

Duchenne UK
Unit G24, The Shepherds Building
Charecroft Way, Hammersmith
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19 November 2024

Dear CEO,

We are writing to you from Duchenne UK and Muscular Dystrophy UK, two patient charities focused on a rare muscle wasting disease called Duchenne muscular dystrophy (DMD), to urge you to support your local neuromuscular team in utilising an Early Access Programme to give patients access to a promising DMD medicine called givinostat.

DMD is a rare, progressive, and cruel muscle wasting condition, which almost exclusively affects males. There is no cure, and there is a huge unmet need. It is the most severe form of muscular dystrophy and a DMD diagnosis, usually made around the age of four, is devastating to families. Parents learn that their toddler will lose the ability to walk by their early teens, that they will need full-time mechanical ventilation by their late teens, and that very few people with DMD live past 30.

Your Trust is fortunate to have a clinician who is part of the NorthStar clinical network, a network of doctors in Britain who are internationally recognised as experts in DMD. NorthStar clinicians are behind some of the most pivotal DMD clinical trials, and produced a measurement called the NorthStar Ambulatory Assessment (NSAA) which is used globally by health regulators such as the United States Food and Drug Administration (as well as NICE here in the UK) when accessing the efficacy of DMD drugs.

There is no effective treatment for DMD. Corticosteroids, which are routinely prescribed for DMD, only treat the symptoms and come with a raft of adverse side effects.

A medicine which could preserve muscle function and prolong ambulation would be transformational for our community, and there is a medicine called givinostat which could be the first. It is already approved in the US and the MHRA is currently reviewing an application for a UK licence through the International Recognition Procedure, with a decision expected in early 2025. The SMC are assessing givinostat, and NICE's appraisal process has already begun with an Appraisal Committee meeting scheduled for May 2025.

The company behind givinostat, ITF Pharma UK, is providing givinostat for free to the NHS through a compassionate use scheme they are calling the Early Access Programme (EAP). We are urging every DMD clinician to apply for access to givinostat for all patients who are eligible, and who they judge will benefit

medically. We also predict that patient demand for this medicine will be very large.

We are writing to you to make you aware of the scheme, and to urge you to ensure your local clinicians have the resources and support they need to access it. Most adults with DMD, as they are not ambulant, will not be eligible for the EAP. But we expect parents of children with DMD will be enthusiastic in their desire to secure givinostat for their child, and to help prolong the amount of their life they spend without a wheelchair.

As we are sure you will appreciate, with a progressive and devastating condition like DMD any delay in accessing a new treatment would be deeply distressing. Any function lost during that delay will not be regained – and could even result in children currently eligible to receive givinostat not being so by the time it is licensed by the MHRA and recommended by NICE and the SMC. If eligible patients are not able to access the EAP following a request from their DMD clinician there is likely to be a considerable reaction from the community.

Everyone knows that the NHS is stretched thin, but ours is a very small and very desperate community who urgently need access to this drug. For someone with DMD, time is muscle. Muscle they will never get back. Please will you ensure your local neuromuscular team has the resources they need to take part in the EAP.

If you need any further information, or if we can be of assistance, please contact us at will@duchenneuk.org.

Yours sincerely,



Emily Reuben OBE

CEO and co-founder, Duchenne UK



Alex Johnson OBE

Co-founder, Duchenne UK



Andrew Fletcher

CEO, Muscular Dystrophy UK