

BRIEFING NOTE

For Elected Representatives

Givinostat - Duchenne Muscular Dystrophy (DVD) Treatment

Changes in how Belfast Trust may treat some cases of Duchenne Muscular Dystrophy (DMD) could prompt questions from you and your constituents.

We understand how this may be an anxious time for families of those impacted by DMD and we want to provide you with some answers to queries you may have and make clear the Trust's position on Givinostat.

Givinostat is a new treatment approved by the Medicines and Healthcare products Regulatory Agency (MHRA) for the treatment of DMD.

The Trust is now in a position to work on introducing an Early Access Programme (EAP) for Givinostat for some children who meet the criteria of being over six years old and ambulant.

This means that eligible patients can be offered the medication ahead of its potential approval by NICE (National Institute for Health and Care Excellence).

We know that families have been waiting a long time for this opportunity and we want to acknowledge how difficult and frustrating this wait has been.

Until recently, due to the impact of significant workplace shortages and the knock-on effect on available skills across a number of professions, we were unable to develop this.

Any new drug for prospective use within the Trust has to be approved by its New Drug Committee. This was granted on 1st October 2025, and the Trust aims to begin the EAP in mid-October.

It's hoped that all eligible patients (approximately 14-16 in total) will be seen within a fortnight.

If Givinostat is approved by NICE then the eligibility criteria for treatment will be reviewed.

We are currently contacting all patients who may be eligible for Givinostat. If your child may be eligible for the drug you should have received a letter.

Your Questions Answered

What is the Givinostat Early Access Programme?

Givinostat is a potential treatment for Duchenne Muscular Dystrophy (DMD) that has been developed by the pharmaceutical company ITF Pharma LTD. This drug showed promise in clinical trials and is awaiting a decision from the National Institute of Clinical Excellence (NICE) as to whether it will be made available to patients on the NHS.

In the meantime, ITF Pharma Ltd have made the drug available to eligible patients through an Early Access Programme.

Givinostat EAP at Royal Belfast Hospital for Sick Children

While the manufacturer has made the drug available to the Belfast Trust to give to eligible patients free of charge, there are significant additional costs to a hospital to enable us to safely administer the drug beyond the cost of the drug itself.

It has taken us some time to ensure we can cover these additional costs and have the staff we need in place to monitor the use of the drug in our patients. We understand how worrying this situation has been for patients and families and we are sorry for this.

We are pleased to confirm that we are now able to offer Givinostat to eligible patients in clinic at the Royal Belfast Hospital for Sick Children. Our clinical team is contacting all patients who may be eligible for the treatment to let them know it will be discussed in their next appointment.

We are working to see all potentially eligible patients (approximately 14-16) before 17th October. We are aiming to see around eight patients a week, which would mean they should be offered an appointment to be seen within a fortnight.

We are offering the drug to children who are over six years old and able to walk.

What is happening now?

Our clinical team is working through our patients and will be in touch with families directly when appointments are confirmed. The team is working incredibly hard to see patients as quickly as possible.

Which patients will be offered Givinostat?

The Early Access Programme for Givinostat is for ambulant patients only, and this means children who are able to walk or at least able to stand. In your child's clinic appointment a member of the clinical team will discuss the eligibility criteria with you and how this relates to your child.

I'm not sure if my child is eligible, what should I do?

Our clinical team has sent letters to all the eligible patients with whom we will be discussing the possibility of them receiving Givinostat in their next appointment.

I think my child might be eligible but I have not had a letter, what should I do?

We will be in contact with all patients who may be eligible and we expect them to have been contacted by Friday 3rd October. If, after this week, you think your child meets the eligibility requirements for Givinostat and you have not heard from our clinical team please contact our physiotherapist on 07385387090.

Givinostat requires regular blood tests for monitoring purposes, will we have to come to the Royal Belfast Hospital for Sick Children for these?

At present we do not have the capacity to facilitate these tests outside of the Royal Belfast Hospital for Sick Children (RBHSC), therefore all blood tests and ECGs will be performed in RBHSC.

What happens if NICE does not approve Givinostat for use in the NHS?

As part of the EAP the manufacturer has committed to continuing to supply the drug to patients who have already commenced treatment.

However, if NICE approval is not granted or if the current staff or funds are pulled, we will have to reconsider the working of the team and this will be discussed with you at the clinic appointment.

