Department of Health & Social Care

From Karin Smyth MP
Minister of State for Health

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Freddie van Mierlo MP
By email to: freddie.vanmierlo.mp@parliament.uk

18 August 2025

Dear Freddie,

Thank you for your correspondence of 27 June, co-signed by members of the All-Party Parliamentary Group for Access to Medicines and Medical Devices, about access to givinostat for Duchenne muscular dystrophy (DMD) and the Early Access Programme (EAP). I apologise for the delay in replying.

I appreciate your concerns. The Government is aware of the impact that DMD has on those affected, and how important it is for them to have access to the most effective treatments.

The National Institute for Health and Care Excellence (NICE) is the independent body responsible for developing authoritative, evidence-based recommendations for the NHS on whether new medicines represent a clinically and cost-effective use of resources.

As you are aware, the manufacturer, Italfarmaco, has opened an EAP for givinostat, which NHS trusts can participate in before a NICE decision. It may be helpful to note that the company only provides the drug itself. The clinical staff and other service costs required to safely administer the medicine would need to be found by participating NHS trusts in addition to their planned resources and budget. Any participating NHS trust would also need to have arrangements to ensure continued access to the treatment, in the event that NICE is unable to recommend givinostat to be commissioned by the NHS.

NHS England has published guidance on free-of-charge medicines schemes, such as the EAP for givinostat, providing advice on potential financial, administrative and clinical risks. However, NHS England cannot compel NHS trusts to participate in company-sponsored EAPs.

I hope you will appreciate that if new funds were to be made available to fund the delivery of one company-sponsored EAP, such as the one for givinostat, they would then need to be made available for all such programmes. The total costs for every provider to do this would be substantial. It is also important to note that making funds available for treatments that have not yet demonstrated clinical and cost-effectiveness would only be possible by diverting money from other established NHS treatments and services with proven clinical and cost-effectiveness.

We appreciate how difficult it is for families while they await a NICE decision. I want to assure you that, if recommended by NICE in draft guidance, NHS England will work with Italfarmaco with the aim of providing early funding through the Innovative Medicines Fund, speeding up access by up to five months.

The *UK Rare Diseases Framework* was published in 2021 following the National Conversation on Rare Diseases, which received nearly 6,300 responses. This helped identify the four priorities of the Framework in tackling rare diseases:

- helping patients get a faster final diagnosis;
- increasing awareness of rare diseases among healthcare professionals;
- better coordination of care; and
- improving access to specialist care, treatment and drugs.

The Government has confirmed its continued commitment to the four priorities and published a new action plan in February. Four action plans have now been published that set out England's approach to addressing the priorities of the Framework. To ensure delivery and accountability, each action lists an owner, the desired outcomes and how we will measure and report progress. Further information can be found at www.gov.uk/government/publications/england-rare-diseases-action-plan-2025.

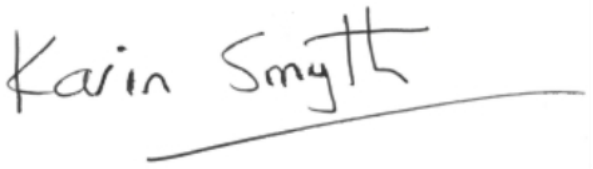
The Framework also sets out improving access to specialist care, treatments and drugs as a priority, and health equity as a cross-cutting theme.

We are also working to review the effectiveness of access schemes for rare disease therapies, which are designed to support access to innovative treatments for patients who need them earlier. The review will broadly consider access to rare disease therapies, with a focus on the Early Access to Medicines Scheme, the Innovative Licensing and Access Pathway, and the Innovative Medicines Fund.

NHS England, NICE and the Medicines and Healthcare products Regulatory Agency met most recently in July, and they will meet annually to continue discussing progress. These meetings include representatives from patient advocacy groups, industry and clinical researchers.

I hope this reply is helpful. I would be grateful if you could share it with your co-signatories.

Yours sincerely,



KARIN SMYTH