

Skyclarys

United Kingdom · access guide

Skyclarys access in the United Kingdom: the Specials Licence pathway

Last reviewed 2026-05-16 by Reserve Meds clinical and regulatory team.

Quick orientation

Skyclarys (omaveloxolone) is Biogen's once-daily oral capsule and the first and only disease-modifying therapy ever approved anywhere in the world for Friedreich's ataxia. The US FDA granted approval in February 2023 for adults and adolescents aged 16 years and older. The European Medicines Agency granted conditional marketing authorisation in February 2024; the MHRA granted UK conditional marketing authorisation via reliance routes thereafter. NICE technology appraisal is in progress, and NHS England commissioning depends on the eventual NICE recommendation. The drug is a Nrf2 activator with the proposed mechanism of reducing oxidative stress in the mitochondrial dysfunction characteristic of Friedreich's ataxia.

UK patients with Friedreich's ataxia have one of the most concentrated specialist networks in the world (the Ataxia UK clinical centres at UCLH, Sheffield, Oxford, Newcastle, Cambridge, and a growing regional footprint). The access gap for Skyclarys is the NICE-NHS commissioning timeline. Reserve Meds coordinates US-side sourcing for UK patients pursuing private supply through the Specials Licence pathway in advance of NHS commissioning.

Why UK Friedreich's ataxia patients pursue Skyclarys

Friedreich's ataxia is an autosomal recessive disorder affecting approximately 1,500 people in the UK. It causes progressive ataxia, cardiomyopathy, diabetes risk, and a range of other systemic features, with onset typically in childhood or adolescence. Until Skyclarys's approval there was no disease-modifying therapy; the standard of care has been symptomatic management, cardiac monitoring, and rehabilitation. Skyclarys's MOXIe registration trial demonstrated a statistically significant improvement on the modified Friedreich Ataxia Rating Scale (mFARS) versus placebo over 48 weeks, with sustained benefit in the open-label extension. The clinical community views Skyclarys as the first foundational disease-modifying therapy for this devastating disorder.

UK patients pursue Skyclarys when NHS commissioning has not yet completed, when they sit just outside the eventual NICE eligibility criteria, or when they want immediate access in advance of the NHS funding decision. UK private medical insurance does not typically fund Skyclarys.

The Specials Licence pathway for Skyclarys in the UK

Skyclarys holds UK conditional marketing authorisation; supply through UK-licensed specialty pharmacy distribution against a private prescription from a UK consultant neurologist is the principal private route. Where the patient is using the medicine outside the licensed indication boundary or where supply is not available through the UK distribution chain, the Specials Licence framework supports a private prescription dispensed through a UK Specials Licence holder with import authorisation.

The clinical safety profile of Skyclarys requires baseline and ongoing monitoring including liver function panel (ALT, AST), B-type natriuretic peptide for the cardiomyopathy component, lipid panel, and a documented assessment for any clinically significant cardiac arrhythmias before initiation. The dosing is 150 mg once daily orally, taken on an empty stomach in the morning.

Where Skyclarys is dispensed in the UK

The UK Ataxia UK clinical network of specialist neurology centres delivers Friedreich's ataxia care. The principal centres are the National Hospital for Neurology and Neurosurgery at UCLH London, Sheffield Teaching Hospitals (the Sheffield Institute for Translational Neuroscience), the John Radcliffe Hospital Oxford, Newcastle upon Tyne Hospitals, Addenbrooke's Hospital Cambridge, and a growing list of regional centres. These centres host the Skyclarys prescribing consultants for NHS-funded patients once commissioning is in place.

For private supply, UK-licensed specialty pharmacies dispense Skyclarys against a private prescription. Sciensus, Lloyds Clinical Homecare, and several London-based private specialty pharmacies handle rare disease oral medicines. Reserve Meds works alongside the chosen UK pharmacy to support US-side sourcing where required for the Specials route, and provides documentation continuity for the patient's clinical team.

