



Results of Quality of Life Questionnaires

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Workpackage 7

- AIM:
 - Assessment of health related QoL among children and adults with DMD
 - To evaluate
 - Change of health related QoL by age and increasing disability
 - Differences between countries or regions
 - Differences in relation to a norm population
 - QoL-Instruments' and their relevance to DMD
 - Relationship to care, social and educational levels

Definitions of QoL are multiple

- **WHO: Individuals'** perception of his/her position in life in context of the **culture** and **value system** in which he/she lives and in **relation** to his/her goals, expectations, standard and concerns.
- **In simple words:** The extent to which hopes and ambitions are matched by experience

Existing studies

Children with a neuromuscular disorder

- regard themselves as resembling healthy children
- regard their QoL as nearly normal to healthy

Bach 1991 and others

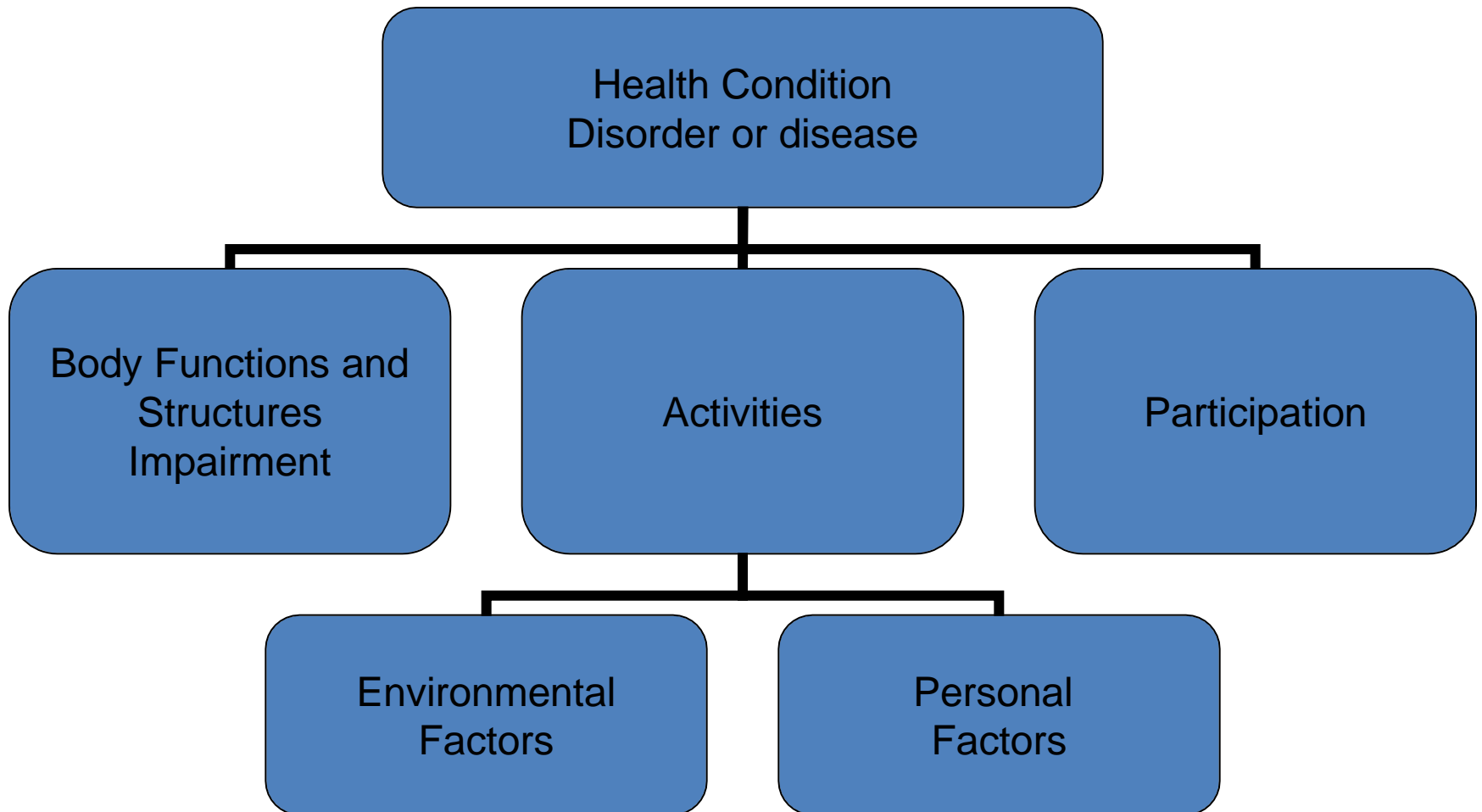
- Parents and children correlate on scoring physical functioning
- Parents and children differ in scoring emotional functioning

Varni, 2005

- Score significant lower QoL compared to unaffected children of the same age

Uzark, 2013

International Classification of Functioning, Disability and Health (WHO 2001)



Elements of ICF that are important from patient's perspective

- **Body function:** physical well being, energy, good mood, lack of pain, the ability to sit, stand or walk safely.
- **Activity and participation:** family, friends, access to leisure activities, school/ education/ work/ and communication with others, can move around independently.
- **Personal factors:** social skills, autonomy and self determination. (Studies show that initiative, self confidence and empathy are commonly missing among boys with DMD.)
- **Environmental factors:** accessibility in-doors as well as out-doors, practical help by other persons or technical aids, economy, to be met/treated by other people at equal terms. Other people's expectations to be an active participant .

Rahbek et al, 2005

Choice of health related QoL instruments

Children:

PedsQL™ (Pediatric Quality of Life Inventory) (generic)
and Neuromuscular Module (disease specific))
KIDSCREEN (generic)

Adults:

SF-36 (generic)
WHOQOL-BREF (WHO Quality of life) (generic)

Health related QoL instruments contain

Dimensions on

- Health
- Physical well-being
- Functional ability
- Emotions
- Social domains

– Tulskey DS, Rosenthal M. Arch Phys Med Rehabil 2002; 83 suppl 2: S1-3

Timeline

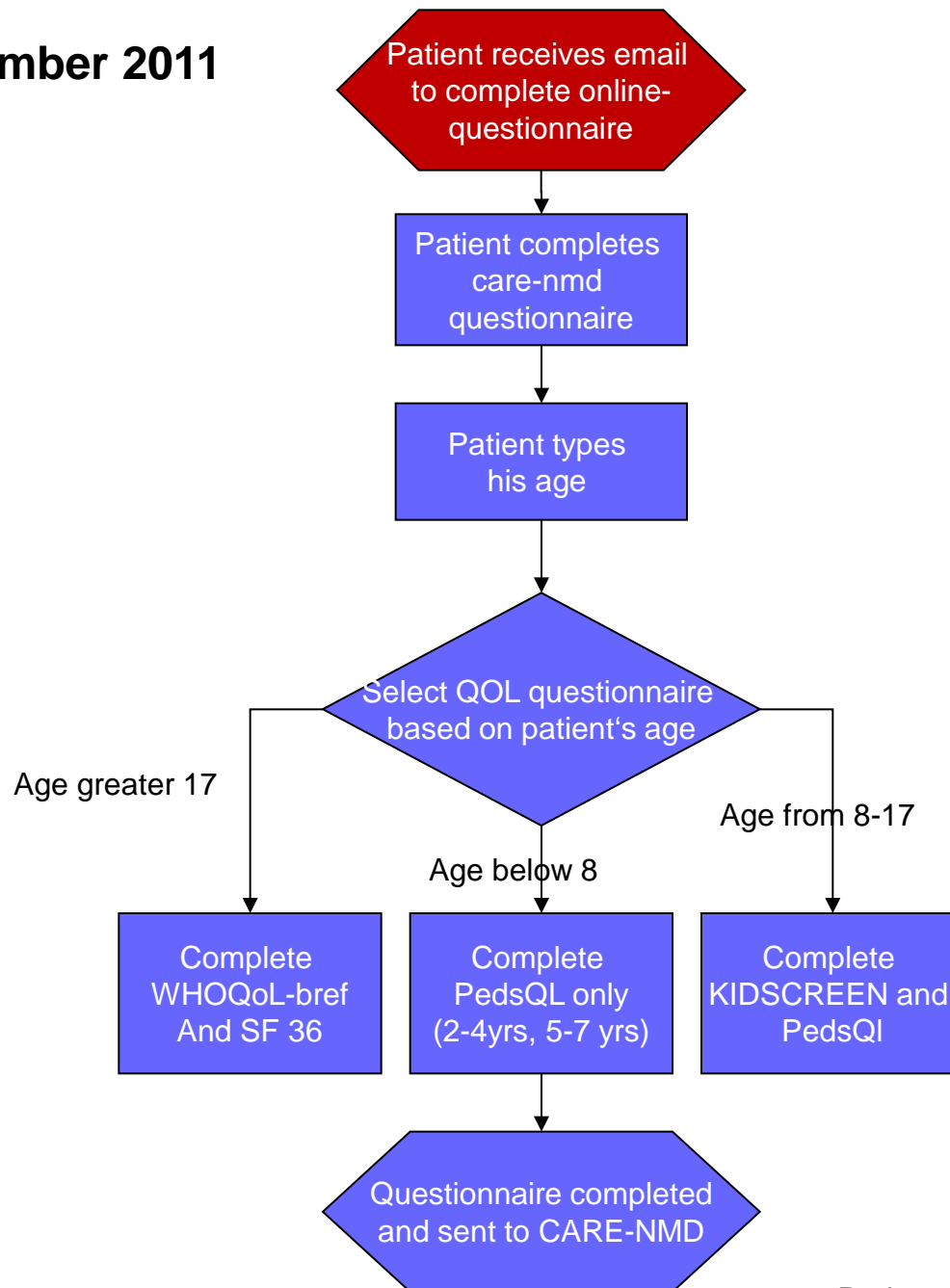
2010 – 2011

- Construction of care questionnaire (DE,DK,UK)
- Tracing and translating QoL questionnaires (BL, HU, PL, DK)
- Setting up Survey Monkey (for electronic response)
- Recruiting patients from registries, hospitals and patient organizations

