baseline BNP and lipid panel (the label specifies cardiac and lipid monitoring given FA-associated cardiomyopathy risk), and the dosing plan (150 mg once daily as three 50 mg

capsules on an empty stomach). The PMDC license verification of the treating physician is attached. The patient identifier is the CNIC for adults or the B-Form for adolescents aged 16 or 17.

Product details include the brand name (Skyclarys), the generic name (omaveloxolone), the manufacturer (Biogen, with originator Reata Pharmaceuticals), strength (50 mg capsules), and the projected three to six month supply quantity. The application also notes that Skyclarys is distributed in the United States through a single exclusive specialty pharmacy, Biologics by McKesson, and Reserve Meds sources through a partner that has procurement access to that channel with a defensible export pathway compliant with United States DSCSA. The dispensing facility's institutional license accompanies the package. Routine personal-use applications typically clear in four to eight weeks from a complete submission. The genetic-confirmation document is the cornerstone that makes DRAP review straightforward.

4. Where Skyclarys gets dispensed in Pakistan

Skyclarys dispensing in Pakistan concentrates at the tertiary neurology services at the major Karachi, Lahore, and Islamabad centers. Aga Khan University Hospital in Karachi has a tertiary neurology service and a 24/7 institutional pharmacy with temperature-controlled storage. Shifa International Hospital in Islamabad serves the federal capital region with neurology services. The Combined Military Hospitals network treats military families and civilian referrals through its tertiary capacity at CMH Rawalpindi and CMH Lahore. Liaquat National Hospital in Karachi has a large adult tertiary service. The Children's Hospital and Institute of Child Health in Lahore is the major pediatric tertiary center for Punjab and is the natural home for adolescent FA cases. Indus Hospital and Health Network in Karachi has institutional infrastructure for ongoing specialty dispensing.

Because Skyclarys is a room-temperature oral capsule with no cold-chain requirement, the dispensing facility constraint is the institutional license and the import-pharmacy workflow rather than refrigerated storage or cell therapy capability. Shipping does not require cold-chain validation, temperature monitoring, or insulated packaging.

5. Real cost picture for Skyclarys in Pakistan

The United States wholesale acquisition cost for Skyclarys was set by Reata at the time of launch at approximately USD 370,000 per year at the labeled 150 mg daily dose. This is the manufacturer-stated United States list price and is widely cited in pricing analyses and trade press coverage of the launch. At approximately 1,095 capsules per year (three capsules per day for 365 days), the implied per-capsule WAC is approximately USD 338. United States patients with commercial insurance and Biogen patient support typically pay a nominal copay; the USD 370,000 figure is the list price before payer negotiation, not patient out-of-pocket. International patient assistance does not apply, and the cross-border anchor is the unsubsidized list-equivalent price.

For a Pakistan family, the cost stack is the United States acquisition cost for the projected refill period plus international air freight (typically USD 400 to USD 800 for a room-temperature capsule) plus the Reserve Meds coordination fee. The Pakistani Rupee has been volatile, trading in the 278 to 280 PKR per USD range in May 2026. Reserve Meds quotes in USD and accepts wire transfers from any USD-accessible source, which lets families consolidate funds across overseas relatives in the Gulf, the United Kingdom, the United States, and Canada. Sehat Sahulat's Rs. 1,000,000 annual ceiling does not stretch to cover annual Skyclarys cost; for FA families this is the single most painful number in the conversation, and pricing transparency upfront is essential so funding can be planned across multiple countries before treatment can start.

6. Typical timeline for Skyclarys in Pakistan

Routine DRAP Personal Use Import applications clear in four to eight weeks from a complete submission. Reserve Meds plans on the longer end of this range for first Skyclarys cases given the recency of FDA approval and the exclusive-specialty-pharmacy procurement path. Because Skyclarys is a room-temperature oral capsule with no cold-chain requirement, the physical logistics leg adds two to four days for international air freight plus FBR Customs clearance at Karachi seaport or Karachi, Lahore, or Islamabad airports. The realistic end-to-end estimate from intake to dispensing is six to ten weeks for the first cycle, with subsequent refill cycles compressing as the documentation pattern is established.

Treatment is chronic and indefinite for as long as the patient tolerates the drug and the prescribing physician judges that clinical benefit is reasonable. Annual supply discussions are appropriate from the first case.