Real cost picture for Skyclarys in the UK

Skyclarys's US WAC is approximately USD 370,000 per year at the standard 150 mg daily dose. At 0.79 GBP to 1 USD the US WAC equivalent converts to approximately GBP 292,000 per year for the medicine alone, before UK supply and fees. NHS contracted pricing under any eventual NICE-recommended commissioning agreement would reflect a confidential commercial discount.

For UK private supply through a UK-licensed specialty pharmacy the price typically lands somewhat below US WAC equivalent depending on supplier and quantity. UK private medical insurance does not typically fund Skyclarys on standard policies; cover requires specialty add-on benefits and is rare in practice. Self-funding is the principal route for UK private patients in advance of NHS commissioning.

Typical timeline for Skyclarys in the UK

For an NHS-routed case the timeline depends on NICE technology appraisal completion and NHS England Specialised Commissioning rollout. Until commissioning is in place, NHS funding is not available, and the route is private supply. For a private Reserve Meds Specials case the documentation and US-side sourcing completes within 2 to 3 weeks; the dispensing pharmacy in the UK then begins supply, with monthly dispensing thereafter. The medicine is a continuous oral therapy until intolerance or clinical decision to discontinue.

What your UK neurology consultant needs to provide

The treating UK consultant neurologist with ataxia subspecialty experience is the prescribing physician of record. The clinical packet typically includes the Friedreich's ataxia genetic confirmation (FXN gene GAA repeat expansion analysis), the patient's age (the licensed indication is 16 years and older), the modified Friedreich Ataxia Rating Scale (mFARS) baseline score, current cardiomyopathy assessment (echocardiogram with ejection fraction, ECG, BNP), liver function panel, lipid panel, glucose and HbA1c, and any concomitant medication review (particularly CYP3A4 substrates and inducers given the drug-drug interaction profile).

The MHRA Yellow Card scheme is the active pharmacovigilance route for any suspected adverse drug reaction, including transaminase elevations, BNP elevations, weight changes, and any cardiac symptom developments.

Common questions about Skylarys in the UK

Will the NHS fund Skylarys? NICE technology appraisal is in progress as of mid-2026. NHS England commissioning depends on the eventual NICE recommendation. The clinical community expectation is for a positive recommendation given the unmet need, although the cost-effectiveness analysis is the critical step.

What is the age range for Skylarys? The MHRA and FDA labels cover patients aged 16 years and older with genetically confirmed Friedreich's ataxia. Paediatric data are being developed but the medicine is not currently licensed for under-16s.

Will my UK private medical insurance cover Skylarys? UK private medical insurance does not typically cover Skylarys on standard policies. Specialty add-on benefits may consider case-by-case funding but this is rare in practice. Self-funding is the principal private route in advance of NHS commissioning.

What about the cardiomyopathy in Friedreich's ataxia? Cardiomyopathy is a feature of Friedreich's ataxia and a major cause of morbidity. Skylarys is associated with elevations in B-type natriuretic peptide (BNP), which can complicate the clinical picture. The patient's cardiologist should be involved in monitoring and the BNP elevation needs to be distinguished from a worsening of underlying cardiomyopathy.

What about diet and food interactions? Skylarys is taken on an empty stomach in the morning, at least 1 hour before food and at least 2 hours after eating. High-fat meals materially affect absorption. Patients should be counselled on this dosing requirement.

What about drug-drug interactions? Omaveloxolone is metabolised through CYP3A4 and is a CYP3A4 substrate. Strong CYP3A4 inhibitors (certain antifungals, certain antiretrovirals, certain calcium channel blockers) and strong inducers (rifampicin, certain anticonvulsants) require dose adjustment or avoidance. The treating consultant performs a full medication review.

Where Reserve Meds fits in Skylarys cases

Reserve Meds is a US-based concierge coordinator. For Skylarys our role is most relevant during the pre-NHS-commissioning window when UK patients pursue private supply through the Specials Licence pathway, for cross-market continuity of care for international patients, and for documentation support across the multi-month therapy initiation and monitoring schedule. We do not replace your UK consultant neurologist or the UK Ataxia clinical network.

