Not all Europeans receive the same care for Duchenne muscular dystrophy

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Duchenne muscular dystrophy (DMD), a progressive muscle disease affecting one in 3800-6300 live male births and leads to ambulatory loss, respiratory problems, cardiomyopathy, and early death of patients in their 20s or 30s. While incurable, multidisciplinary treatment can raise life expectancy into the fourth decade. However, in a survey across seven European countries, researchers found striking differences in access to appropriate care. There were significant inequities between different countries and different age groups, which would likely lead to different health outcomes.

The CARE-NMD survey examined care practices and quality of life through DMD patient registries in Bulgaria, the Czech Republic, Denmark, Germany, Hungary, Poland, and the United Kingdom. A total of 1,062 valid responses were received, including 861 children and 201 adults. The average age of respondents was 13.0 years and 53% had lost the ability to walk at a median age of 10.3 years.

The standard of care for DMD patients typically includes corticosteroid medication, physiotherapy, regular echocardiograms, and regular check-ups (1-2 per year) at a neuromuscular center. Patients older than 6 years should also receive pulmonary function assessment regularly. The surveys showed adherence to guidelines was low in ambulatory and strikingly poor in non-ambulatory patients, with pronounced differences in particular between Eastern and Western European countries, but also within Western Europe.

According to lead investigator Janbernd Kirschner, MD, Department of Neuropediatrics and Muscle Disorders, Medical Center at the University of Freiburg, Germany, "Access to corticosteroid treatment, which has been shown to slow the progression of DMD, varied between countries and by age: only 21% of those who had lost the ability to walk remained on corticosteroids. Extraordinary differences in access to physiotherapy were observed by country (for example, more than 90% in Germany, and less than 50% in the UK), and adults were less likely to receive physiotherapy than children."

Access to regular heart and lung function tests also varied greatly. Nearly a quarter of patients, especially adults, did not receive regular echocardiograms. Adherence to pulmonary function assessment guidelines was low in patients who were still walking (62.8%) and very low (30.5%) among non-ambulatory patients. It also varied notably between countries for both groups. In the UK, for example, while two thirds of ambulatory patients received lung function testing according to guidelines, this dropped to less than half of non-ambulatory patients. Many patients reported being insufficiently informed about DMD-related breathing problems, with only 31.3% of ambulatory and 39.9% of non-ambulatory patients knowing their current lung capacity.

The researchers also found that patients who visited specialized neuromuscular centers at least once annually tended to receive more frequent heart and lung checks as well as corticosteroid medication, to be better informed about their condition, and to be more satisfied with their treatment. Care from a neuromuscular specialist also reduced the duration of stay during unplanned admissions to hospitals.

Dr. Kirschner and his co-investigators emphasize that access to harmonized, best-practice care in line with international recommendations is not only associated with improved life expectancy and quality of life for those living with DMD, but it is also critically

important to the increasing number of clinical trials currently underway testing treatments with a more curative approach. "Standardized care allows researchers to be confident that any improvements seen in a trial are due to the drug being tested, rather than differences in care received by patients," noted Dr. Kirschner.

More information: Julia Vry et al, European Cross-Sectional Survey of Current Care Practices for Duchenne Muscular Dystrophy Reveals Regional and Age-Dependent Differences, *Journal of Neuromuscular Diseases* (2016). DOI: 10.3233/JND-160185

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