2011: September – January 2011

- Care and QoL questionnaires were sent to aprox 1650 patients

September 2011



Jan. 2012

Data collation
completed



Cleaning data from the
Care questionnaire



Jul. 2012

Cleaning completed



Cleaning QoL questionnaires and
converting raw scores to domains



March 2013

Cleaning and converting
QoL data finished
Datasets merged

**Totally 1062
respondents**

		country						region		total	
		BL	HU	PL	CZ	DK	DE	UK	Eastern Europe*		North-western Europe**
age group	0 to 1 year	0	2	0	1	0	7	0	3	7	10
	2 to 4 years	1	7	10	9	3	27	13	27	43	70
	5 to 7 years	7	11	28	20	6	79	32	66	117	183
	8 to 12 years	14	19	49	28	17	134	91	110	242	352
	13 to 17 years	11	13	39	20	19	96	48	83	163	246
	18 to 22 years	5	4	7	10	10	46	21	26	77	103
	23 to 27 years	1	1	5	1	9	17	14	8	40	48
	28 to 46 years	1	0	4	0	24	14	7	5	45	50
<i>total</i>	40	57	142	89	88	420	226	328	734	1062	

* Bulgaria, Hungary, Poland and The Czech Republic

** Denmark, Germany and The United Kingdom

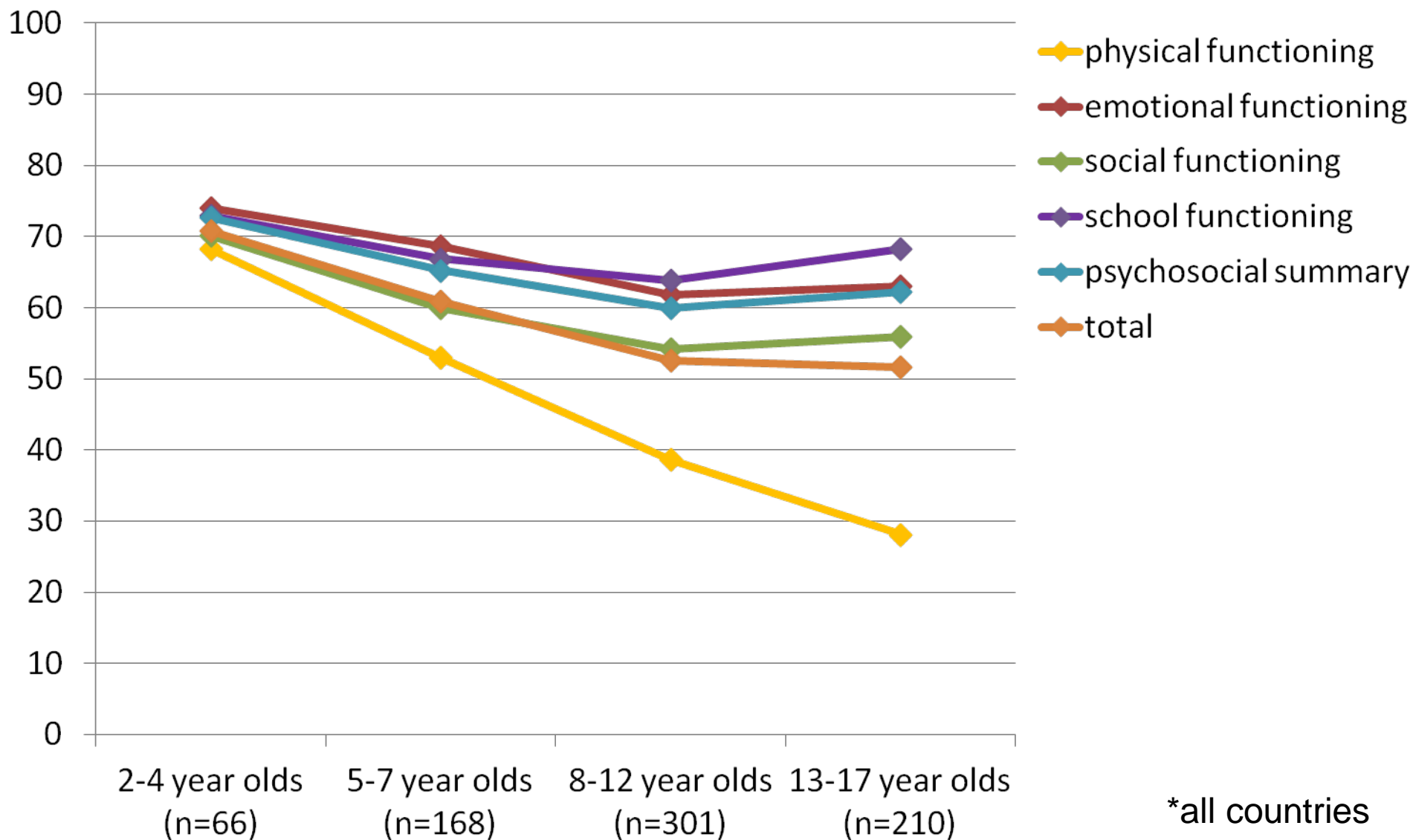
Respondence to Questionnaires (at least on scale calculable)

Questionnaire	Total responses	Eastern Europe	North-West Europe
PedsQL generic			
Child	646/781	229	417
Parent	745/921	264	481
PedsQL NMM			
Child	640/781	226	413
Parent	672/781	236	436
KIDSCREEN 27			
Child	503/598	169	334
Parent	516/598	175	341
SF 36	182/201	35	147
WHOQL-BREF	182/201	35	147

Results

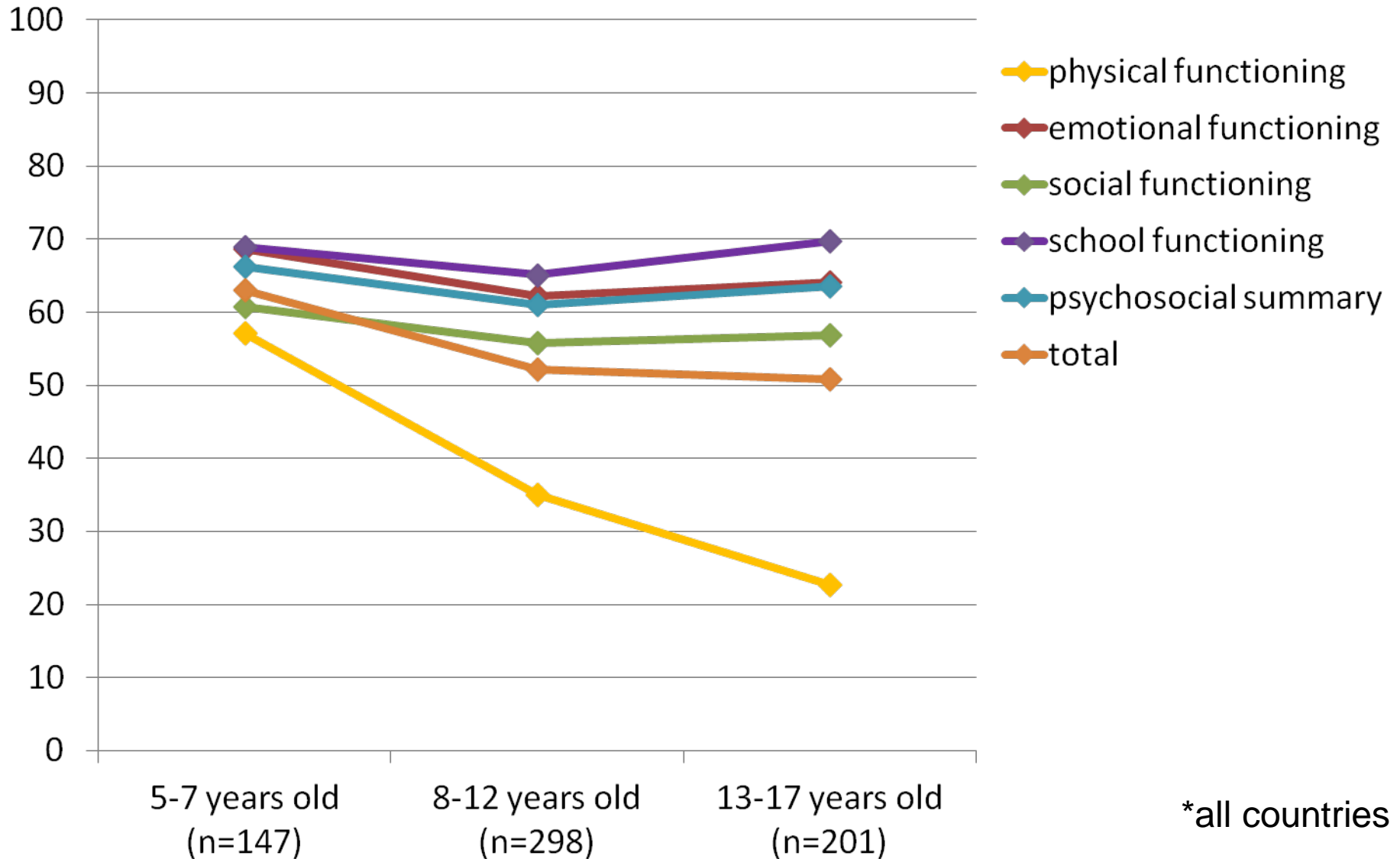
Does health related QoL change by age in boys and adults with DMD?

