UNIVERSITÄTS KLINIKUM FREIBURG

Results of the
CARE-NMD questionnaire

Jan Kirschner

CARE-NMD Conference
18-19 April 2013 – Budapest, Hungary

Care recommendations for DMD

Consensus process

Care recommendations for DMD

Consensus process

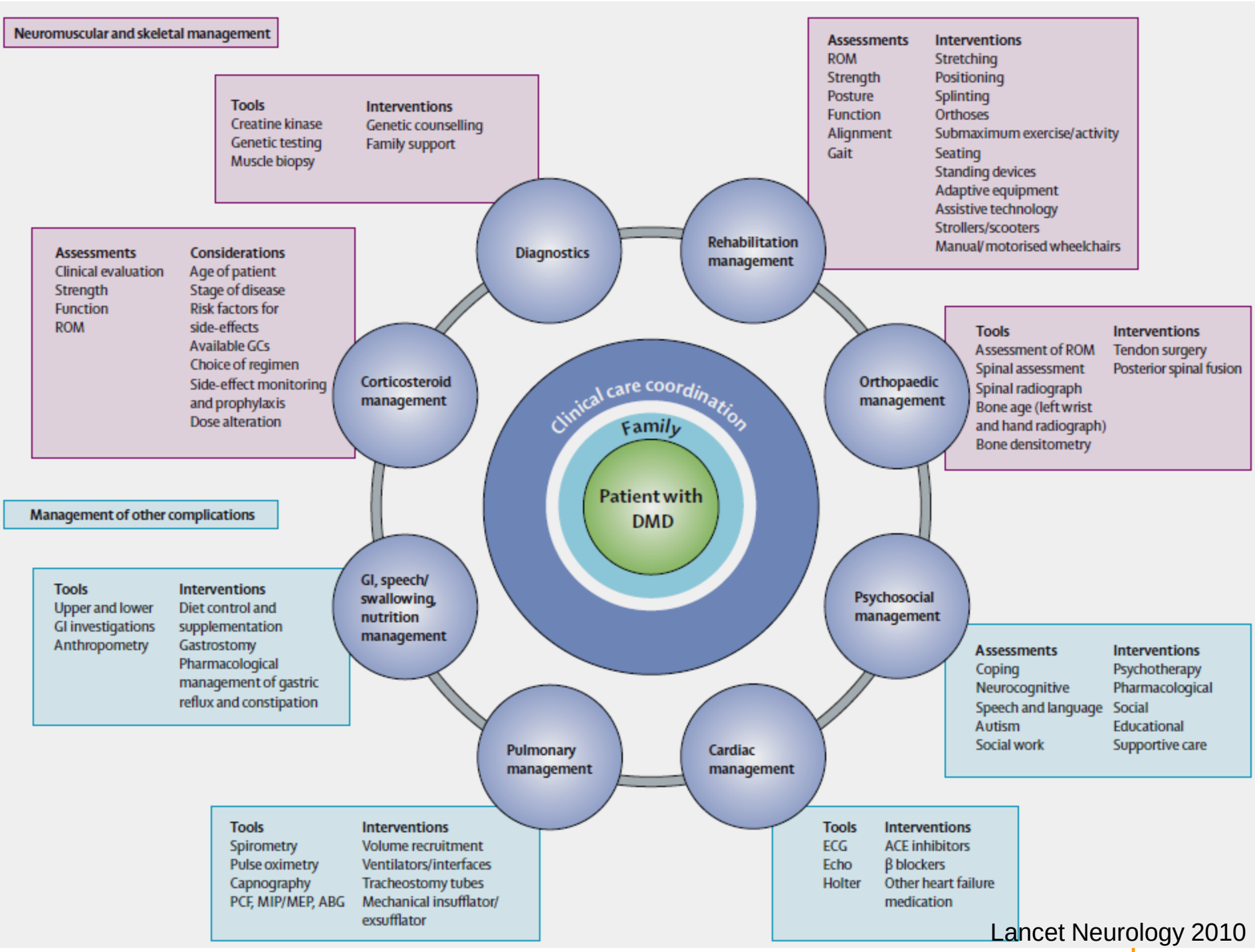
Scientific publication

MDA Helping Jerry's Kids | Parent Project Muscular Dystrophy LEADING THE FIGHT TO END DUCHENNE | TREAT-NMD Neuromuscular Network From 2007 to 2011 inclusive, TREAT-NMD is a "network of excellence" supported through Priority 1 (Life Sciences, Genomics and Biotechnology for Health) of the European Union's Sixth Framework Programme. | United Parent Projects Muscular Dystrophy

Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management

*Katharine Bushby, Richard Finkel, David J Birnkrant, Laura E Case, Paula R Clemens, Linda Cripe, Ajay Kaul, Kathi Kinnett, Craig McDonald, Shree Pandya, James Poysky, Frederic Shapiro, Jean Tomezsko, Carolyn Constantin, for the DMD Care Considerations Working Group**

CARE-NMD



Lancet Neurology 2010

Care recommendations for DMD

Consensus process

↓

Scientific publication

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Family guide

MDA Helping Jerry's Kids

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

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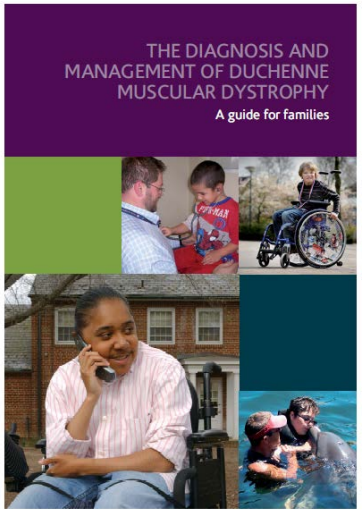

CARE-NMD

Care recommendations for DMD

Consensus process

↓

Scientific publication

↓

Family guide

?

? Care providers ?

Patients/Families

MDA Helping Jerry's Kids | Parent Project Muscular Dystrophy – LEADING THE FIGHT TO END DUCHENNE | TREAT-NMD Neuromuscular Network – From 2007 to 2011 inclusive, TREAT-NMD is a "network of excellence" supported through Priority 1 (Life Sciences, Genomics and Biotechnology for Health) of the European Union's Sixth Framework Programme. | United Parent Projects Muscular Dystrophy

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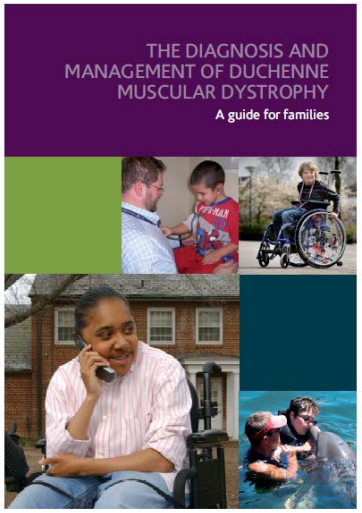


