Rt Hon Wes Streeting MP
Secretary of State for Health and Social Care
Department of Health and Social Care
39 Victoria Street
London
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RE: Access to Omaveloxolone for People with Friedreich's Ataxia – Response to Correspondence Ref: DE-1614221

Dear Rt Hon Wes Streeting MP

Thank you for your response to our letter. We appreciate your engagement with, and understanding of, the challenges faced by those living with Friedreich's ataxia (FA), a rare, progressive, and devastating condition. I am writing to clarify and reiterate a few points raised in our recent correspondence and to highlight the urgency this issue demands.

While we fully recognise the constraints of NICE's approval process and NHS England's funding framework, it is precisely the gap left by these procedures particularly in rare diseases such as FA that requires empathetic and practical intervention. The community raising concerns through us is not seeking to bypass the process but is instead requesting a temporary, equitable solution aligned with Priority 4 of the UK Rare Disease Framework, which calls for faster, fairer access to treatments for rare conditions.

We also wish to highlight the growing disparities within the UK. On 2 June 2025, following the company's submission for evaluation of omaveloxolone by the Scottish Medicines Consortium (SMC), a pathway was created for clinicians in Scotland to put forward a case for individual patients to access omaveloxolone early. While we welcome this progress for Scottish patients, there is a growing sense of inequality among patients, families, and friends in England, Wales, and Northern Ireland, where no such mechanism exists.

I am sure you will understand that families are now facing a harsh truth: their future depends on where they live. Even though omaveloxolone has been licensed by the MHRA and is available in other countries, access within much of the UK remains a distant challenge. Every passing day represents an opportunity lost. In many European countries, early access schemes are in place often with shared financing between the company and national health services ensuring that many people with FA are already benefitting from the drug.

The termination of the NICE appraisal following Biogen's withdrawal has left patients in a dire situation. Without interim access, patients face preventable and irreversible worsening of their condition. To reiterate: there are no other disease-modifying treatments available, as this is the first licensed drug for FA anywhere in the world.

We therefore request:

- A meeting between the Department of Health and Social Care, Ataxia UK, clinical experts, and relevant stakeholders to identify a practical solution to address concerns and access gaps.
- That this issue is seen not as a technical matter but as one of saving lives slowing the progression of potentially irreversible damage through the introduction of a temporary, funded compassionate access programme for omaveloxolone, to provide immediate relief while long-term regulatory processes are resolved. Numerous shared funding models in other European countries could be adapted for the UK.
- That you consider the inequality within the UK in view of Scotland's early access programme and commit to addressing regional disparities in access to a medicine already approved by the MHRA. A just and compassionate solution should be provided for patients in England, Wales, and Northern Ireland. Interim access should be granted in line with Priority 4 of the UK Rare Disease Framework, which calls for faster and equitable access to specialist care, treatments, and drugs for such conditions.
- That your department seizes this opportunity to lead a compassionate and human-centred response to deliver an equitable solution for this community. Examples from other countries, particularly in Europe, and previous UK precedents demonstrate that interim access pathways are not only feasible but lifesaving.

We look forward to working together to find a solution through dialogue and collaboration. Time is not on the side of FA patients and their families, and we must act before more is lost.

Yours sincerely,

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