7. What your physician needs to provide

The clinical justification letter, signed by a PMDC-licensed neurologist, is the cornerstone of the application. For Skyclarys, the letter sets out the Friedreich's ataxia diagnosis with the molecular genetic confirmation attached (biallelic GAA expansion in the FXN gene, or one GAA expansion plus a pathogenic FXN point mutation), the patient's age confirming the label-required 16 years or older, the documented baseline mFARS where recorded, the baseline ALT, AST, and total bilirubin (the label requires monthly monitoring of these for the first three months then periodically), the baseline BNP (with cardiac evaluation if elevated, given the FA-associated cardiomyopathy risk), the baseline lipid panel, and the dosing plan of 150 mg once daily as three 50 mg capsules on an empty stomach. The label specifies dose adjustments for moderate hepatic impairment (Child-Pugh B): 100 mg once daily with reduction to 50 mg once daily if adverse reactions emerge. Severe hepatic impairment (Child-Pugh C): avoid use. The treating neurologist owns the safety framing.

Genetic confirmation documentation arriving from Pakistani national or private labs may need translation if originally produced in a non-English language; Reserve Meds intake anticipates that step. Cardiac and hepatic baseline workup is coordinated locally before shipment; Reserve Meds is not a clinical provider and does not perform this workup. Adverse events, if any, are reported through the DRAP Pharmacovigilance Centre.

8. Common questions about Skyclarys in Pakistan

Is genetic confirmation required? Yes. Reserve Meds requires documentation of biallelic GAA expansion in the FXN gene, or one GAA expansion plus a pathogenic FXN point mutation, before coordinating supply. Clinical suspicion of Friedreich's ataxia without genetic confirmation is not sufficient. Pakistani patients without an existing genetic report typically arrange testing locally before the case proceeds.

Will Adamjee, Jubilee, EFU, or State Life cover Skyclarys? Coverage for named-patient imports of unregistered ultra-rare specialty drugs is uncommon across Pakistani health plans. Reserve Meds supplies the documentation an insurer needs to assess the claim, but the claim itself is yours or your hospital's to file. The realistic default is cash-pay.

What is the safety profile? The most common adverse reactions reported in the MOXIe pivotal trial were elevated liver transaminases, headache, nausea, abdominal pain, fatigue, diarrhea, and musculoskeletal pain. Elevations of ALT or AST above 5 times the upper limit of normal occurred in 16 percent of treated patients; elevations above 3 times ULN occurred in 31 percent. These elevations were generally reversible on dose reduction or discontinuation.

Is there a competitor or alternative? No. There is no other FDA-approved or EMA-approved disease-modifying therapy for Friedreich's ataxia. Supportive care (physical therapy, occupational therapy, cardiology management for FA-associated cardiomyopathy, endocrine management for FA-associated diabetes) remains essential and is not displaced by Skyclarys.

Does Skyclarys reverse existing damage? No. The MOXIe pivotal trial demonstrated a placebo-corrected improvement of 2.41 points on the modified Friedreich Ataxia Rating Scale (mFARS) at 48 weeks, interpreted as slowing of measured neurological decline rather than restoration of function. The clinical conversation between the patient, family, and treating neurologist is essential before initiation.

How does diaspora-pooled funding work for Skyclarys refills? Pakistan's diaspora pattern is well-established. Many ultra-rare disease families fund chronic specialty care by pooling resources across overseas relatives in Saudi Arabia, the UAE, the United Kingdom, the United States, Canada, and Australia. Reserve Meds quotes in USD and accepts wire transfers from any USD-accessible source. The pricing transparency on our site lets families plan funding before contacting us.

9. Where Reserve Meds fits in Skyclarys cases

Reserve Meds is a United States-based concierge coordinator. We do not replace your treating neurologist, we do not replace DRAP, we do not replace your dispensing hospital pharmacy or the in-country importer. What we do for a Skyclarys case is orchestrate the United States-side sourcing through a partner with procurement access to the exclusive Biologics by McKesson specialty pharmacy channel, the regulatory documentation kit for the DRAP Personal Use Import application, the international logistics for a room-temperature oral capsule through bonded air freight, the FBR Customs coordination at the port of entry, and a single named coordinator who stays with your family across refill cycles. Because Friedreich's ataxia is a progressive lifelong genetic disease and Skyclarys therapy is chronic, Reserve Meds plans the relationship as a multi-year coordination rather than a one-time procurement.

No prior Reserve Meds closed-case experience for Skyclarys at this module date. Standard NPP coordination applies. Coordination is straightforward from a logistics standpoint and document-heavy on the regulatory side, particularly the FA genetic confirmation step.

10. Next step

If you or a family member has genetically confirmed Friedreich's ataxia and your treating neurologist at AKUH, Shifa, CMH, Liaquat National, or another tertiary center is considering Skyclarys, the next step is to join the Reserve Meds waitlist. We confirm eligibility within 24 to 48 hours and send the documentation kit to your physician and hospital pharmacy.

Reserved for you.