Parent-reported Health related Quality of Life (HrQoL) (due to the PedsQL generic core instrument)*



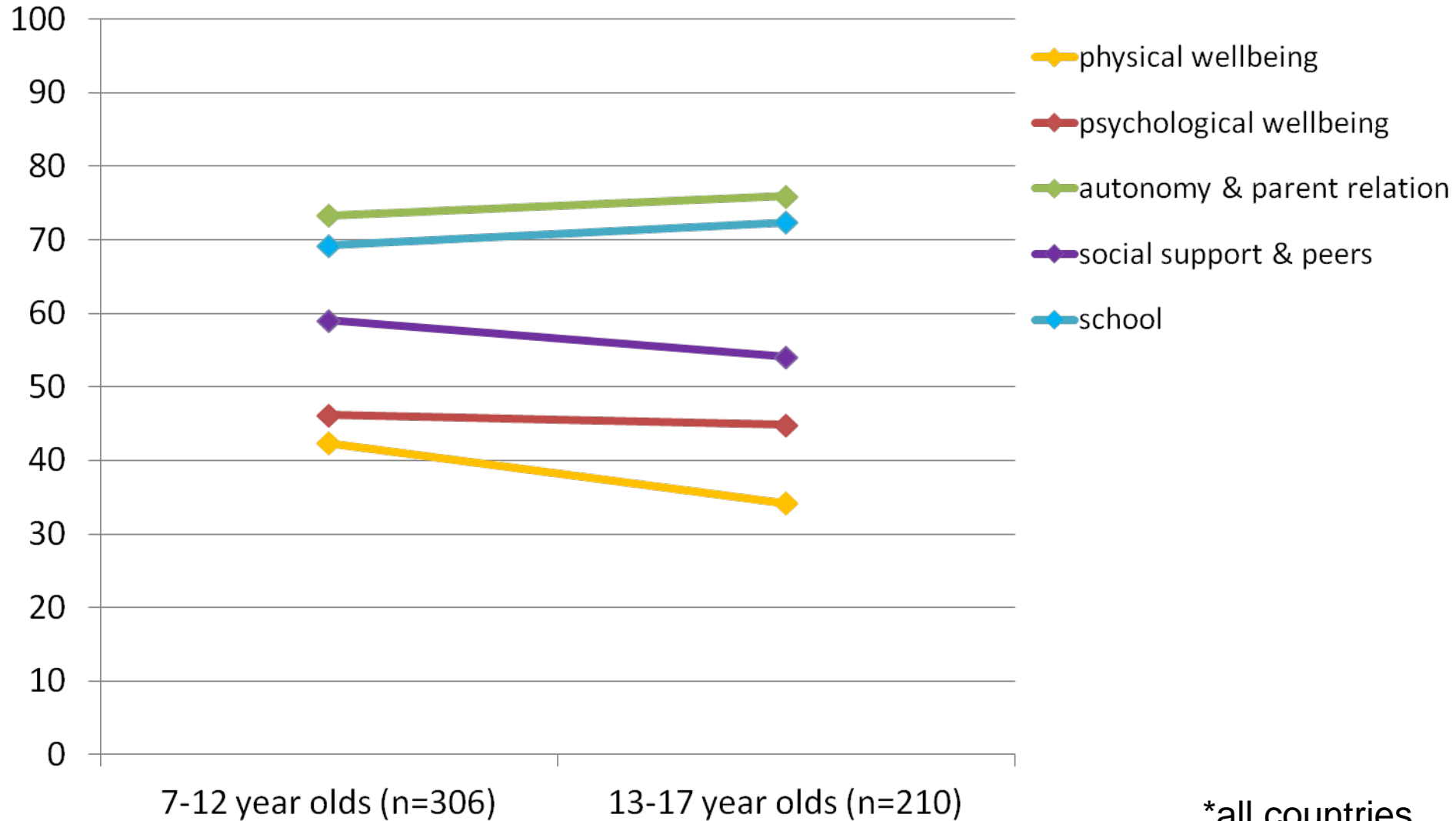
Self-reported HrQoL

(due to the PedsQL generic core instrument)*



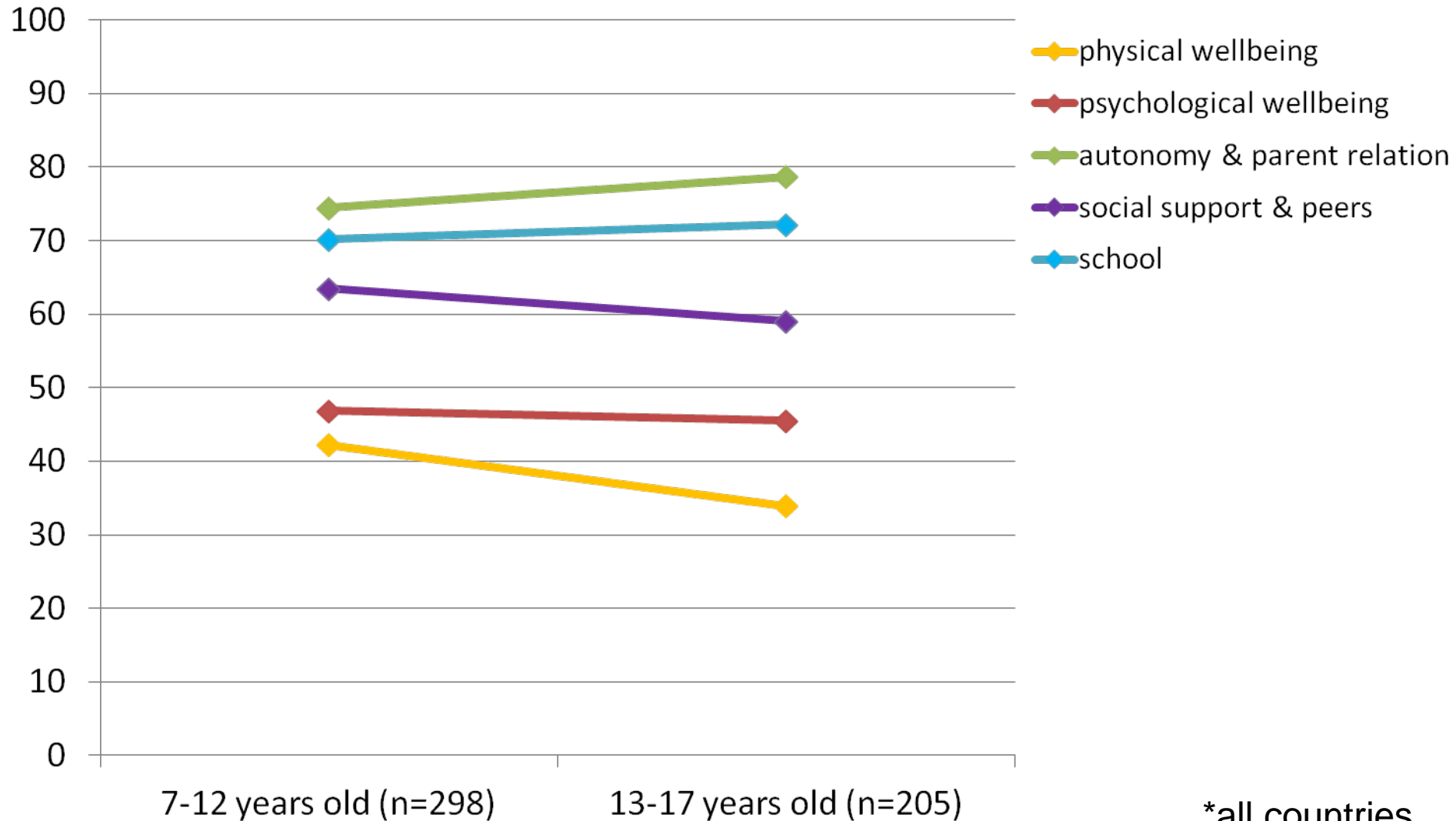
*all countries

