

16 April 2024

The Treatment of Duchenne Muscular Dystrophy

I would like to request the following information in regard to the treatment of Duchenne muscular dystrophy (DMD) by your organisation:

Does your organisation commission any services for people with DMD?

If no, does your organisation expect to commission services for DMD in the future?

If yes, what services for people with DMD does your organisation commission?

Paediatric:

Paediatric Neurology Consultant with 0.2wte of time for all patients.

Paediatric Neuromuscular Physiotherapist one whole time equivalent.

Paediatric Neuromuscular Clinical Nurse, one whole time equivalent.

Paediatric Neuromuscular Respiratory Nurse.

Adult:

The Belfast Health and Social Care Trust does not commission services, this role is carried out by the SPPG (Strategic Planning and Performance Group). DMD patients are seen within dedicated Neuromuscular clinics.

How many people with DMD does your organisation serve?

Paediatric patients: 46 patients

Neurology adult services: There are 34 adult DMD patients (5 of which are manifesting carriers).

Does your organisation have any DMD-specific (or muscle-wasting disease-specific) policies or guidance?

Paediatric:

The Paediatric service strives to implement the “DMD Standards of Care International Recommendations Published in The Lancet Neurology in 2018”, within

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the available resources. Unfortunately as there is a small team with limited resources it is not always possible to meet timeframes outlined within the standards.

The Paediatric service are also part of the North Star Network and attend meetings either virtually or in person. They collect data and maintain the DMD database.

The service ensures that respiratory, cardiac and endocrine colleagues are all kept up to date, and they share the published articles and patient information booklets produced.

Adult:

There is no Belfast trust or NI guidance however, the Neuromuscular service try and follow the DMD standards of care documents. Similar with any of the conditions that have standards of care – eg Myotonic dystrophy and have benchmarked against Scotland regarding these. SMA patients are signed up to SMA Reach which are assessed accordingly as part of the risdiplam treatment and requirements of NICE. We do not input into the database.

Do any of your organisation's strategies, objectives, plans, performance indicators, audits, inspections, or similar, cite DMD (or any muscle-wasting disease) or use either as a metric?

Paediatric:

The Paediatric team are keen to complete the MDUK centre of excellence audit, but due to limited resources in the previous audit year this was not achievable.

The team have, however, completed local audits such as bone health and implemented change in accordance with finding such as emergency steroid training for parents.

The service also took part in Multicentre Study "A benchmarking audit of the pre-diagnosis pathway in patients with DMD". This was a UK wide audit and findings were published:- Gowda VL, et al. Arch Dis Child 2022;107:160–165.
doi:10.1136/archdischild-2020-321451.

Adult:

The Neuromuscular service have benchmarked against Scotland for myotonic dystrophy and audited same for the service. The Neuromuscular service input into the Adult Duchenne Northstar database and have also completed the MDUK Excellence award last year which is essentially a large audit of work carried out in terms of Muscle disease.

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**Is your organisation aware of the work of DMD Care UK
(<https://dmdcareuk.org/>)?**

Paediatric:

The Paediatric service are aware of the valuable work carried out by DMD Care UK. They also use the Duchenne UK family folder for all newly diagnosed patients in Northern Ireland.

Adult:

Yes, the Neuromuscular nurse is part of the working group on transition for the organisation.