

## 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 ( $\geq 18$  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 register in any of the seven countries. Questions of generic HRQoL (SF-36 and WHOQOL-BREF) were sent to participants  $\geq 18$  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-factorial ANOVA.

## SF-36

is a patient-reported survey of health; it consists of 36 items covering eight domains: Physical functioning, Role-physical, Bodily pain, General health, Vitality, Social functioning, Role-emotional and Mental health (Ware & Gandek, 1998). In this study on DMD-populations the scale Physical functioning was excluded from the analyses since the majority of patients had a scale score of 0 indicating extremely low physical functioning.

## WHOQOL-BREF

consists 26 items covering four domains: Physical, Psychological, Social relationships and Environment (The WHOQOL Group, 1998).

In both scales - the higher score the higher perceived quality of life.

## 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=.017) and Mental health (p=.034) (figure 1) and in two WHOQOL-BREF domains: Physical (p<.001) and Environment (p=.002), additionally, a trend was found for the scale Psychological (p=0.56) (figure 2).

One patient ( $\leq 22$  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.

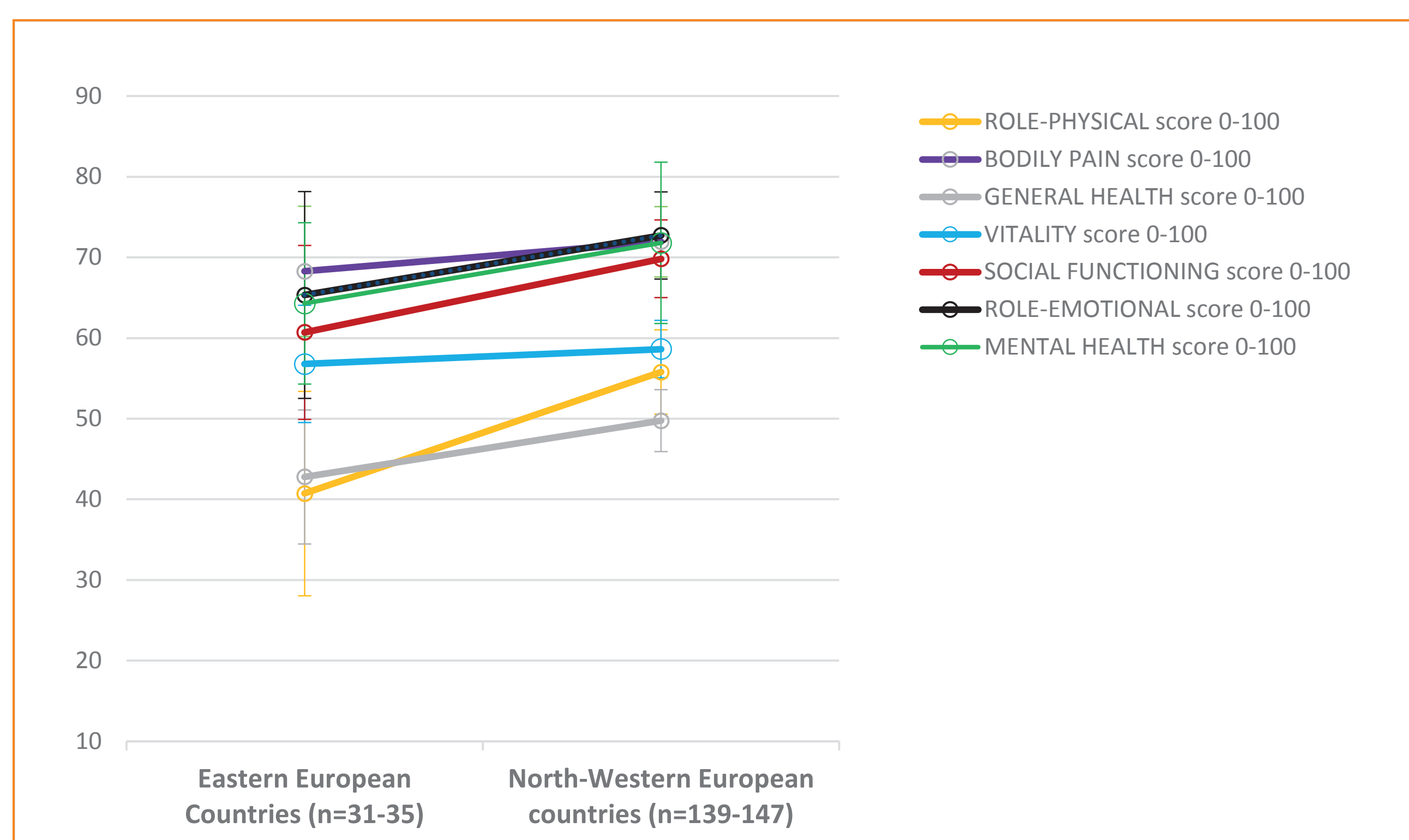
No differences were seen in any of the SF-36 domains or the WHOQOL-BREF domains according to the state of steroid-use.