Parent-reported generic HrQoL (due to the KIDSCREEN-27)*



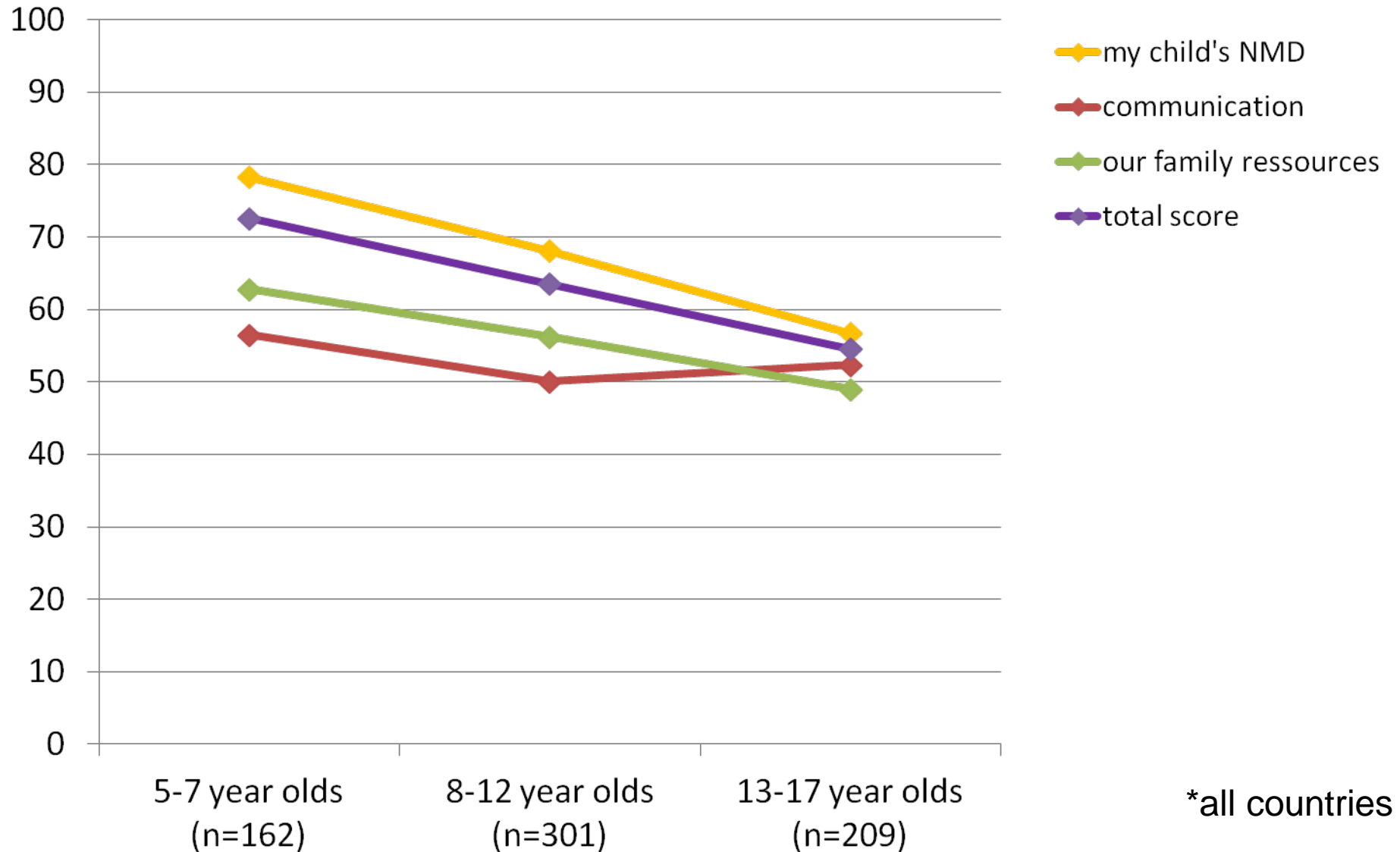
*all countries

Self-reported generic HrQoL (due to the KIDSCREEN-27)*



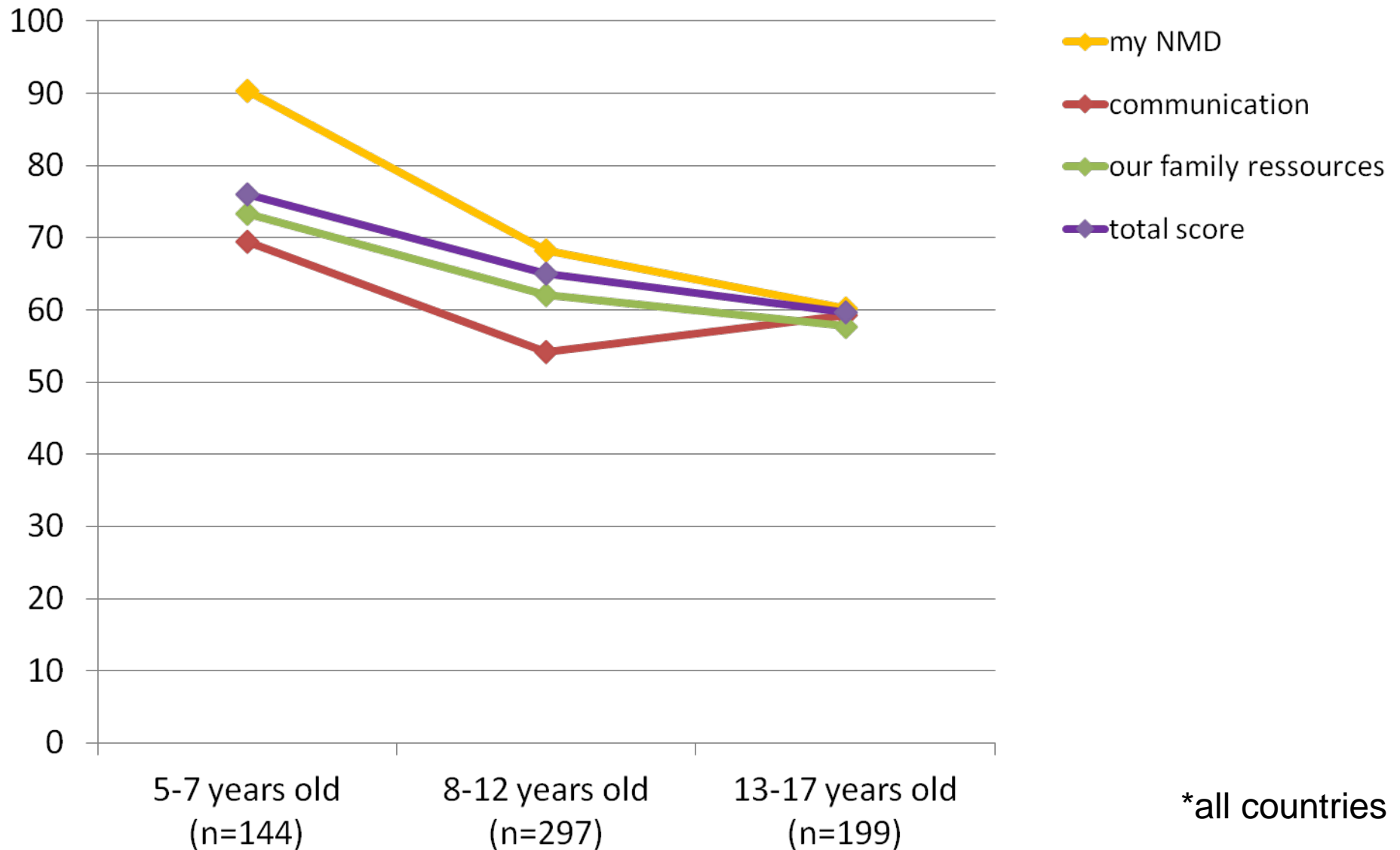
*all countries

Parent-reported disease-specific QoL (due to the Neuromuscular module of the PedsQL)*