CARE-NMD

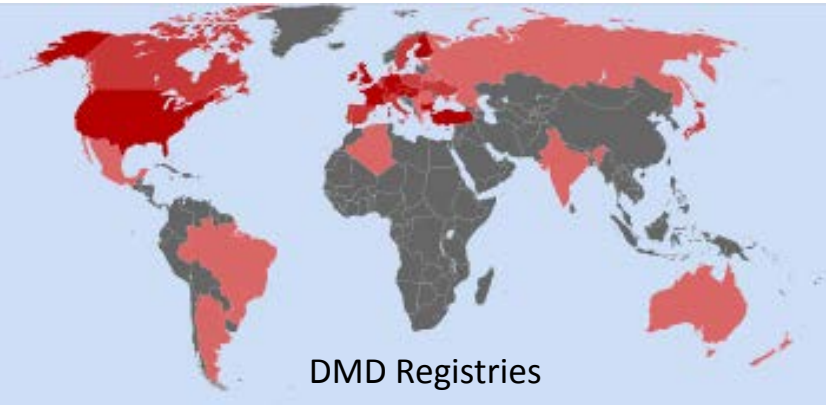
Current treatment situation

- Although guidelines are published many patients do not receive recommended treatment for many reasons
  - Treating health professionals are not trained adequately
  - No access to reference centers
  - No resources for treatment according to recommendation
- Quality of life and life expectancy varies significantly
- Few data available concerning health services for DMD

TREAT-NMD infrastructure

TREAT-NMD infrastructure

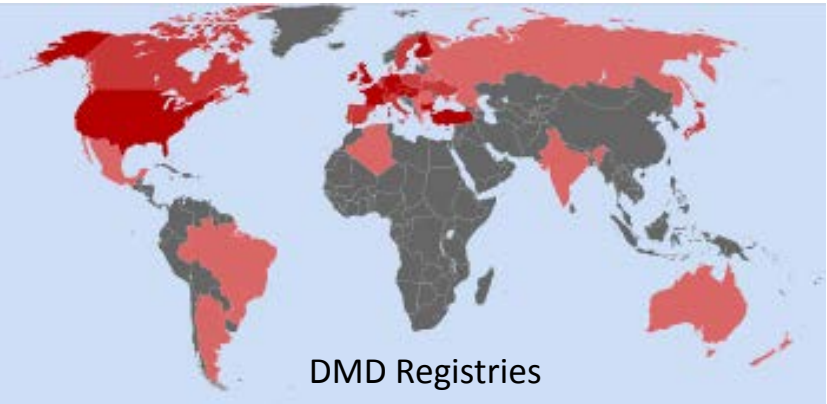
- Patient registries for DMD in more than 40 countries



CARE-NMD

TREAT-NMD infrastructure

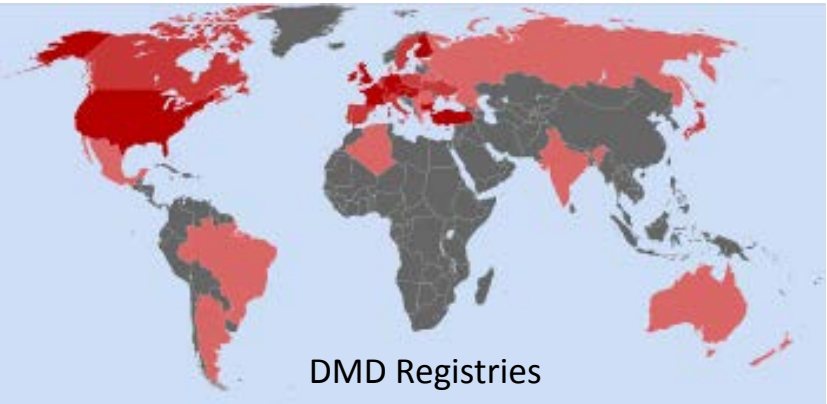
- Patient registries for DMD in more than 40 countries
- Care and Trial Site Registry with data on more than 250 neuromuscular centres with a worldwide distribution



CARE-NMD

TREAT-NMD infrastructure

- Patient registries for DMD in more than 40 countries
- Care and Trial Site Registry with data on more than 250 neuromuscular centres with a worldwide distribution
- Partners in most European countries

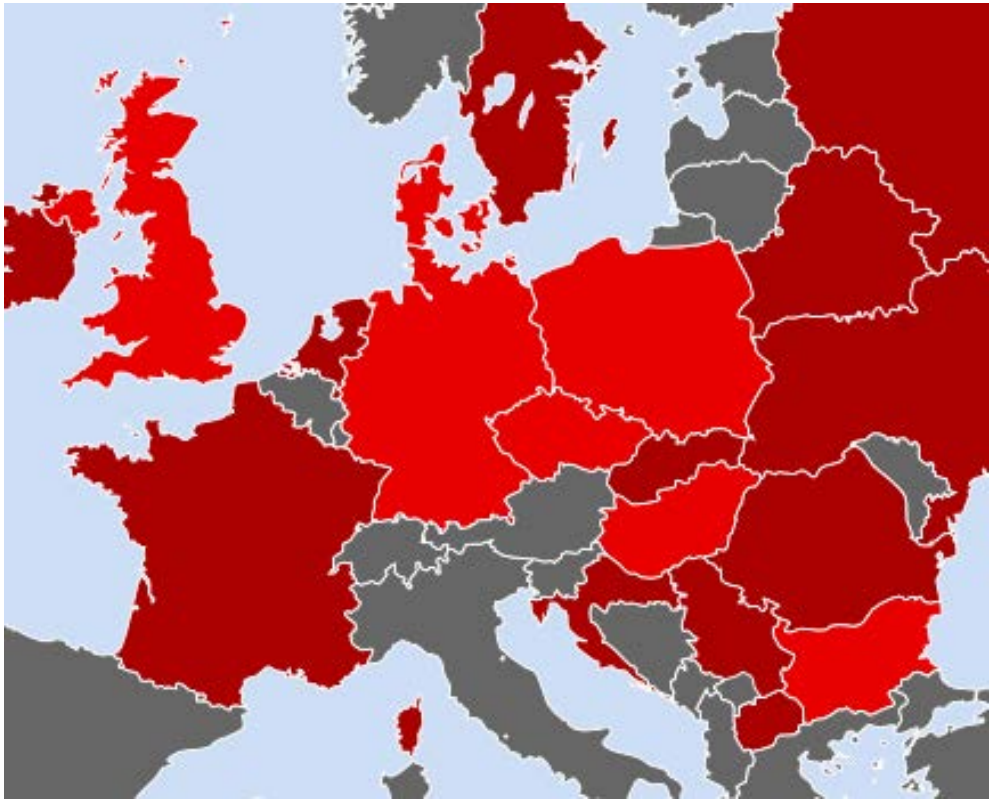


CARE-NMD

Objectives of CARE-NMD

- Evaluate current care practice across Europe
- Identify reasons for non-compliance with recommendations
  - Lack of knowledge/training
  - Professional attitude
  - Financial reasons
  - Social/ethical reasons
- Assess impact on quality of life of patients with DMD
- Dissemination and Training
- Establish a reference network of care centres

