Omaveloxolone and Friedreich's ataxia

This is a policy briefing note on the issue of access to the life-changing treatment omaveloxolone (Omav) for adults with Friedreich's ataxia (FA) in the UK produced by Ataxia UK, the national charity for all ataxias.

Need to know: Policy intersections

NICE thresholds: FA does not fit neatly into existing NICE evaluation routes. Although rare, it is not rare enough for the Highly Specialised Technology evaluation and is also not adequately served by the standard cost-effectiveness threshold.

UK Rare Disease Framework: The framework recognises that 3.5 million people in the UK live with a rare disease and commits to faster diagnosis and improved access to care.

Devolved nations: There are interim access routes to omaveloxolone in Scotland and Wales, meaning that Omav can be potentially accessed now. There is no such route in England or N.I. This creates inequalities in the UK.

Lack of prevalence data: The lack of reliable prevalence data for ataxias and rare diseases has been acknowledged by DHSC. This presents significant challenges in the national management of rare diseases.

Urgent issue affecting approx. 1,100 people across the UK:

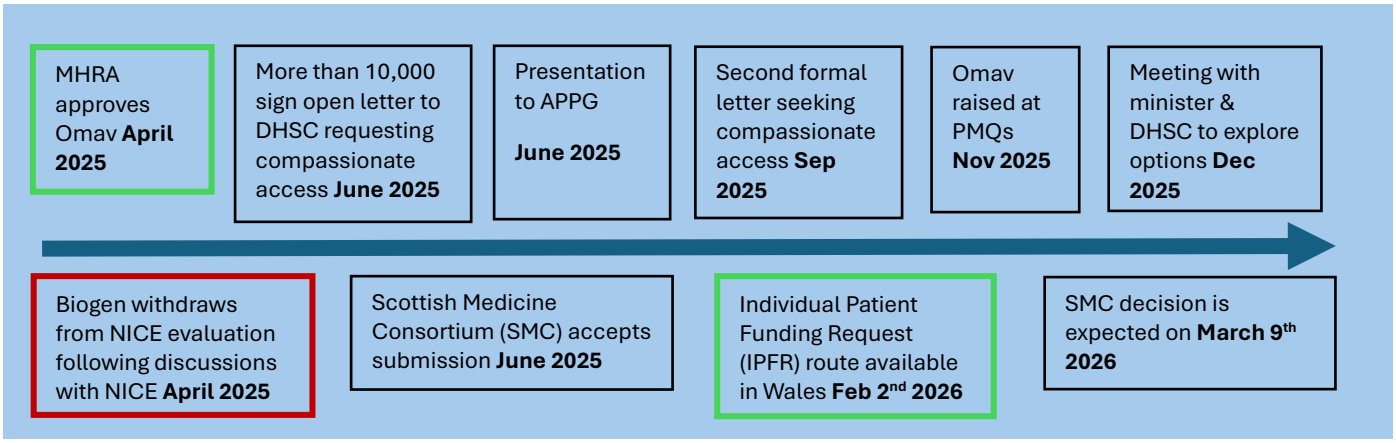
Friedreich's' Ataxia (FA) is estimated to affect around 1,100 people in the UK though due to the lack of reliable prevalence data, the true number is not clear.[1]

FA is progressive and life-limiting, with symptoms typically starting in adolescence and many individuals requiring a wheelchair within a decade.

Prior to omaveloxolone, there was no disease-modifying treatment. Extensive real-world evidence from people who have access to omaveloxolone across the globe has been overwhelmingly positive.

In April 2025, the UK drug regulator, the MHRA, approved omaveloxolone for the treatment of FA in those aged 16 and over in the UK, however since then the access to treatment remains an ongoing challenge.

Omav timelines in the UK



Friedreich's Ataxia (FA)

FA is a hereditary, neurodegenerative, severely disabling and life-limiting disease. It is the most common type of hereditary ataxia and is estimated to affect around 1,100 people in the UK – though due to the lack of reliable prevalence data, the true number is not clear.[1] **The average life expectancy for people with FA is only 37 years.**[2]

Symptoms of FA usually develop between the ages of 12 to 15 years old, and people generally become wheelchair-dependent within 10-15 years of symptom onset.

Early symptoms include poor co-ordination, balance problems when walking and slurred speech.[3] Along with a myriad of symptoms, FA can also cause diabetes, and a serious disease of the heart muscle (cardiomyopathy), which is the leading cause of death for people with FA.[4]

Omaveloxolone (Omav)/Skyclarys

Omaveloxolone is the **first and only** licensed treatment for FA globally and its regulatory approval marks a significant milestone, very warmly welcomed by the FA community worldwide.

It received regulatory approval for people with FA aged 16 and over by the FDA in February 2023, the EMA in February 2024 and the MHRA in April 2025.