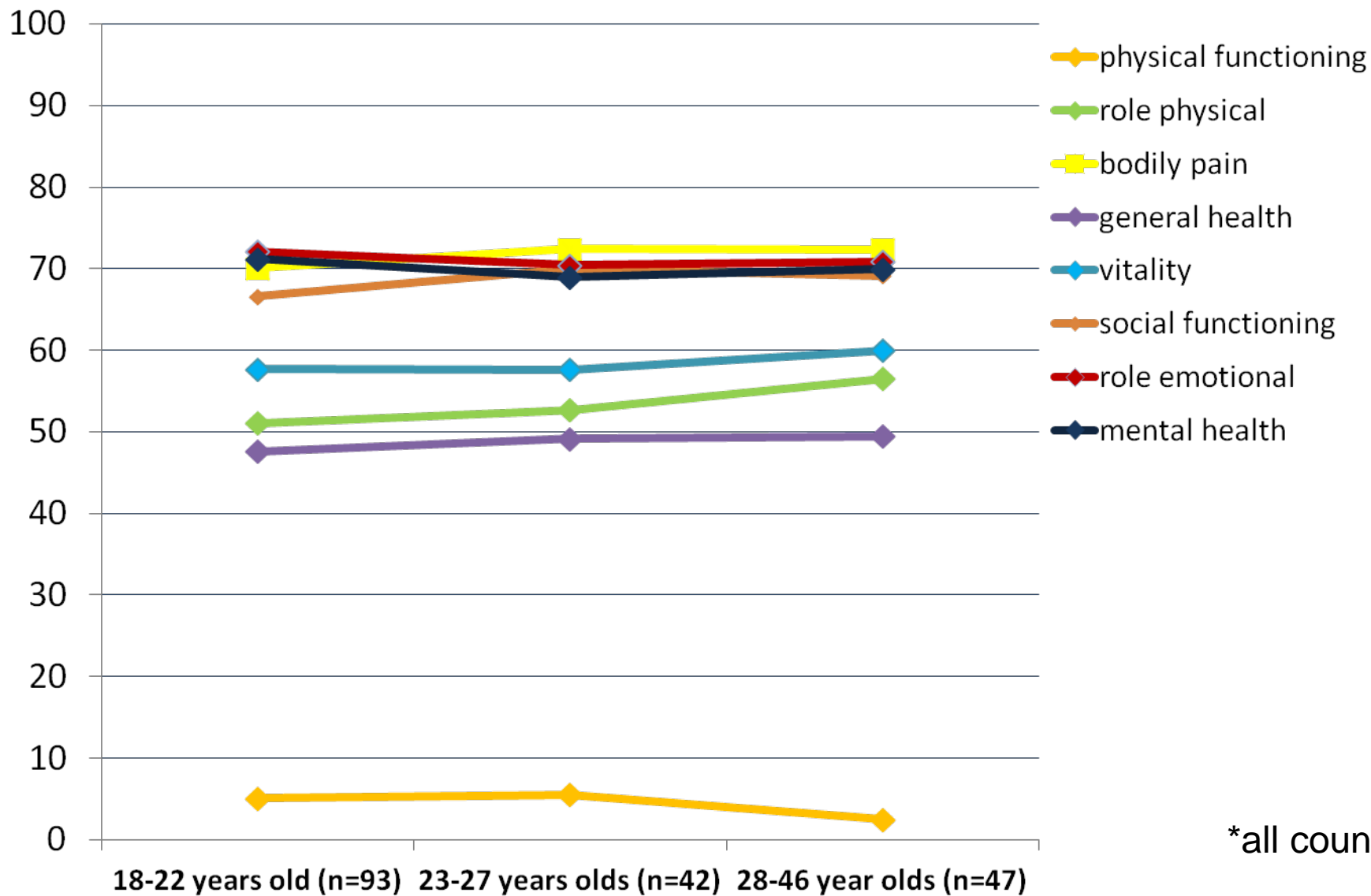


Self-reported disease-specific QoL

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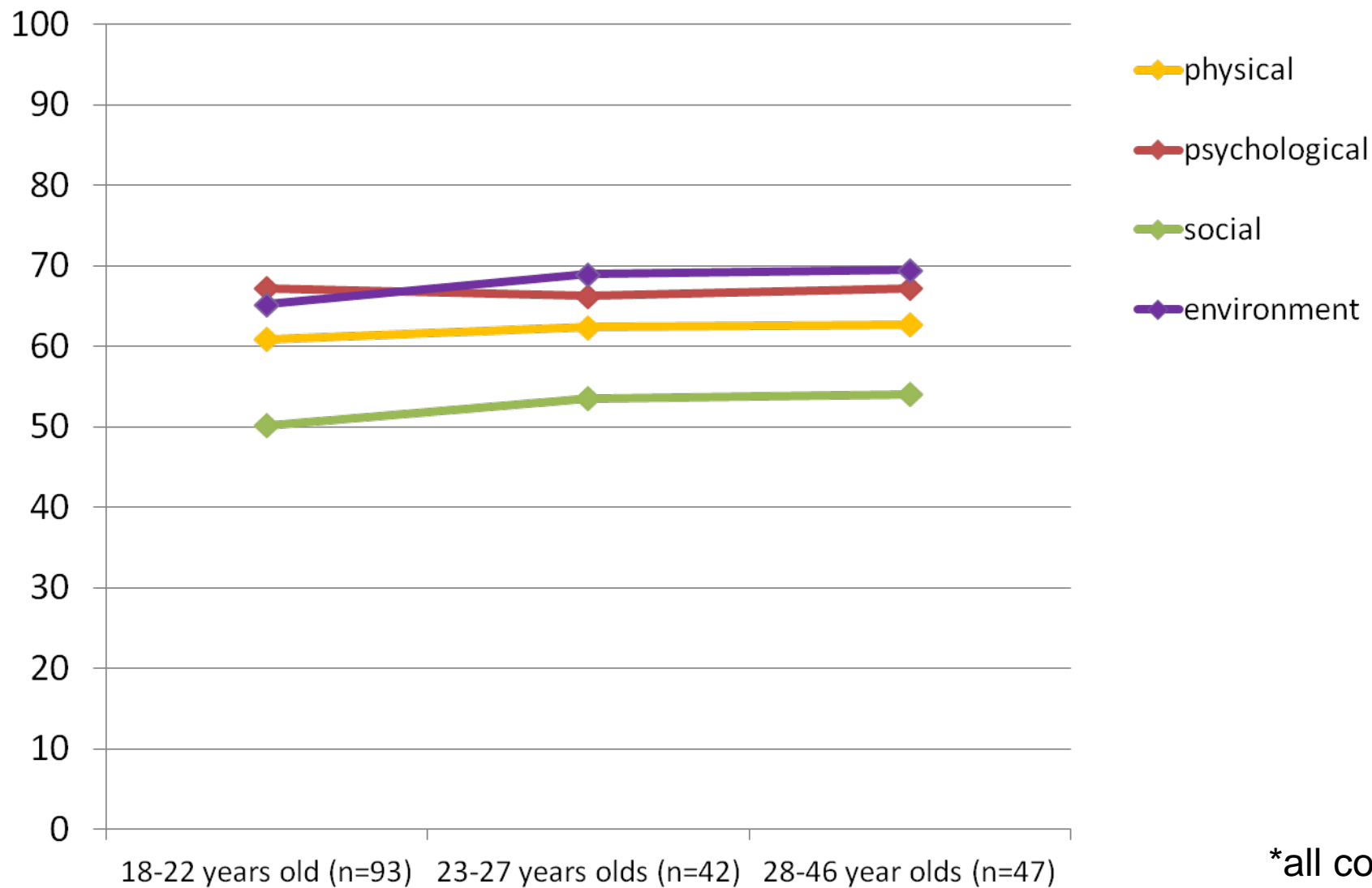