CARE-NMD partners



University Medical Center
Freiburg, Germany

University of Newcastle,
United Kingdom

Sofia Medical University, Bulgaria

University Hospital Brno,
Czech Republic

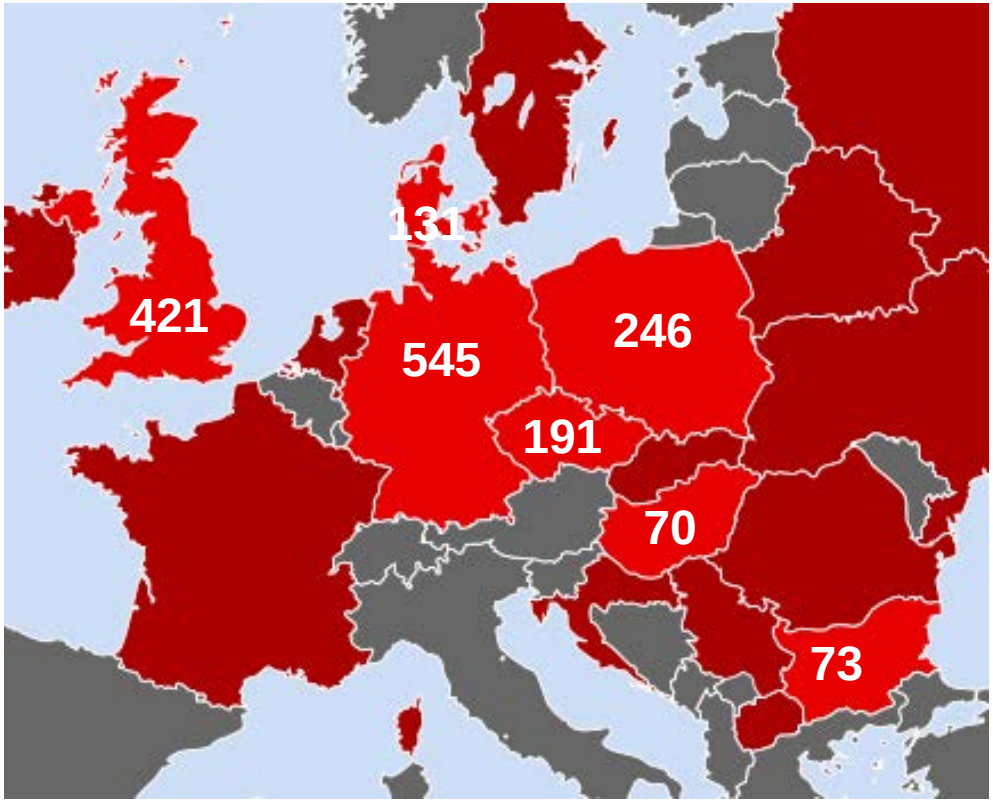
Hungarian Institute of
Environmental Health, Hungary

Medical University of Warsaw,
Poland

The National Danish
Rehabilitation Centre for
Neuromuscular Diseases,
Denmark

CARE-NMD

CARE-NMD partners



Total of 1,677 DMD patients

University Medical Center Freiburg, Germany

University of Newcastle, United Kingdom

Sofia Medical University, Bulgaria

University Hospital Brno, Czech Republic

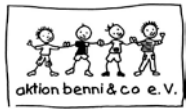
Hungarian Institute of Environmental Health, Hungary

Medical University of Warsaw, Poland

The National Danish Rehabilitation Centre for Neuromuscular Diseases, Denmark

CARE-NMD

Partners (associated and collaborating)



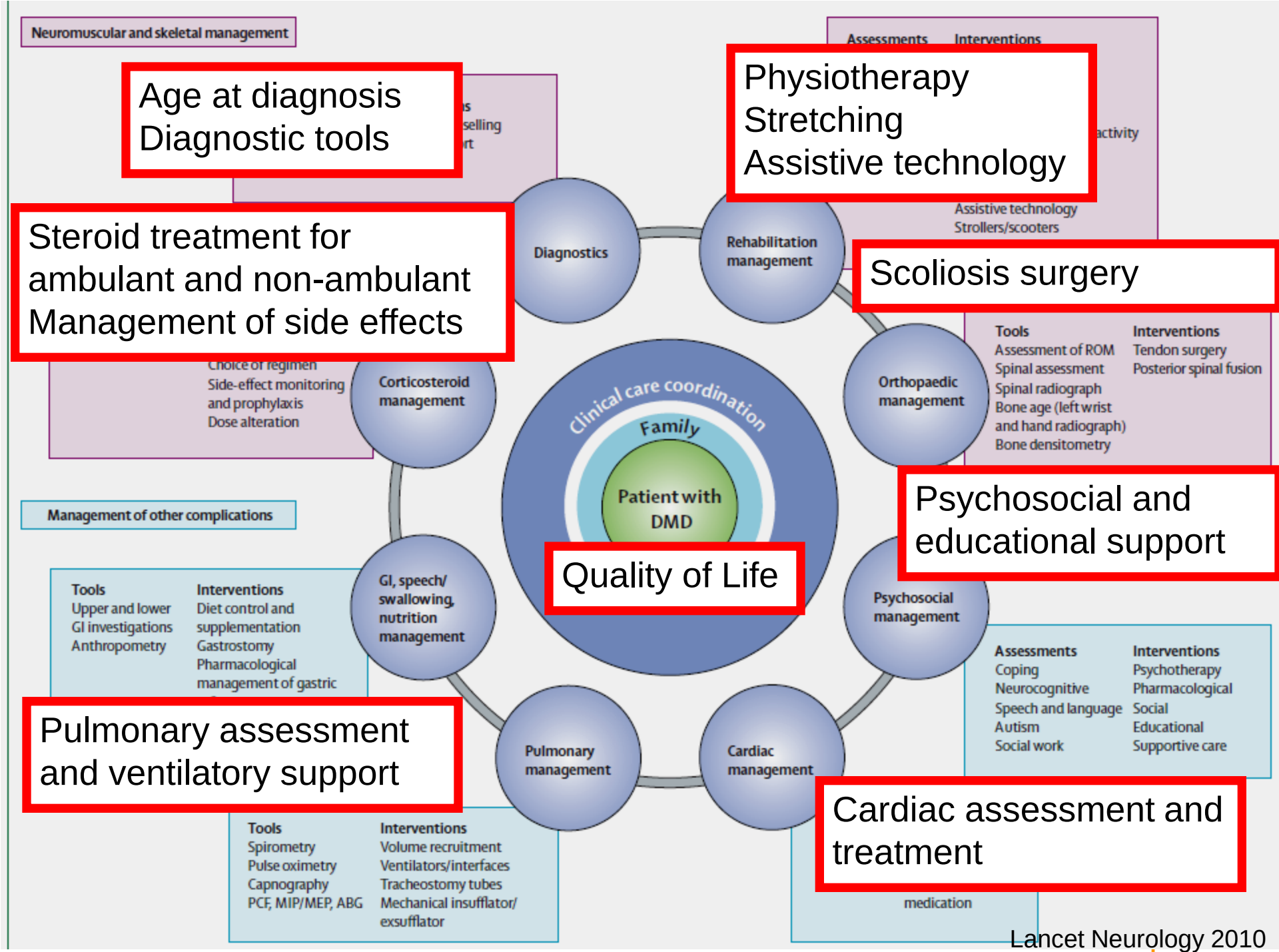
Evaluate Care Practice and QoL

- Questionnaires to all registered patients
  - 42 questions on key aspects of daily life and medical care
  - Online and paper version in all 7 languages
  - Current functional ability and disease stage
  - Education and participation in society
  - Outcome indicators (e.g. age at loss of ambulation, days in hospital)
  - Process indicators (e.g. frequency of cardiac/pulmonary assessment)

