Health related quality of life in European adults with DMD – results from the Care-NMD-project

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INTRODUCTION

CARE-NMD is a 3-year multi-centre study (2010-2013) to implement best-practice standards of care for Duchenne muscular dystrophy (DMD) across Europe. Care Centres in seven European countries (Bulgaria, the Czech Republic, Hungary, Poland, Germany, the United Kingdom and Denmark) formed a network to evaluate existing treatment practices, assess Care and the subjective perception of Health-related Quality of Life (HRQoL) in persons with DMD in the seven countries.

The study of HRQoL aims at assessing the differences between countries or regions, change in HRQoL by age and the influence of medical treatment (steroids). Eligible and valid data on HRQoL were available for 944 patients aged 2-46 years. This part of the study presents the results on HRQoL among adult patients (>18 years) with DMD.

METHODS

Patients were recruited from the TREAT-NMD national DMD patient registries in the participating countries. In September 2011, an invitation to participate in the study was sent to each patient who had registered in the national registry in any of the seven countries. Questions of generic HRQoL (SF-36 and WHQOL-BREF) were sent to participants ≥ 18 years together with a comprehensive questionnaire on Care. All communication and questionnaires were in the local language.

Adult patients were categorized into three sub-age groups of 18-22 years (n=94), 23-27 years (n=92) and 28-46 year-olds (n=47). Scale scores ranging from 0 to 100 were calculated for both HRQoL questionnaires and the influence of age, Eastern/North-Western part of Europe and use of steroids on HRQoL were compared using one-factor ANOVA.

RESULTS

Overall, n=201 adult patients participated in the study. Out of these, n=183 adult patients had valid data on at least one of the analysed scales of the WHOQOL-BREF and/or SF-36. Distribution of participants according to age and European country is illustrated in Table 1. No difference was detected in any of the SF-36 domains or the WHOQOL-BREF between age-groups of adult patients.

Adults from North-Western European countries had higher mean scores in two SF-36 domains: Role-Physical (p=0.017) and Mental health (p=0.034) (figure 1) and in two WHOQOL-BREF domains: Physical (p=0.001) and Environment (p=0.002), additionally, a trend was found for the scale Psychological (p=0.056) (figure 2).

One patient (<22 y) from Eastern Europe and 12 patients (aged 18-26 y) from North-Western European countries were on steroids. Six patients from Eastern European countries and 45 patients from North-Western European countries had used steroids, and 28 patients from Eastern European countries and 91 patients from North-Western European countries had never had steroids.

No differences were seen in any of the SF-36 domains or the WHOQOL-BREF domains according to the state of steroid-use.

In general, scores on SF-36 were lower in DMD patients compared to normative data.

DISCUSSION

The results indicate that increasing age in adults with DMD does not lead to a lower HRQoL as measured by SF-36 and WHQOL-BREF, nor does the use or non-use of steroids. The differences between participants in some of the domains in the HRQoL scales (e.g. Mental and Environmental) could be due to better economics in North-Western European countries where a person with DMD has more aids and, thus, other preconditions for participation. However, the results must be “handled with care” since the age and country specific patient groups differed in size. There were four times as many adult participants from North-Western European countries compared to Eastern European countries and only ten participants were more than 22 years in the Eastern European countries. The latter could be due to a possible low registration rate in the Eastern European countries, but it could also indicate a need for improving rehabilitation and respiratory care in order to increase the adult population.

PARTNERS

CARE-NMD is led by Dr Janbernd Kirschner at the University Clinic Freiburg.

The UK academic partners are Prof Kate Bushby and Prof Hannah Lochmuller at Newcastle University.

Both Action Duchenne and the Muscular Dystrophy Campaign and are UK partners, and have been actively involved in the project.

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Unfunded partners include clinicians, universities, and patient organisations across more than 20 countries. For a complete list of partners, see the CARE-NMD website.

www.care-nmd.eu