Adult generic HrQoL (due to the SF-36)*



*all countries

Adult generic HrQoL (due to the WHOQoL)*



*all countries

Results

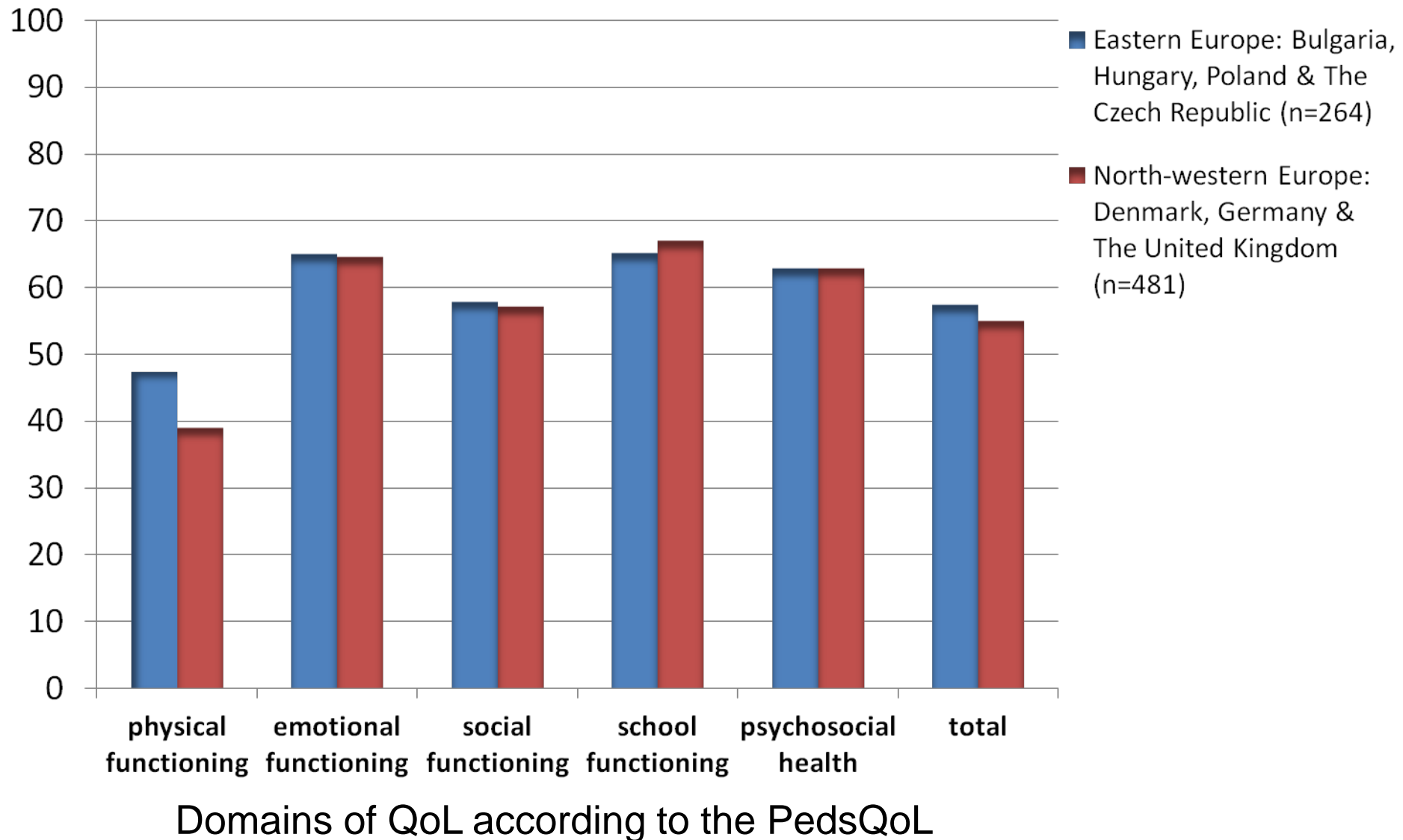
Comparing regions

Means of patients from Eastern and North-western European countries

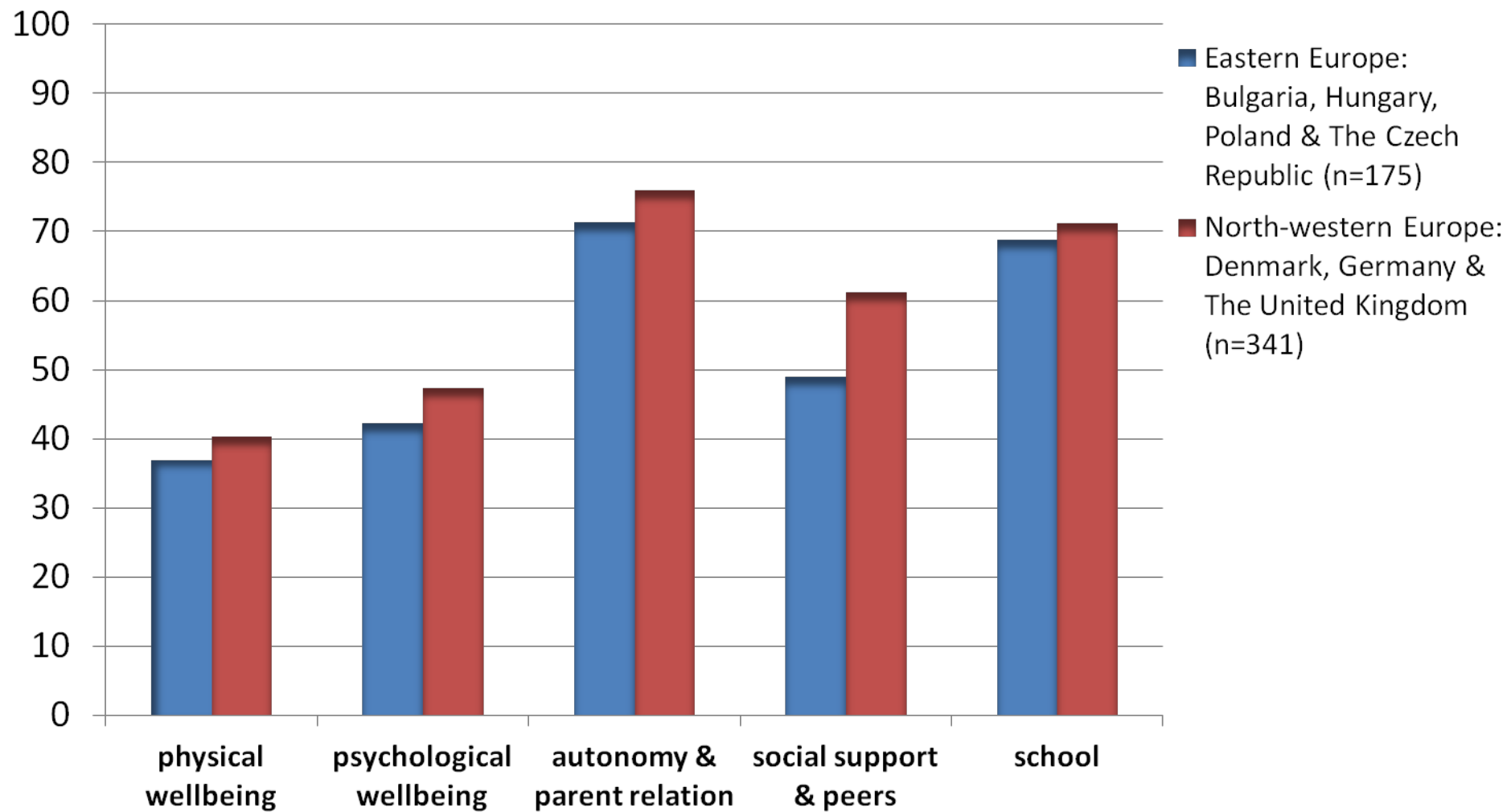
PedsQL generic: parent-report, child-report

- PedsQL NMD module: parent-report, child-report
- Kidcsreen-27: parent-report, child-report
- SF-38: adult-report
- WHOQoL: adult-report

Parent-reported HrQoL in pediatric patients from Eastern vs. North-western European countries

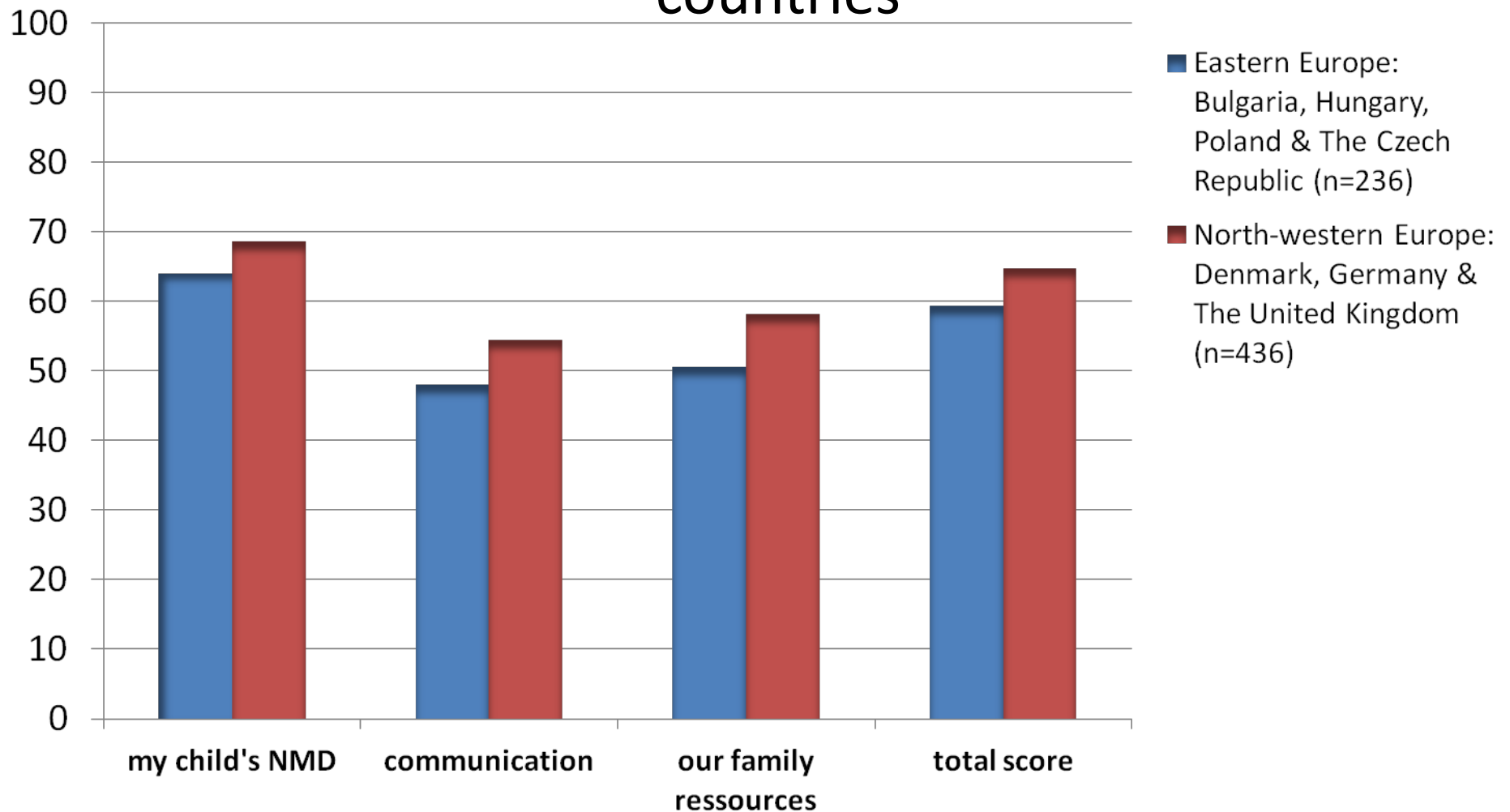


Parent-reported QoL in pediatric patients from Eastern vs. North-western European countries