- Assess impact on Quality of Life
  - Standardized quality of life questionnaires
  - Child and parent versions for different age groups
  - PedQL, KIDScreen, SF36, WHO-QoL Bref

Patient survey: Topics

- Personal data
  - Age, education, occupation, household income
  - Participation in society, patient advocacy groups
- Functional status, activities of daily life, assistive devices
- Medical care
  - Neuromuscular center, frequency of assessment, hospital admissions
  - Diagnosis, Counselling about DMD
  - Use of steroids
  - Cardiac and respiratory treatment
- Satisfaction with medical treatment



Lancet Neurology 2010

Patient/Family questionnaire

16. How many days a week do you / does your son spend outside home or take part in activities outside your home (excluding during the winter months where the weather may be unsuitable)?	
1-2 days	☐
3-5 days	☐
6-7 days	☐
none	☐

Patient/Family questionnaire

24. Information about DMD				
Has a medical professional ever talked to you/your son about	**Yes, sufficiently**	**Yes, but not enough**	**No, not at all**	**I don't remember**
1. Genetic counselling?	☐	☐	☐	☐
2. The course of the disease and the main problems that may arise?	☐	☐	☐	☐
3. Treatment with steroids in DMD?	☐	☐	☐	☐
4. Breathing problems in the course of DMD?	☐	☐	☐	☐
5. Cardiac problems in DMD?	☐	☐	☐	☐
6. Prevention of curved spine (scoliosis) and joint contractures in the course of DMD?	☐	☐	☐	☐

CARE-NMD

Patient/Family questionnaire

30. Are you / is your son on steroid treatment (prednisone/prednisolone or deflazacort) for DMD?	
Yes	☐
No, I have never taken them as a treatment for Duchenne.	☐
No, I have stopped.	☐

31. If you have/ your son has never taken steroids, why is this?	
They are too expensive.	☐
They are not available in our region/country.	☐
They were not proposed by the doctor.	☐
We didn't want to take steroids.	☐
Not applicable, I am taking/have taken steroids.	☐

CARE-NMD

Patient/Family questionnaire

	GER	HU	CR	DK	UK	BG	PL	Total
Registered patients	545	70	191	131	421	73	246	1677
Total responses	424	57	90	78	228	40	142	1071
Response Rate	78%	81%	47%	60%	54%	55%	58%	64%