Since EMA approval the drug has become available to patients in many European countries, through state reimbursement or early access programmes. Extensive real-world evidence from people who have access to omaveloxolone has been overwhelmingly positive. In clinical trials, Omav was shown to slow disease progression and significantly improve neurological function compared to those taking placebo.[5]

The drug impacts people's lives far beyond what can easily be understood by those not experiencing the devastating daily consequences of FA.

What is ataxia?[6,7,8]

Ataxia is an umbrella term for a group of rare neurological disorders that affect co-ordination, balance and speech. An estimated 10,500 people in the UK are living with ataxia – and in most cases there is no cure for the disease. Common symptoms include:

- Coordination and balance problems
- Slurred speech
- Difficulty swallowing
- Tremors or shaking
- Fatigue or tiredness
- Problems with vision

Most ataxias are progressive, meaning symptoms worsen over time, although the speed at which this happens will vary depending on the type of ataxia a person has. Likewise, the prognosis for people with ataxia varies considerably depending on the type of ataxia. For people with hereditary ataxia (where the faulty gene that causes ataxia has been passed on from parent to child), life expectancy is generally shorter than normal, although some people can live into their sixties and beyond. In more severe cases, the condition can be fatal in childhood or early adulthood.

- **Friedreich's ataxia is progressive and irreversible.**
- **Every moment of delay is hope lost for approximately 1,100 across the UK.**
- **Function lost during this period cannot be restored.**
- **The current situation, due to gaps in the medicines access process, is unfair for affected families.**
- **An interim access route to the treatment is the only acceptable option while Biogen re-engages with NICE and a more permanent solution is found.**

Devolved Nations

In Scotland, a pathway exists through which clinicians may submit individual requests seeking access to omaveloxolone with or without an appraisal decision. A route is also now available in Wales, where clinicians can make individual funding requests for access to omaveloxolone in the absence of an appraisal recommendation.

However, there is no interim access route for the FA community in England and N.I. This deviation demonstrates inequality within the UK for a life-limiting and irreversible condition. This undermines the principles of equity between the UK nations.

Recommendations:

1. The introduction of a new funded, temporary compassionate programme to help people with this devastating progressive condition. Patients are facing an unmet need for a treatment with supporting clinical evidence. Denying access is allowing the irreversible progress of the disease. This is extremely distressing for all affected by FA.

2. A variation in the criteria for the NICE assessment of medications for rare diseases like FA that are more common than the 300 patients required by the Highly Specialised Technology appraisal system, but less common than common conditions. This would support Priority 4 of the Government's Rare Disease Framework and recognise that rare conditions require particular consideration and cannot always be accommodated in mainstream arrangements.

3. NHS England to initiate discussions with the manufacturer (Biogen) to explore options for an interim access pathway for Omav in England for adults with FA while the company prepares to re-submit its proposal to NICE for appraisal.

4. Investigate and rectify the issue of lack of prevalence data for the ataxias.

5. Removing the barriers affecting the evaluation of medicines for rare diseases.

6. Continuous dialogue between NHS, DHSC, Ataxia UK, Biogen and all stakeholders.

Ataxia UK's Interim Access Campaign for FA treatment

Ataxia UK has led an evidence-based, and community backed campaign to secure interim equitable access to the only available treatment for Friedreich's ataxia.

Over 10,000 supporters signed our open letter to the Secretary of State for Health and Social Care to address this issue. As a result of our sustained campaign, the issue was also raised at Prime Minister's Questions as well as presented at the All Party Parliamentary Group (APPG) for Rare Disease.

We have engaged directly with NHS England, DHSC ministers, clinicians, researchers, and industry partners. On 10th December 2025, our delegation led by our Chief Executive along with Jonathan Brash MP met with the Parliamentary Under-Secretary of State for Health Innovation and Safety to press for interim access to Omav along with addressing structural barriers faced by the rare disease community in general.

Our campaign continues and we invite you to work with us to ensure that people living with FA can gain access to this life-changing treatment.

About Ataxia UK

Ataxia is the umbrella term for a group of neurological conditions and Ataxia UK is the UK's leading ataxia support and research-active charity. Our Services team is dedicated to supporting everyone affected by ataxia in the UK to live their best possible life. We offer a Helpline and Advocacy service that provides trusted information, tailored advice, and one-to-one support to help people access their rights. Alongside this, we deliver a range of activities, engagement opportunities, and volunteer-led initiatives designed to bring the ataxia community together and reduce feelings of isolation.

For policy enquiries, please contact Communications@ataxia.org.uk | ataxia.org.uk

Ataxia UK works across the whole of the UK and is a charity registered in Scotland (no SC040607) and in England and Wales (no 1102391) and a company limited by guarantee (4974832).

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[4] Tsou et al. *Mortality in Friedreich Ataxia*. Journal of the Neurological Sciences 2011. 307 (1-2) 46-49.
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[8] Ataxia UK, *Ataxia UK's Research Strategy for 2022 to 2025*, 2022. https://www.ataxia.org.uk/ataxia-research/for-researchers/our-research-strategy/ataxia-uk-2022-2025/