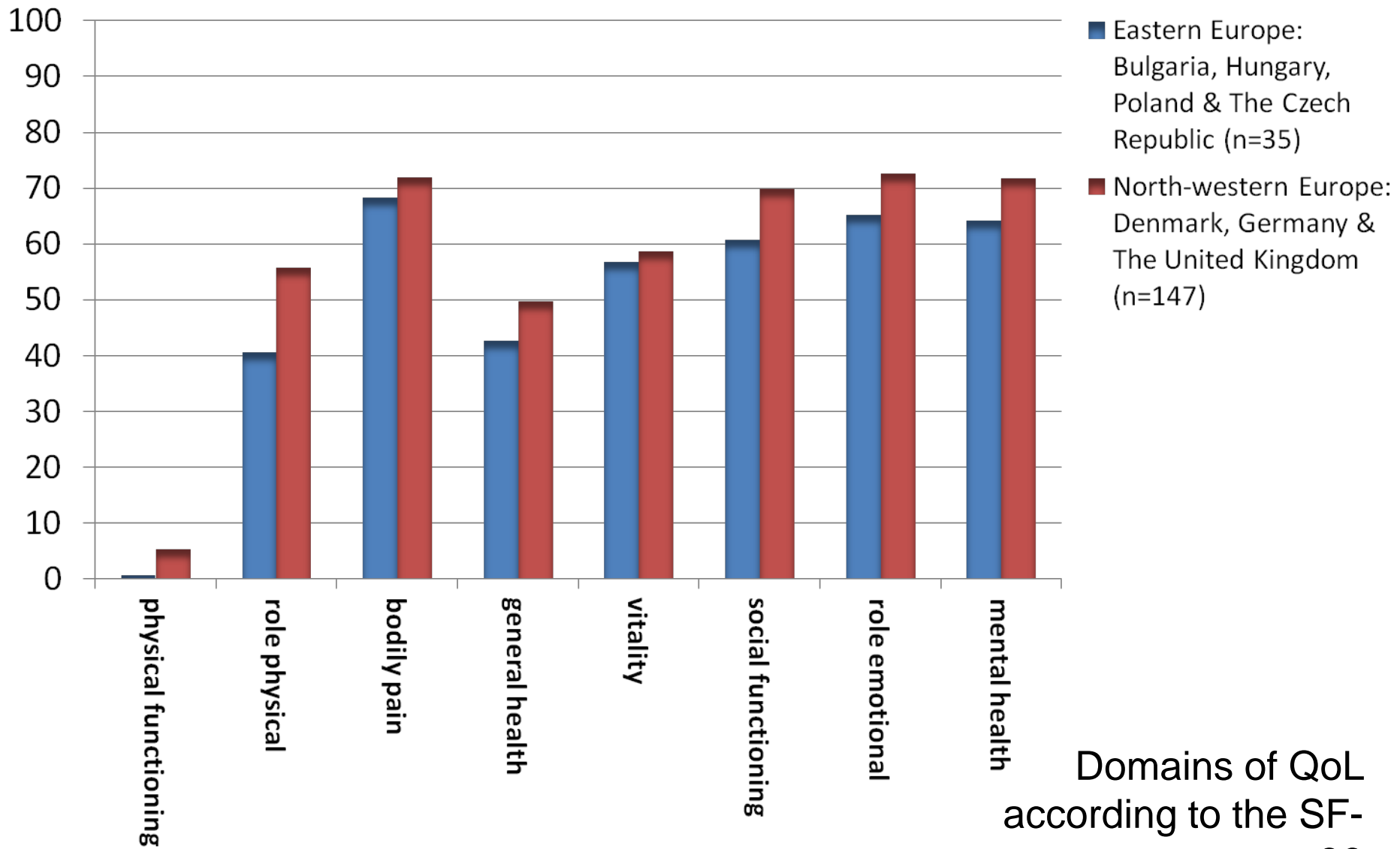
Domains of QoL according to the Kidscreen-27

Parent-reported disease-specific QoL in pediatric patients from Eastern vs. North-western European countries

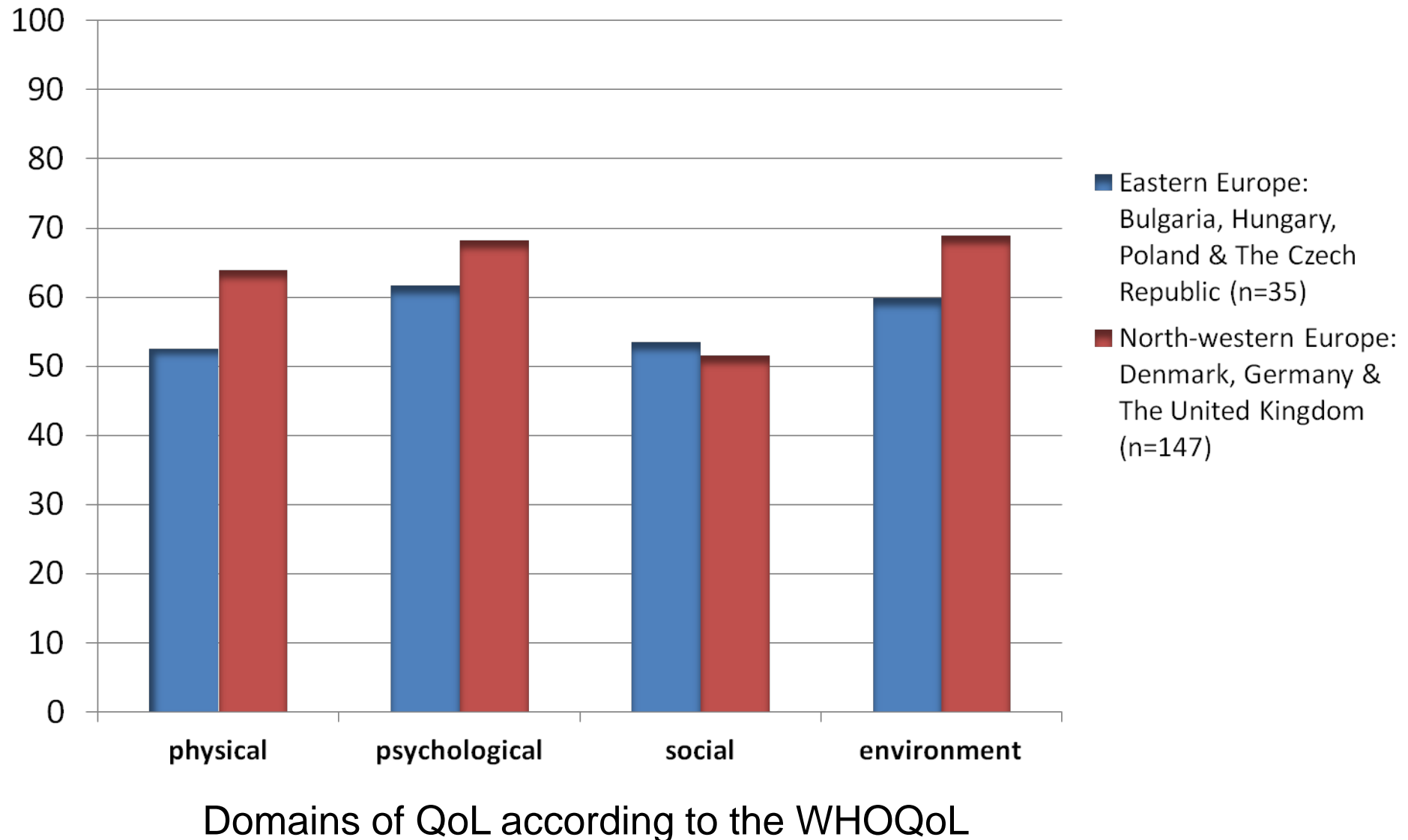


Domains of QoL according to the Neromuscular (NMD) module of the PedsQL

Adult generic HrQoL in patients from Eastern vs. North-western European countries



Adult generic HrQoL in patients from Eastern vs. North-western European countries