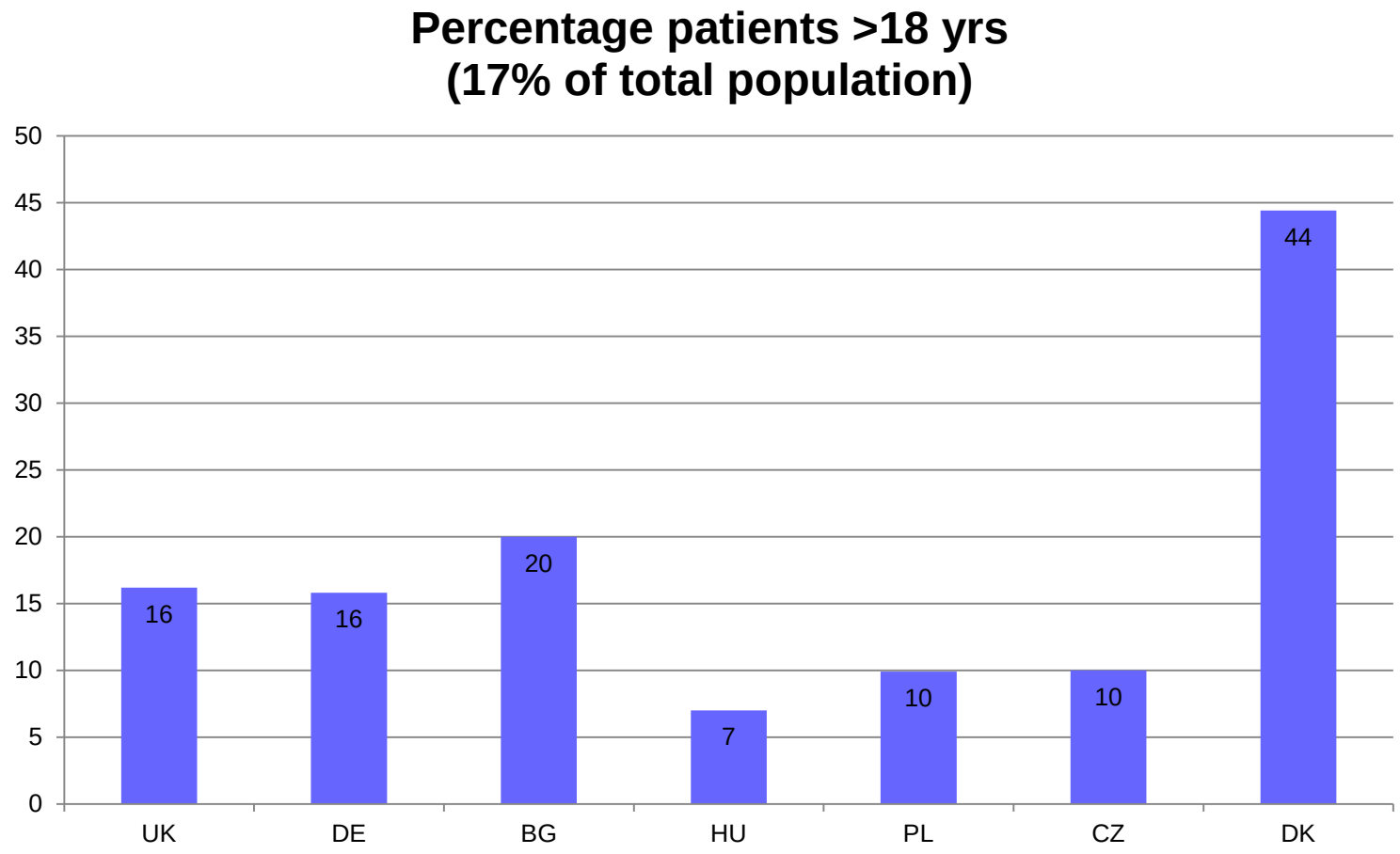
Final response rates

CARE-NMD

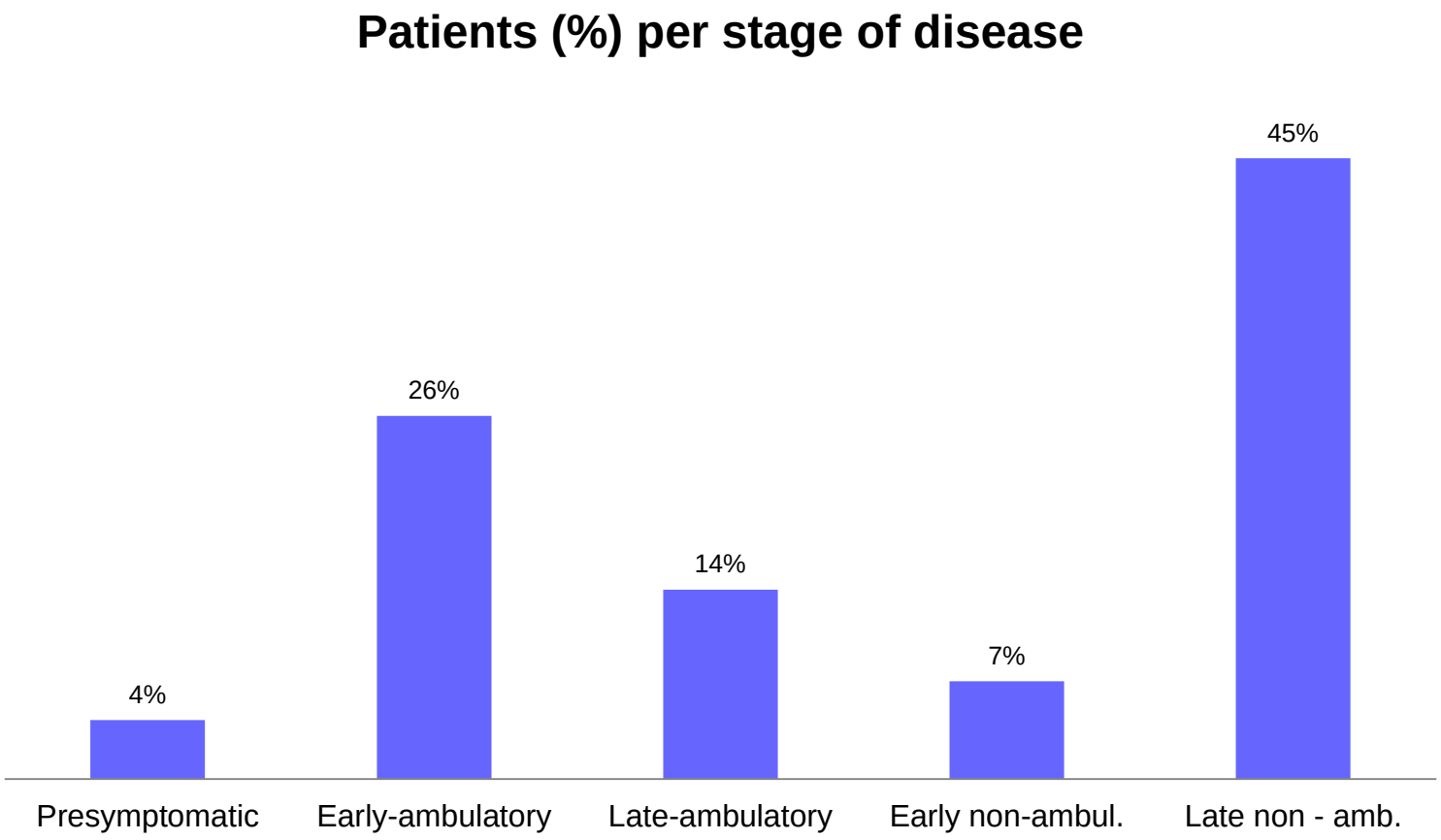
Patient survey: Cohort

- The mean patient age was 13.1 yrs (range 0.8-46.2)
- 17% of patients were aged >18 years
- 46 % of patients were ambulatory, 52 % were not

