Rare Diseases Ireland
Carmichael House,
North Brunswick Street,
Dublin 7, Ireland
www.rdi.ie
advocacy@rdi.ie

24th August 2026

HSE Senior Leadership Team
Dr. Steevens' Hospital,
Dublin 8, D08 W2A8

Re: Skyclarys (omaveloxolone) – request for reimbursement and a more appropriate approach to reimbursement of rare disease medicines

Dear Members of the HSE Senior Leadership Team,

Rare Diseases Ireland is writing to ask you to reimburse Skyclarys (omaveloxolone) for eligible people living with Friedreich's ataxia through a managed access arrangement.

We recognise the importance of responsible stewardship of public resources and evidence-based decision-making. However, for rare disease medicines the system is ill-equipped to address the particular circumstances of rare disease medicines. The Skyclarys case highlights the challenges: small populations, limited clinical evidence, unavoidable uncertainty, high unmet medical need and irreversible disease progression. While regulatory authorities and European policy frameworks have evolved to accommodate these realities, our national reimbursement system has not yet caught up.

Importantly, this is not simply an issue for the HSE. The Government has already recognised the need for change. The Programme for Government 2025: Securing Ireland's Future commits to "Publish and fund a new National Rare Disease Plan, improving access to orphan drugs" and to "Investigate new methods for earlier reimbursement of certain treatments, including early access schemes for rare diseases." Furthermore, the National Rare Disease Strategy 2025-2030 subsequently recommends maximising access and availability to treatments and technologies for people living with a rare disease by 1) earlier reimbursement, 2) early access schemes for rare disease medicines, and 3) enhancing Ireland's engagement with international initiatives and HTA collaboration. The Skyclarys decision before you provides you with an opportunity to implement rather than simply acknowledge these commitments.

The National Centre for Pharmacoeconomics (NCPE) assessment identifies significant uncertainty around Skyclarys, including the magnitude and clinical significance of the treatment effect and the longer-term evidence. We accept that uncertainty exists. However, uncertainty is an inherent characteristic of evidence generation in rare diseases. Small patient populations and limited natural-history data make it difficult to generate the same volume and maturity of evidence that can be obtained for common conditions.

The policy question should not simply be: *Is there sufficient certainty to meet conventional HTA requirements?* It should also be: *How can the health system responsibly manage unavoidable uncertainty while ensuring that patients do not lose access to potentially beneficial treatment during the period in which further evidence is generated?*

A binary decision today between full reimbursement and no reimbursement is poorly suited to this situation with Skyclarys, and more generally to reimbursement assessment of rare disease medicines.

The HSE should be able to use mechanisms such as managed access, conditional reimbursement, evidence generation, national registries, specialist prescribing, outcomes monitoring, negotiated pricing and defined reassessment points to manage this uncertainty. This would allow the HSE to protect public resources while also protecting patients from the irreversible consequences of waiting for more evidence to be generated, evidence that may never reach the level possible in larger populations. In the case of Skyclarys, patients have now been waiting for more than 30 months since EMA/European authorisation. How much longer must they wait?

If conventional cost-effectiveness analysis systematically favours interventions serving larger populations with greater certainty of evidence, while rare disease medicines face greater uncertainty precisely because their populations are small, rarity itself becomes a structural disadvantage in access to innovation.

We recognise the legitimate principle that finite healthcare resources should be used to maximise health benefit across the population. However, that cannot be the only consideration. Friedreich's ataxia is a severe, progressive and debilitating rare disease. This creates exceptionally high unmet medical need. Skyclarys is the first licensed pharmacological treatment for the condition, and there are no equivalent disease-modifying alternatives.

High unmet medical need must be an explicit and meaningful consideration in reimbursement decisions. This is particularly important where delaying treatment may result in irreversible loss of function that cannot subsequently be recovered.

We believe there is a need for greater transparency around the Drugs Group deliberations. Professor Michael Barry, clinical director at NCPE, has publicly explained his understanding of the rationale for the Drugs Group's recommendation not to reimburse Skyclarys in recent RTÉ and Newstalk interviews. However, we have not heard directly from any official Drugs Group spokesperson on their deliberations or from HSE's Corporate Pharmaceutical Unit.

Professor Barry's contribution provides valuable insight into the economic and HTA perspective of deliberations. However, there is no equivalent public account from either the prescribing clinical or patient perspective, as neither group is invited to attend Drugs Group meetings. This raises a legitimate governance concern about both balance and transparency.

Professor Barry participates in the Drugs Group deliberative process as a non-voting attendee. Patient representatives and prescribing clinicians do not, however, have an equivalent opportunity to participate in the meetings meaning they cannot respond to the arguments being considered or questions being asked. We believe the HSE should ensure that different forms of evidence and expertise have an appropriately balanced opportunity to contribute to Drugs Group deliberations.

Health Technology Assessment should inform the reimbursement decision, not become the reimbursement decision. It provides important evidence and should form a central part of the decision-making process. However, the final reimbursement decision rests with the HSE and wider statutory considerations must be taken into account. The HSE must therefore consider the economic assessment alongside:

- Severity and progression of disease;
- High unmet medical need;
- Absence of alternative treatment;
- Patient and clinical evidence;
- Uncertainty in the evidence base;
- Consequences of delayed treatment; and
- Potential for managed access and further evidence generation.

We would welcome greater transparency regarding how Drugs Group deliberations account for clinical evidence, patient evidence, unmet medical need and the other statutory criteria. When Skyclarys evidence was considered, what was the relative weight given to the NCPE assessment versus other contributions and criteria?

In light of these considerations, we ask the HSE to reimburse Skyclarys. We believe the appropriate response to the current evidence is not to deny access, but to manage the uncertainty. We therefore ask the HSE Senior Leadership Team to:

1. Reconsider the recommendation not to reimburse Skyclarys and reimburse Skyclarys through a managed access arrangement for eligible people with Friedreich's ataxia;
2. Negotiate an appropriate price and/or risk-sharing arrangement with the manufacturer;
3. Establish managed access with appropriate clinical eligibility and specialist prescribing;
4. Establish structured collection of clinical, functional and patient-reported outcomes;
5. Commit to formal reassessment following an agreed period of evidence generation; and
6. Use the Skyclarys case to implement the Government's commitment to earlier reimbursement and early access schemes for rare diseases, as set out in the Programme for Government and the National Rare Disease Strategy 2025–2030.

The decision on Skyclarys is about more than one medicine. It is a test of whether Ireland's health system can translate its stated commitment to rare diseases into a reimbursement system capable of responding to the realities of rare disease evidence and high unmet medical need. Skyclarys provides an immediate opportunity to turn commitments made in the Programme for Government and the National Rare Disease Strategy into action.

We therefore urge the Senior Leadership Team to reimburse Skyclarys through a managed access arrangement, and to use the Skyclarys case to implement the Government's commitment to earlier reimbursement and early access schemes for rare diseases, and to begin developing a sustainable and consistent rare disease medicines pathway for the future.

For patients with Friedreich's ataxia, uncertainty can be managed. Evidence can be generated. Prices can be negotiated. What cannot be recovered is time lost to irreversible disease progression.

Yours sincerely,

Chief Executive Officer, Rare Diseases Ireland

Rare Diseases Ireland Board of Directors*

Anne Micks

Avril Daly

Darran Brennan

Eamonn O'Neill

Fergal Griffin

Laura Egan

Mairead Hennessy

Susanne O'Reilly

*This letter reflects our personal views and opinions only. They are provided in our personal capacity as directors of RDI and should not be interpreted as representing the views, opinions or position of their employers or any affiliated entity.