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Lord Sharkey

House of Lords

SWIA 0AA

28 August 2025

Dear Lord Sharkey,

Lords Oral Question – Duchenne Muscular Dystrophy: Givinostat – 22 July

Thank you for your contributions to the debate concerning the availability of givinostat for boys living with Duchenne muscular dystrophy (DMD) on 22 July, particularly in your capacity as a trustee of Muscular Dystrophy UK. I want to reiterate that the Government understands that there is a huge unmet need for patients with Duchenne muscular dystrophy and how important it is to families that they are able to benefit from rapid access to effective new treatments.

I am sorry I was unable to respond to all of your questions during the debate, but I hope the following will provide clarity on the points you raised regarding the success of early access programmes and access to givinostat.

Firstly, I think it would be helpful if I provided some wider context regarding the various routes to early access for medicines. Companies may put in place early access programmes (EAPs) to allow early access to new medicines before they are routinely available to patients, such as the one for givinostat. Under these programmes, the cost of the drug is free to both patients taking part in it, and to the NHS, but NHS trusts must normally still cover what may be substantial costs of administering the medicine and associated monitoring and follow up. As such, participation is decided at an individual trust level who would be required to find funding outside of their allocation. 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.
NHS England cannot, however, centrally direct NHS trusts to participate in company sponsored EAPs like this, or on any other private activity. If new money were to be made available to fund the delivery of one company sponsored early access programme, such as givinostat, it would then be the case that all such programmes required funding for the clinical administration costs of all drugs offered through company-sponsored early access. The total costs for every provider to do this would be substantial, and 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.

Outside of company sponsored EAPs, there is an established route for patients to get access to new, innovative medicines, prior to them being licensed. The MHRA's Early Access to Medicines Scheme (EAMS) has been running since 2014 and is one of the UK's offerings for Early Access Programmes (EAPs), through which companies have a framework for providing unlicensed medicines to patients. EAMS is designed to give patients with life threatening or seriously debilitating conditions access to medicinal products that may be used for preventing, diagnosing or treating those conditions but which are either not authorised or not authorised for that use. EAMS is supported by key partners including the MHRA, NICE and NHS England, and is a key part of this Government's commitment to accelerate patient access to innovative, life changing treatments and support the UK's position as a global leader in life sciences.
To date, EAMS has received 67 full applications across 50 different medicines. Of the 67 applications, 51 have received a positive scientific opinion from the MHRA. There have been 33 full licences, of which NICE was able to recommend 30 medicines across 35 indications for routine use in the NHS. NICE was unable to recommend 1 of the medicines within its licensed indication and 1 medicine was not selected for evaluation as there was an existing NHS England clinical commissioning policy for its use. The appraisal for the 1 remaining licensed medicine is currently in progress.

As you are aware, NICE is currently appraising givinostat for treating Duchenne muscular dystrophy in people 6 years and over. The NICE process ensures that funding for new medicines does not displace funding for other cost-effective treatments and services that are so important to patients. It has enabled many thousands of patients to benefit from access to treatments in a way that represents value to the taxpayer, including recently the drug vamorolone which was recommended by NICE for children with Duchenne muscular dystrophy in January this year.

If recommended by NICE in draft guidance, the Secretary of State has been clear that NHS England should aim to work with the pharmaceutical company to provide early interim funding for givinostat through the Innovative Medicines Fund (IMF), which has made available £340 million of ringfenced funding for the NHS to fund early access to medicines. This could potentially speed up access to givinostat by up to 5 months.

This Government is committed to ensuring that we do provide access to the most innovative medicines for rare diseases and we are working hard with industry, NHS England, NICE and MHRA to make this happen. England's 2025 Rare Diseases Action Plan was published in February 2025 and reports on a number of actions aimed at furthering the commitment to improve access to specialist care, treatment and drugs, including a review of the effectiveness of the Early Access to Medicines Scheme (EAMS), the Innovative Licensing and Access Pathway (ILAP) and the Innovative Medicines Fund (IMF), access pathways across the regulatory and access system designed to support innovative treatments being available to patients who need them earlier, in supporting access to treatments for people living with rare diseases.

Thank you for taking the time to engage on these matters. I hope these further details have been useful and I will place a copy in the House libraries.

Yours sincerely,

BARONESS BLAKE OF LEEDS, CBE