Belfast Health and Social Care Trust
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FOI/33252

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Givinostat EAP

Under the Freedom of Information Act 2000, I would like to request the following information in relation to the Early Access Programme (EAP) for givinostat operated by ITF Pharma UK. Givinostat is a treatment for Duchenne muscular dystrophy (DMD) granted market authorisation by the MHRA in December 2024. The EAP is a free of charge scheme/compassionate use scheme which your organisation is eligible to take part in.

- **What assessment has your organisation made of participating in the EAP for givinostat?**
- **Does your organisation have plans to take part in the EAP for givinostat?**
  If not, what was the rationale for not taking part?
- **Has your organisation made an estimate of the cost of taking part in the EAP?**
  If so, what is that cost?
- **Has your organisation estimated how many patients may be eligible for the EAP?**
  If so, how many patients are currently eligible for the EAP?

The Royal Belfast Hospital for Sick Children provides comprehensive care for patients with DMD and are part of the NorthStar clinical network which consists of consultants, physiotherapists and other allied health professionals at 23 specialist paediatric tertiary centres across the UK. At the children's hospital the Neurologist along with geneticists makes a diagnosis and commence appropriate treatment and regularly review the patient. They can also take the opportunity to refer patients to trials in other UK centres if appropriate. A multi-disciplinary team approach is adopted for patients with DMD, and this includes neurology, cardiology and respiratory specialists as well as endocrine specialists. Physiotherapists are also involved with the patient's care.

Givinostat is a new treatment approved by the Medicines & Healthcare products Regulatory Agency (MHRA) for the treatment of Duchenne Muscular Dystrophy (DMD) in boys aged 6 and older. The National Institute for Health and Care Excellence (NICE) is currently developing recommendations for use of Givinostat within the NHS in England. Treatments that have been recommended by NICE for routine use in the NHS in England are typically also routinely available in Northern Ireland.

Belfast Trust is required to work within the process for commissioning of new medicines following a NICE recommendation for use.
The drug is available for an Early Access Programme (EAP) for eligible patients, pending decisions about wider access following NICE recommendations.

Givinostat treatment, requires close monitoring for each patient. This includes blood tests every 2 weeks for the first 8 weeks of treatment, followed by additional blood

tests at 12 weeks and every 3 months thereafter. These tests are used to look for the presence of significant side effects, such as low platelet count (which can lead to bleeding tendencies), high triglyceride levels in the blood, and gastrointestinal side effects like diarrhoea. If these tests show any abnormality, the test must be repeated within a week and the Givinostat dose may be adjusted.

It is important that there are sufficient resources in place to ensure smooth supply and good monitoring, that families provide informed consent that Givinostat is prescribed as necessary and that exit strategies are discussed; and subsequently to monitor blood test results every 2 weeks, supply necessary prescriptions and make changes to Givinostat treatment regimes in the event of adverse effects.
In addition, pharmacy resources are required to ensure the safe and appropriate monitoring of treatment, as well as the procurement, storage and dispensing of treatment continuing after commencement. Clinical centres also need to have phlebotomy services with sufficient capacity, a suitable physiotherapy service to objectively measure the functional effects of Givinostat treatment, and psychological services if there is needle phobia. At present, many neuromuscular teams have little spare capacity to undertake this work, meaning that solutions will need to be found if the programme is to be delivered fully.

Currently Belfast Trust is not in a position to proceed under the EAP as its implementation will need to be managed within defined and agreed protocols and additional staffing resources will also be required to ensure the treatment can be provided safely. We recognise this is very disappointing for families and the Belfast Trust sincerely apologises to them.

Should NICE recommend treatment with Givinostat, Belfast Trust will liaise with commissioning colleagues in the Department of Health's Strategic Performance & Planning Group (SPPG) to establish what is needed to allow the Trust to be in a position to support its delivery.

In an attempt to quantify how much resource would be required to start an EAP, the funding resources are outlined below based on 13 ambulant patients who currently meet the criteria for early access. However we are aware that the company may be willing to give Givinostat for all Duchenne patients above 6 years. There are 39 patients who meet this criteria. This would increase the costs below.

The NICE guidance when approved may include non-ambulant patients and a change in age profile, this will increase the patient cohort who are eligible for the treatment. Further outworking of pathways across specialties will need to considered, ensuring a full financial appraisal is completed.

Table 1: Resources required to provide a service for 13 ambulant patients through EAP

Nurse Specialist (Co-coordinator)	B7	1 WTE
Pharmacist	B8A	0.5 WTE
Physiotherapist	B8A	0.3 WTE

Phlebotomist	B3	0.5 WTE
Play Specialist	B4	0.5 WTE
Psychology	B7	0.2 WTE
Consultant Paediatrician (with specialist interest)		0.5 WTE
ECG Physiologist	B7	0.3 WTE
Admin	B4	0.25 WTE

The costs associated with the above resource requirements with a full year effect is £309,000.

However this information needs to be heavily caveated with the following points noted.

1. It is critical to note that this is not simply a matter of the financial resource availability. Even if money was allocated, there would be a significant lead in time to recruitment of the specialist staff required to start this service.
2. Recruitment to jobs that are lower than a WTE are often very hard to recruit to and require that the post is made fulltime to attract the correct staff.
3. There may be difficulty recruiting into the Neurology team for all posts and each post holder may need a period of upskilling or training.
4. These figures reflect the minimum resource requirement based on our current intelligence and the numbers within the ambulant population but would increase depending on the final NICE guidance.