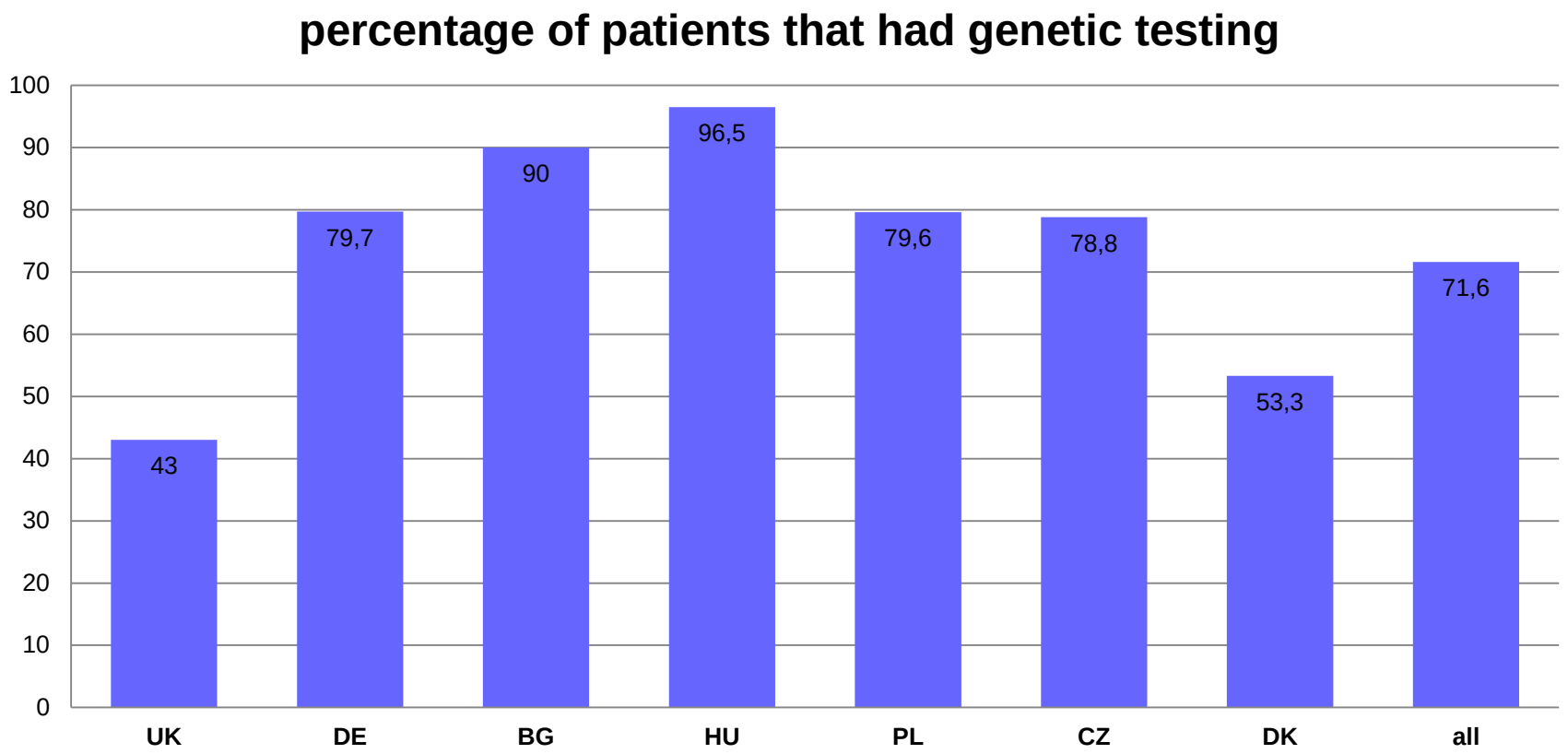
Patient survey: Cohort



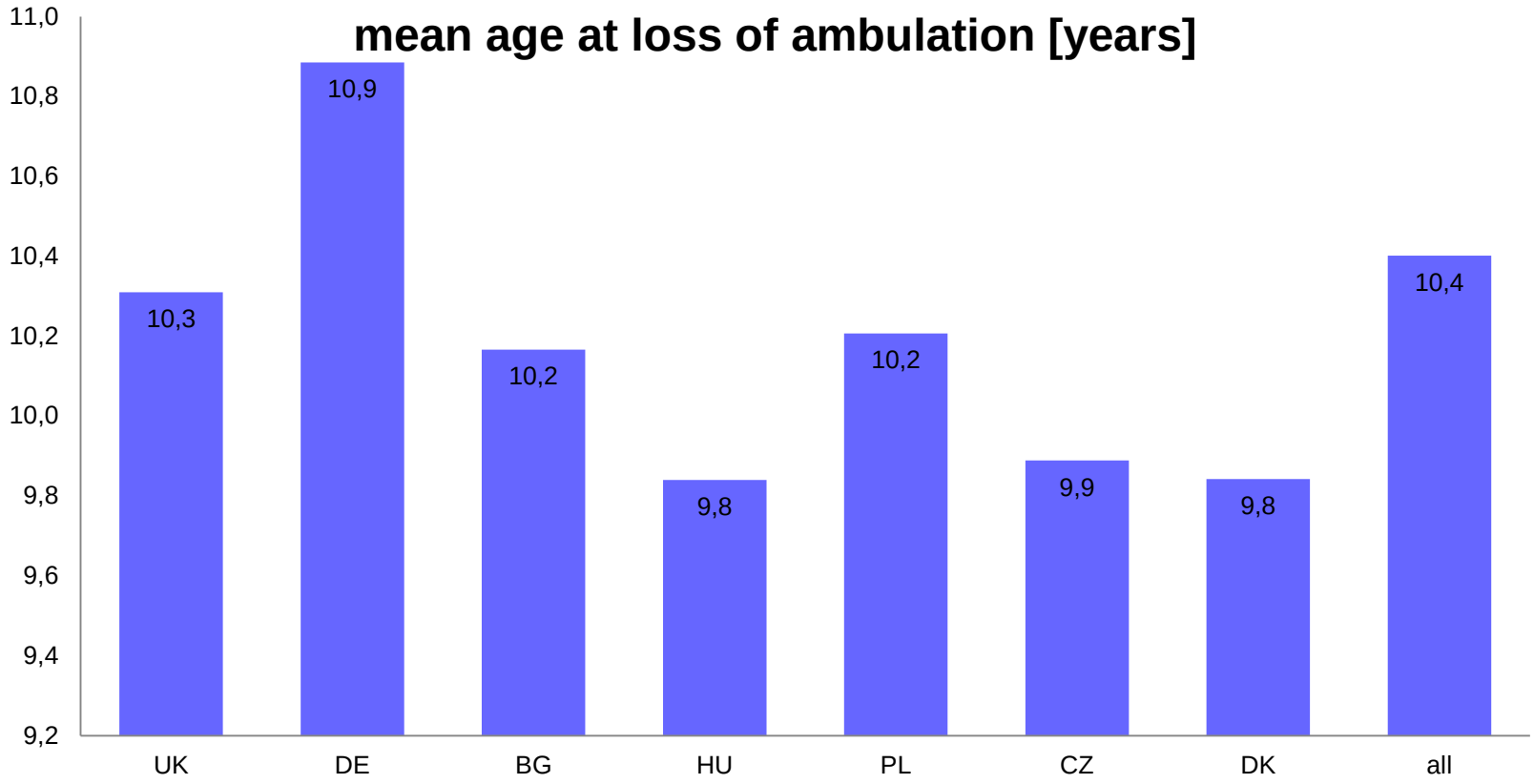
Patient survey: Cohort



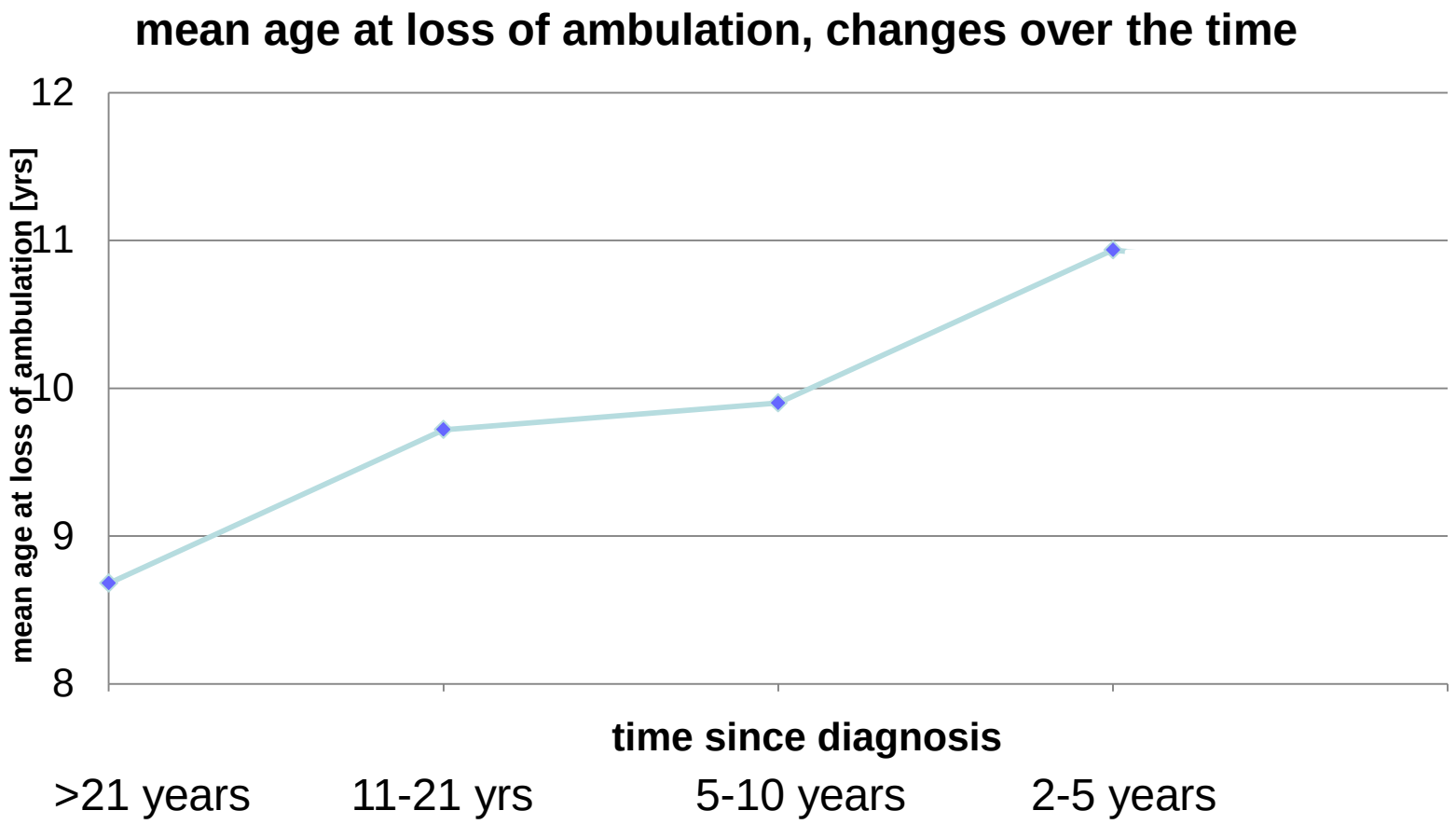