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

Table 1 183 participants according to age-group and European country

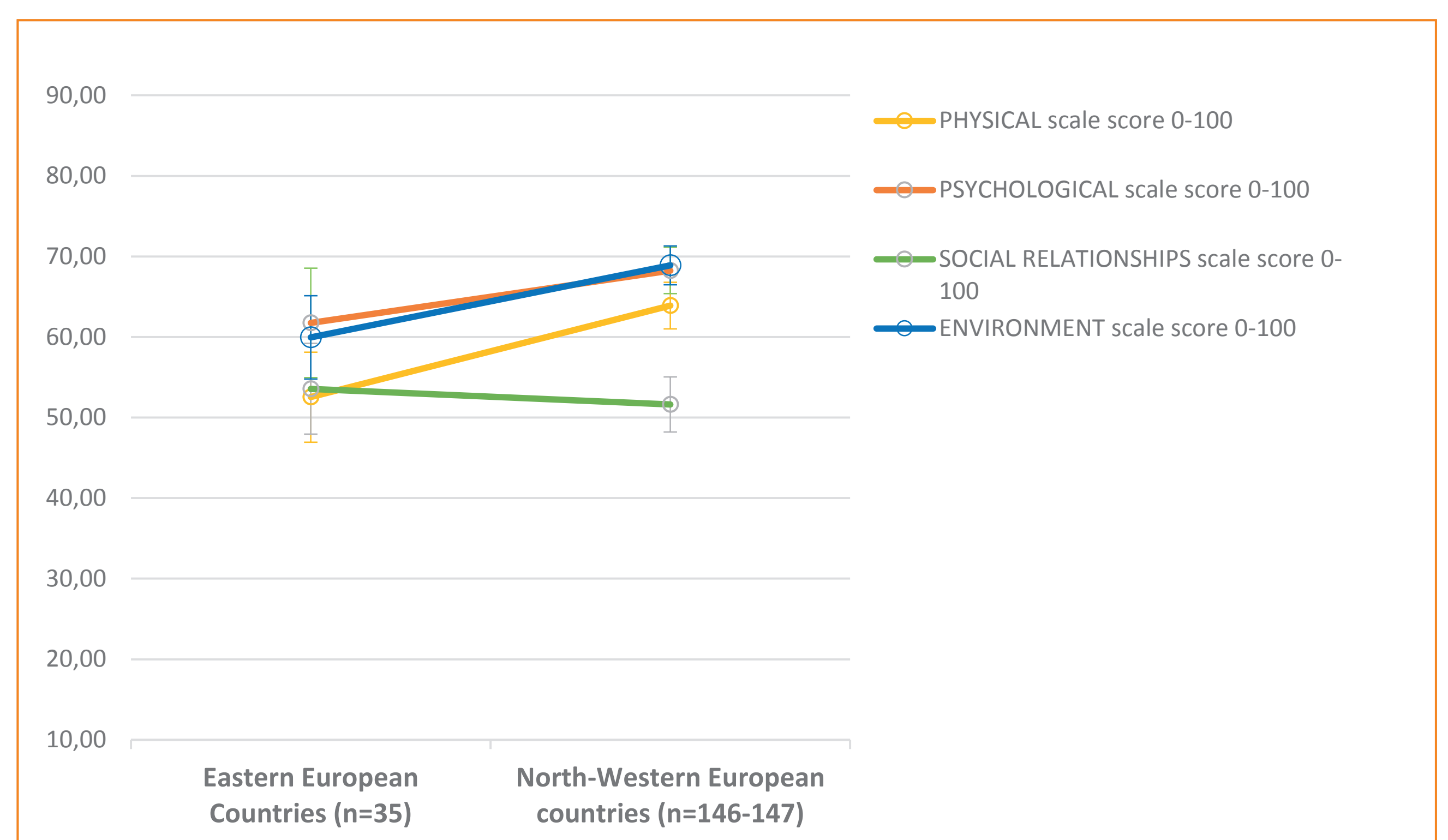
Age-group	Bulgaria (n)	Poland (n)	Hungary (n)	The Czech Republic (n)	Eastern European countries (n)	Denmark (n)	Germany (n)	The United Kingdom (n)	North-Western European countries (n)
18-22 years	5	7	4	9	25	9	42	18	69
23-27 years	1	3	-	1	5	8	16	13	37
28-46 years	1	4	-	-	5	23	12	7	42
Total	7	14	4	10	35	40	70	38	148

Fig. 1 Health-Related Quality of Life due to the SF-36 in adult patients by European region.



Error bars represent Confidence intervals (95%) of means. The SF-36 scale Physical function was excluded from the analyses.

Fig. 2 Health-Related Quality of Life due to the WHOQOL-BREF in adult patients by European region

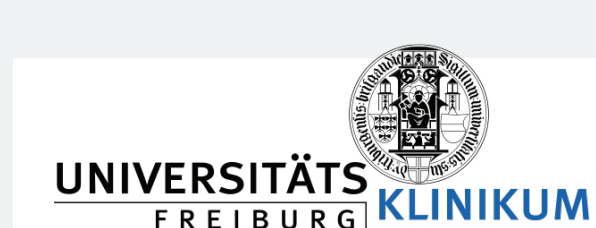


Error bars represent Confidence intervals (95%) of means.

## DISCUSSION

The results indicate that increasing age in adults with DMD does not lead to a lower HRQoL as measured by SF-36 and WHOQOL-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 Environment) 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

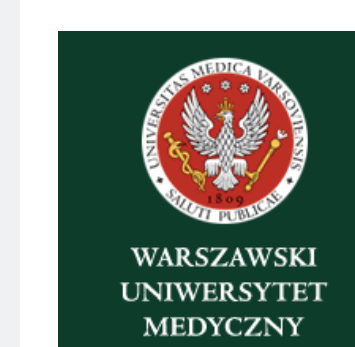


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The UK academic partners are Prof Kate Bushby and Prof Hanns Lochmüller at Newcastle University.

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

Other funded partners include the Bulgarian Neuromuscular Disorders Society (BGNMDS), Brno University in the Czech Republic, the Danish Rehabilitation Centre for Muscle Diseases, the Hungarian National Institute for Environmental Health, and Warsaw Medical University in Poland.



БДНМЗ  
BGNMDS

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](http://www.care-nmd.eu)