The Yellow Card pharmacovigilance pathway for Skylarys

The MHRA Yellow Card scheme is the UK's national pharmacovigilance reporting system. Healthcare professionals, patients, and carers can report suspected adverse drug reactions, medical device incidents, defective medicines, and counterfeit medicines through the scheme. For specialty medicines accessed through the Specials Licence pathway, Yellow Card reporting is the operational mechanism that connects the UK clinical experience back to the global pharmacovigilance dataset that the MHRA, FDA, EMA, and other regulators rely on.

For Skylarys specifically, Reserve Meds coordinates the pharmacovigilance reporting chain in three ways. First, the prescribing UK consultant or the dispensing pharmacy submits any suspected adverse reactions through the Yellow Card scheme as standard practice. Second, the manufacturer's UK pharmacovigilance contact receives the case report through the standard regulatory channel and connects the case to the global safety database. Third, where the patient's clinical follow-up continues across markets, Reserve Meds provides the documentation continuity that lets the patient's consultants and the manufacturer's safety team coordinate across borders.

The MHRA also operates the Black Triangle (inverted black triangle) safety monitoring scheme for medicines that are under additional monitoring (typically newer medicines or medicines for which additional safety data are being collected). The Yellow Card scheme works the same way for Black Triangle medicines but with heightened attention to reporting.

UK consumer protection and patient rights for Skylarys

UK patients accessing specialty medicines through private pharmacy supply have the same consumer protections that apply to any UK regulated medicine purchase. The Care Quality Commission regulates private healthcare providers in England; Healthcare Improvement Scotland, Healthcare Inspectorate Wales, and the Regulation and Quality Improvement Authority in Northern Ireland are the parallel regulators in the devolved nations. The General Pharmaceutical Council regulates pharmacy professionals and registered pharmacy premises. The General Medical Council regulates doctors. The Nursing and Midwifery Council regulates nurses, including specialist nurses involved in cell therapy and homecare administration.

For UK patients accessing Skylarys, the relevant protections include the prescribing consultant's professional duty under GMC Good Medical Practice, the dispensing pharmacist's professional standards under General Pharmaceutical Council standards, the homecare provider's regulatory framework (where applicable), and the manufacturer's UK pharmacovigilance obligations. Reserve Meds operates as a US-based coordinator and is subject to US regulatory frameworks for our US-side operations; we do not replace or substitute for UK consumer protections, which the UK clinical and pharmacy chain provides directly.

Special considerations for international UK residents and dual-citizen families

The UK is home to a substantial population of international residents, dual-citizen families, and patients who spend significant time across multiple markets. For Skyclarys cross-border continuity of care across the UK, the United States, the Gulf, India, and other markets is a recurring operational pattern. Reserve Meds is structured to support this cross-market reality with a single coordinator who understands the regulatory frameworks across the relevant jurisdictions, the documentation portability across markets, and the operational connection back to the UK clinical team during periods of UK residence.

UK patients who spend time in the United States may also pursue treatment at a US authorised treatment centre when this is clinically or operationally preferable. Reserve Meds provides the US-side liaison, the documentation packet for the US treatment centre, and the operational support across the UK-US clinical handover both at the start and on return to the UK for long-term follow-up.

Where to read more about Skyclarys and the UK Specials pathway

Reserve Meds publishes detailed reference material across the regulatory pathways, country specifics, and condition-specific access guides. For the regulatory framework underlying the UK route to Skyclarys, the named-patient pathway overview covers the international framework and the United Kingdom country deep-dive covers the MHRA Specials Licence, NICE technology appraisal, NHS England Specialised Commissioning, and the dispensing infrastructure in detail. The MHRA's own guidance on the supply of unlicensed medicinal products (often called the MHRA Guidance Note 14) provides the formal regulatory framing for prescribers and pharmacists. The General Pharmaceutical Council's standards on the dispensing of unlicensed medicines provide the pharmacy practice framework.

For UK patient information on the NHS-funded pathway, the National Institute for Health and Care Excellence (NICE) publishes the relevant technology appraisal guidance, and NHS England Specialised Commissioning publishes the corresponding clinical commissioning policy. Patients can search the NICE website for the specific technology appraisal that applies to their medicine and indication.

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.

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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