Patient survey: Cohort



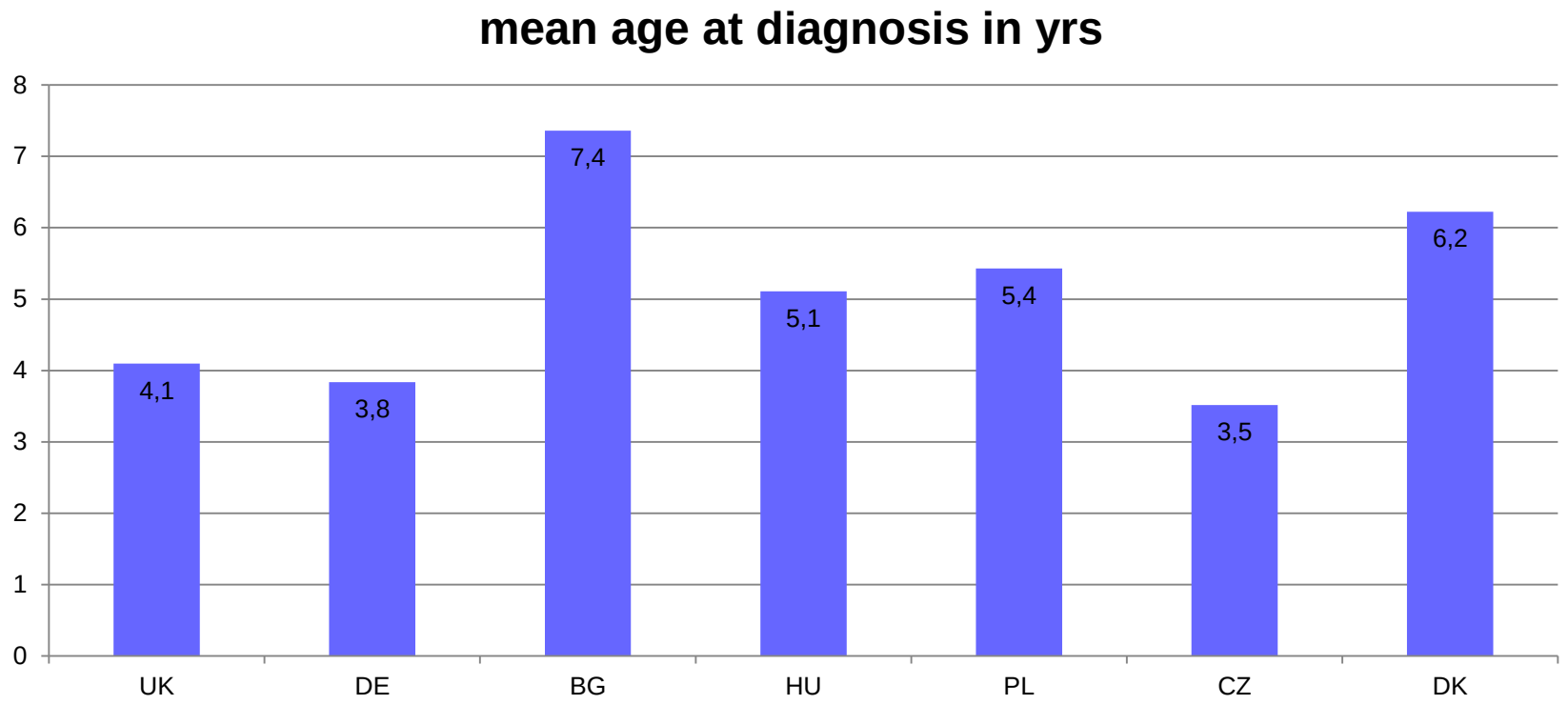
Outcome indicators



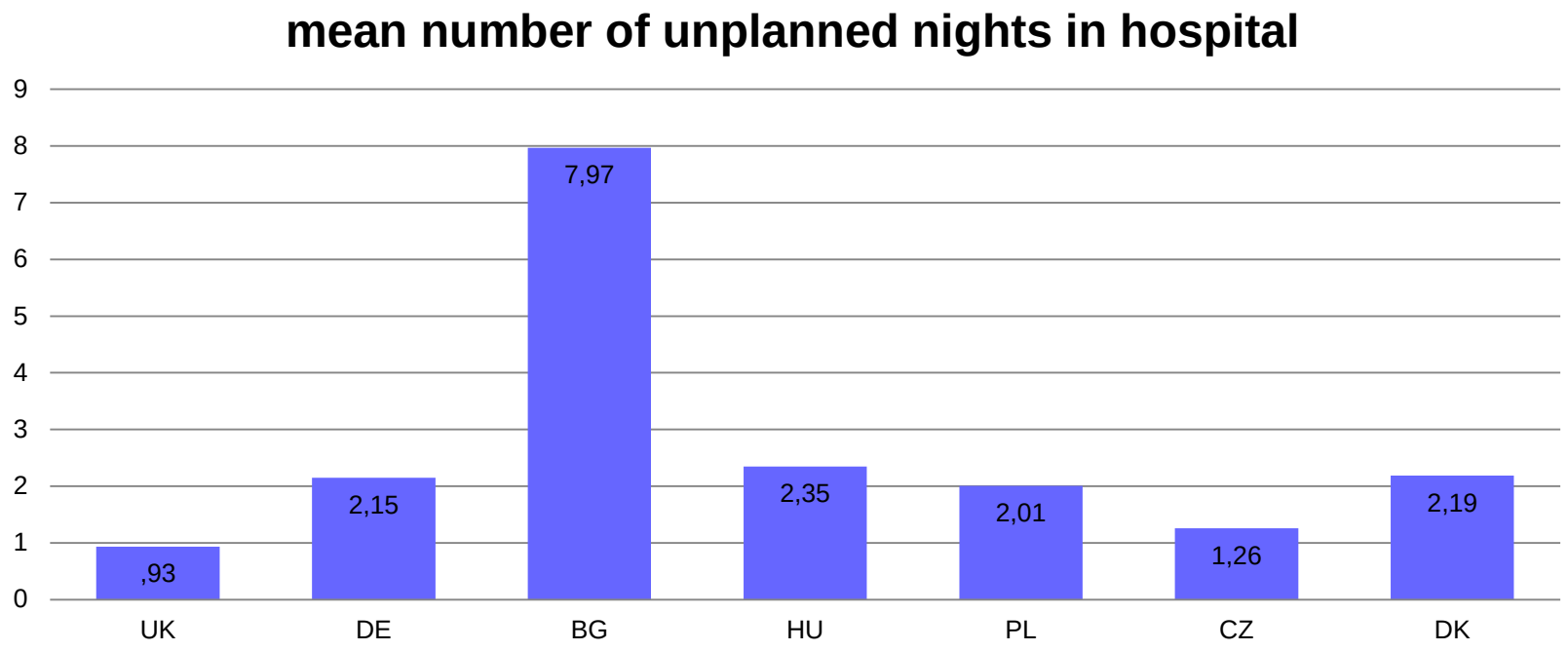
Outcome indicators