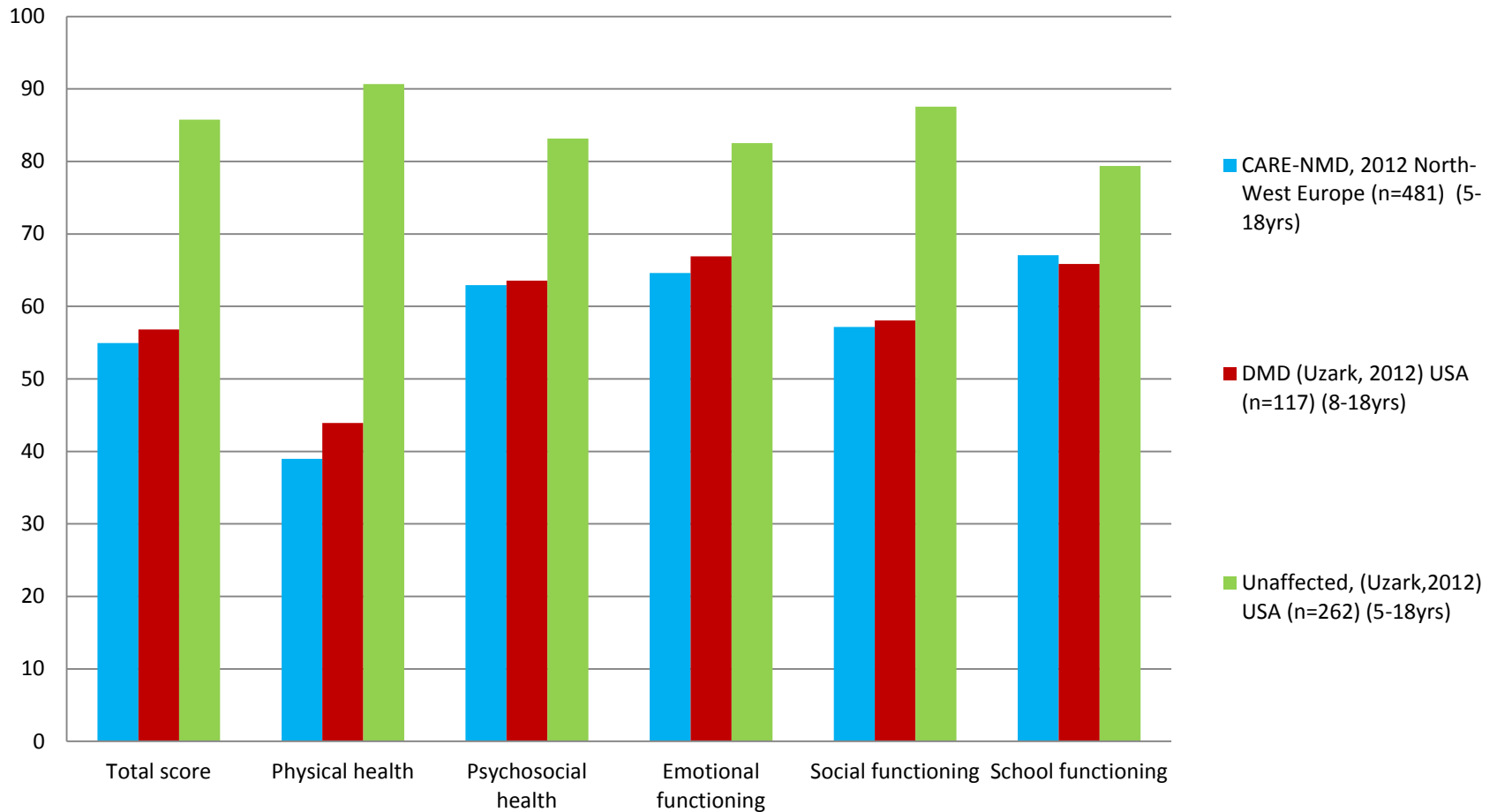
Results

Differences of QoL in relation to other studies
and to a norm population

Comparison: North-western Europe with USA

PedsQL generic core set

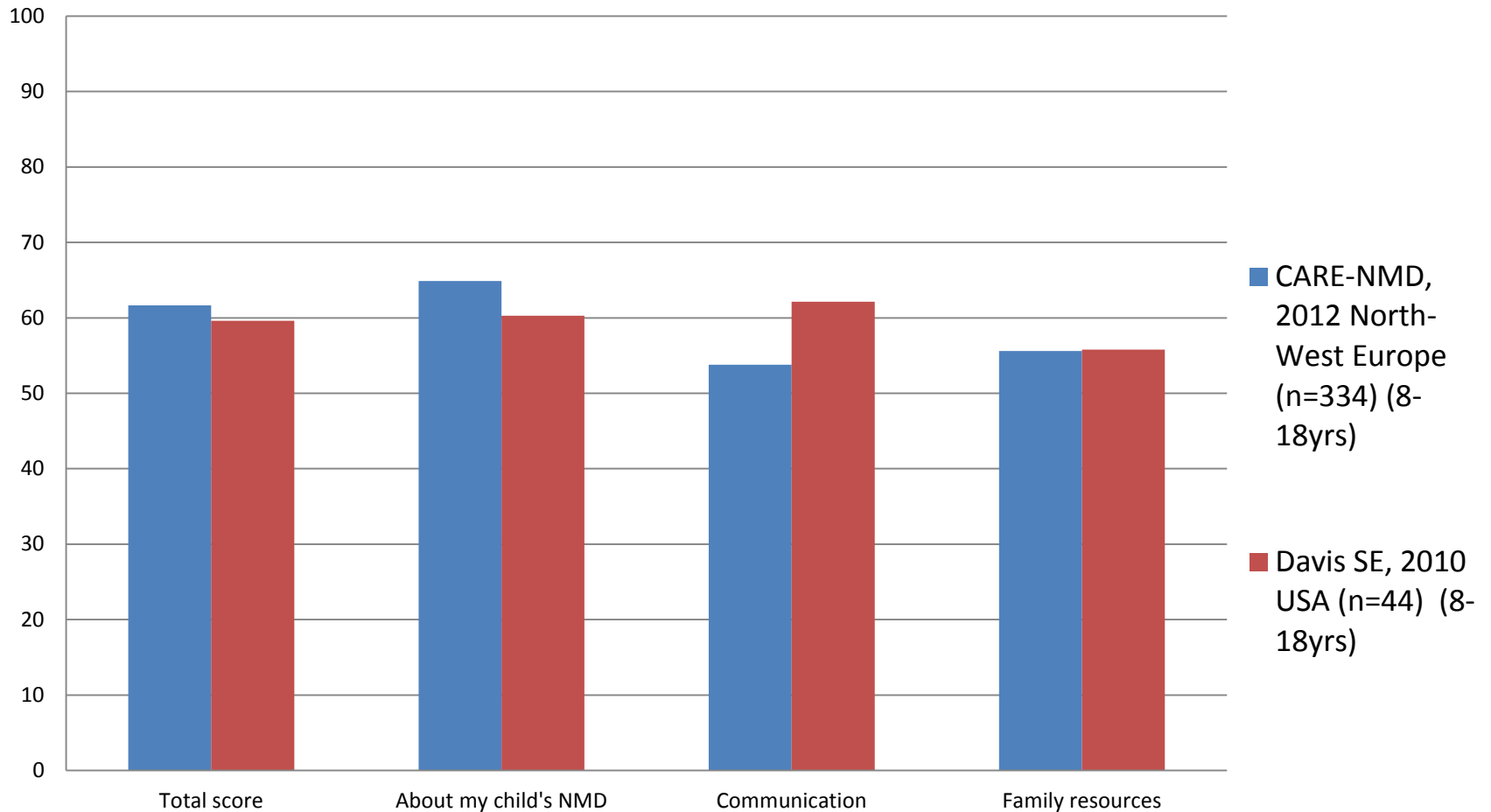
Parents' report



Comparison North Western Europe with USA

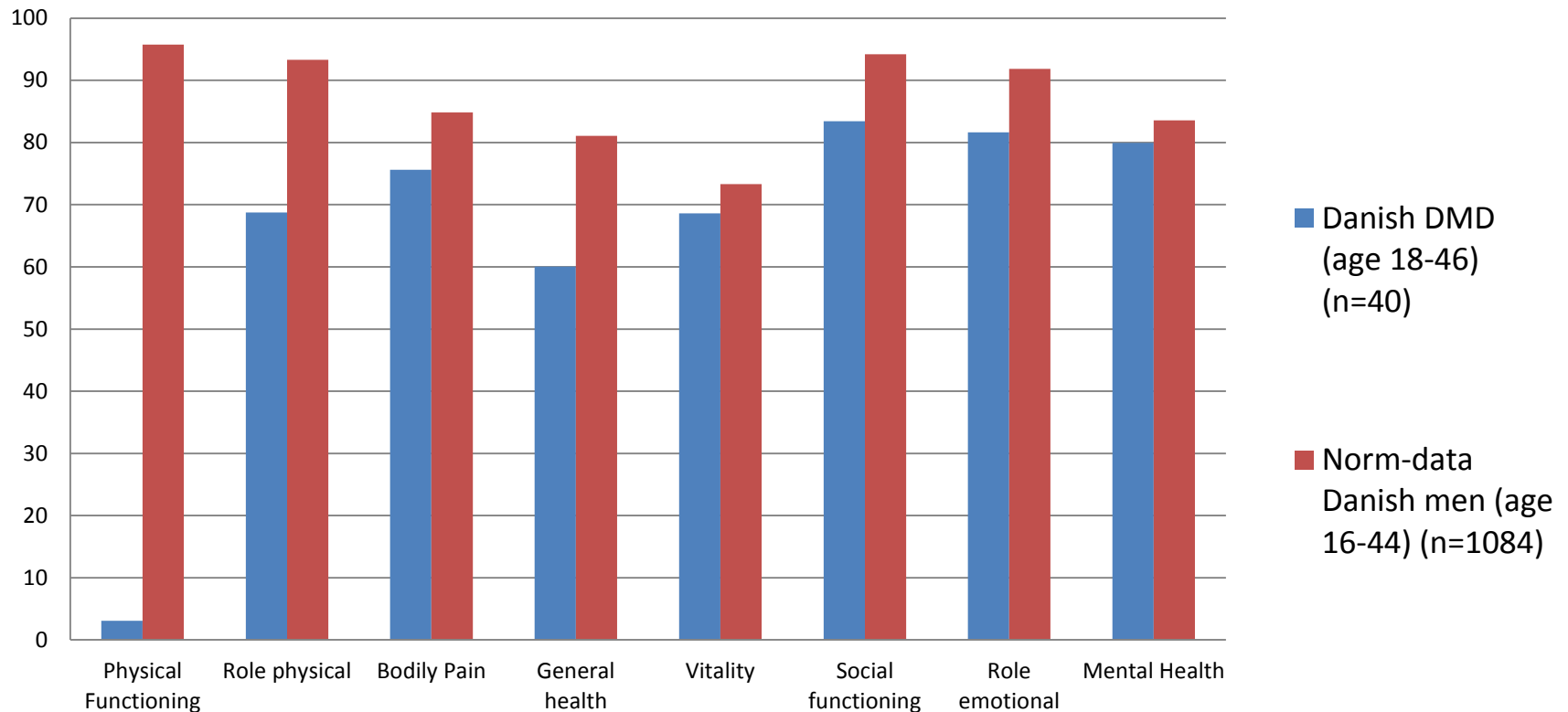
PedsQL NMM

Parents' report



Danish adults with DMD vs. Danish norm data

SF-36



Conclusion: General aspects

- Largest inter-cultural multicentre study in DMD populations
- Inter-cultural multicentre studies are feasible
- Our data do not represent a total DMD population in individual countries (except Denmark)
- Small but consistent differences between Eastern and North Western European countries

Conclusion: Responses concerning children

- Physical domain decrease until 18 yrs (all questionnaires)
- Emotional and school domains increase by age after 12 yrs (all questionnaires for children)
- Social support and relationships after 12 yrs: inconsistency (KIDSCREEN vs PedsQL)
- Autonomy increase slightly after 12yrs (KIDSCREEN)
- Slight tendency to children scoring themselves better than their parents

Conclusion: Responses concerning adults

- Few responses from all countries
- No change by age as measured by SF-36 and WHOQL-BREF (including users of assisted ventilation)
- Differences in physical functioning in the two questionnaires is caused by different questions asked

Conclusion: QoL-Instruments and their relevance to DMD

Generally the QoL questionnaires chosen were relevant and informative in combination

- PedsQL generic core scale
 - Comparable with other DMD studies
 - Focus on negative aspects
- KIDSCREEN
 - Not yet comparable with DMD studies
 - Focus on positive aspects and participation
- SF-36
 - Comparable with other studies
 - Aspects of physical activity not relevant for DMD
- WHOQL-BREF
 - Focus on autonomy and mood