PRESS RELEASE

Ataxia UK Stresses Urgent Action on Friedreich's Ataxia Treatment Access

London, UK – Ataxia UK has once again appealed to the Department of Health and Social Care to take early action on access to omaveloxolone, the only approved treatment for Friedreich's ataxia (FA), among delays and emerging regional disparities across different parts of the UK relating to the availability of this life changing treatment.

In June, the charity wrote an open letter to the Secretary of State for Health and Social Care, Rt Hon Wes Streeting MP informing about the urgency of the issue and requesting interim access to omaveloxolone following its MHRA approval in April this year. The letter signed by more than 10,000 people called for compassion and consideration to put an interim system in place while NICE re-engagement or decision remains in waiting.

To further support the campaign, Sue Millman Ataxia UK CEO and Professor Paola Giunti, Head of the London Ataxia Centre presented a strong case at the All-Party Parliamentary Group (APPG) on Rare Disease, highlighting the pressing need to align omaveloxolone approval process with Priority 4 of the UK Rare Disease Framework which is precisely in place for improving access to specialist care, treatments and drugs for rare conditions such as FA.

On 8 July 2025, the Department of Health and Social Care (DHSC) responded to Ataxia UK that NHS England would not fund access to the treatment as the manufacturer has withdrawn from the NICE approval process because NICE indicated to them that they would not proceed the submission to the Committee phase, where the drug would be evaluated and a decision for reimbursement on the NHS would be made.

While Ataxia UK is already aware of this fact, the charity has warned that the lack of interim access during re-initiation of appraisal process is putting patients in a state of extended uncertainty and further irreversible deterioration of their condition.

It is to be noted that on 2 June 2025, following the company's submission for the evaluation of omaveloxolone by the Scottish Medicines Consortium (SMC), a pathway is now available for clinicians in Scotland to put forward a case for individual patients to gain early access to omaveloxolone. While this is welcomed by Ataxia UK, it also highlights growing inequity across the UK, as patients in England, Wales and Northern Ireland still do not have access to any similar potential solution. Furthermore, in many European countries early access schemes (with shared financing between the company and their national health services) are already in place, ensuring that many people with FA are benefiting from the drug.

“Families are now facing a harsh truth that access to treatment may depend on where they live,” said Sue Millman. “Even though omaveloxolone has been licensed by the MHRA and is available in other countries, access within the UK remains a distant challenge.”

Ataxia UK is submitting another formal letter to health authorities for a temporary, compassionate access programme for omaveloxolone aligned with the UK Rare Disease Framework and for a meeting with DHSC, clinicians and FA community to explore a mutually agreed way forward.

“We are not seeking to bypass the system but rather looking for a temporary, equitable arrangement at the moment before a permanent solution is found” Millman added. “Time is not on the side of FA patients, friends, and their families, so we must act now.”

Ataxia UK remains committed to this campaign and to our collaborative work with the ataxia community, patient advocates, DHSC, and the pharmaceutical sector to find a timely and equitable solution for everyone living with FA in the UK.