Outcome indicators



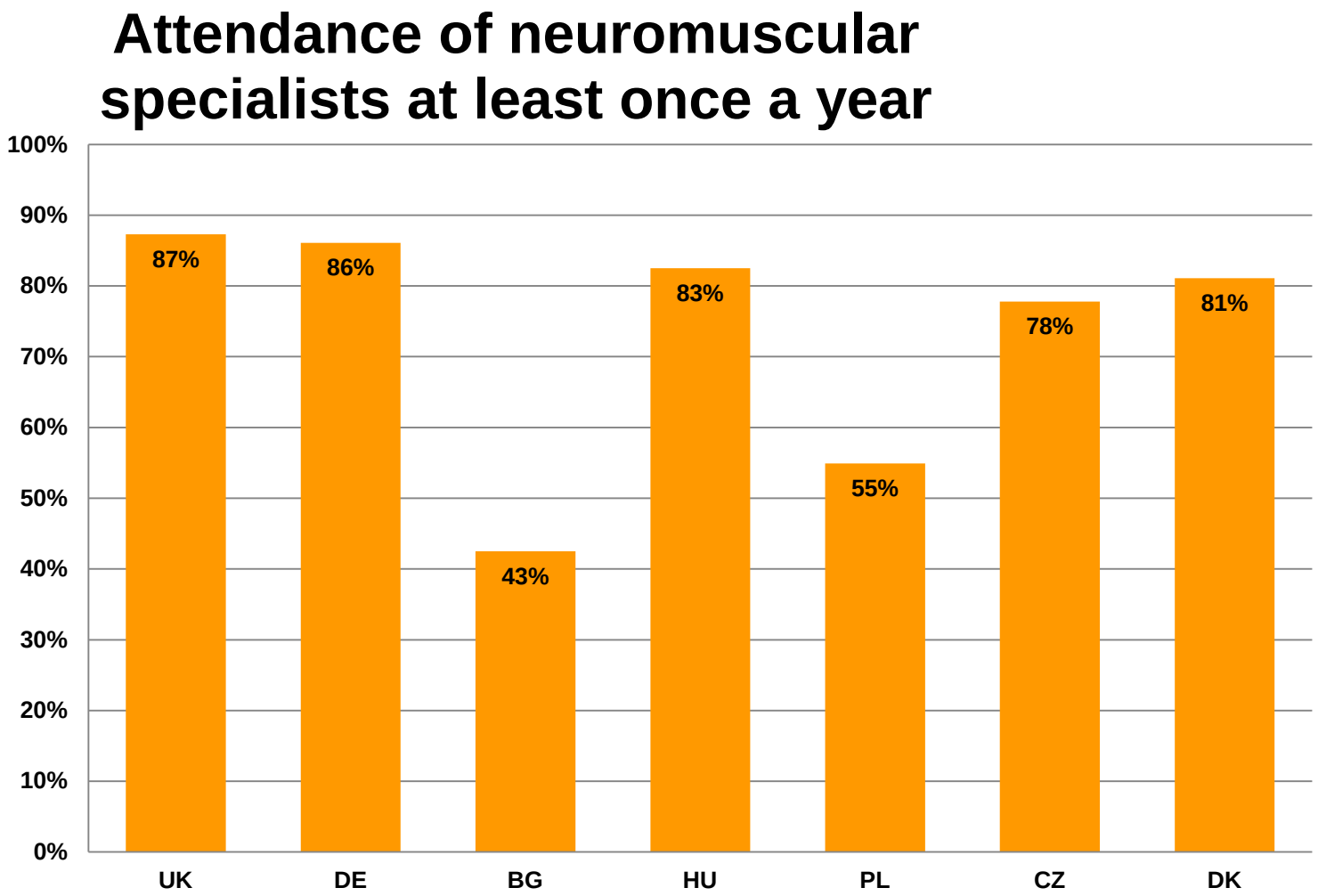
Outcome indicators



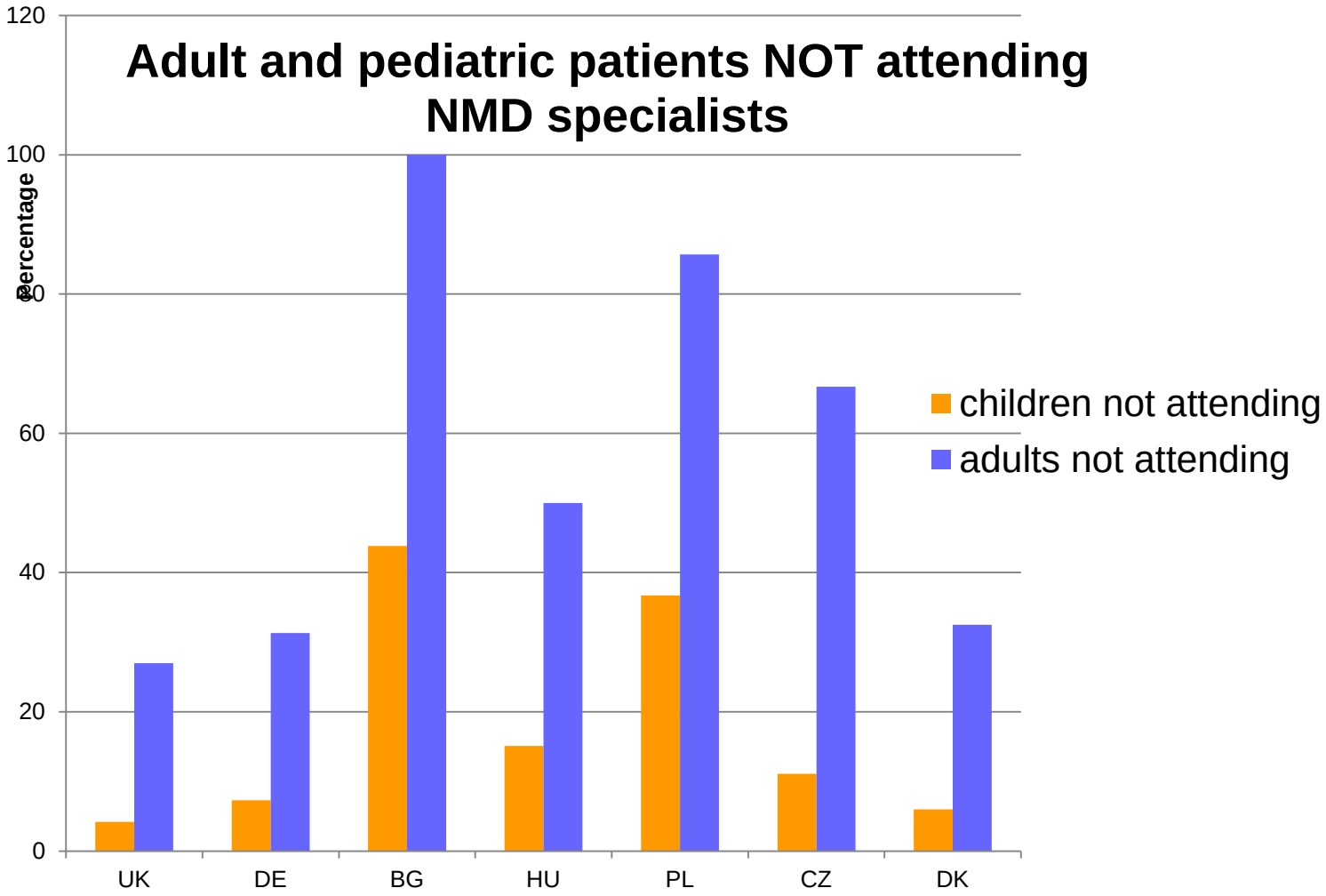
RESULTS

Neuromuscular Centers

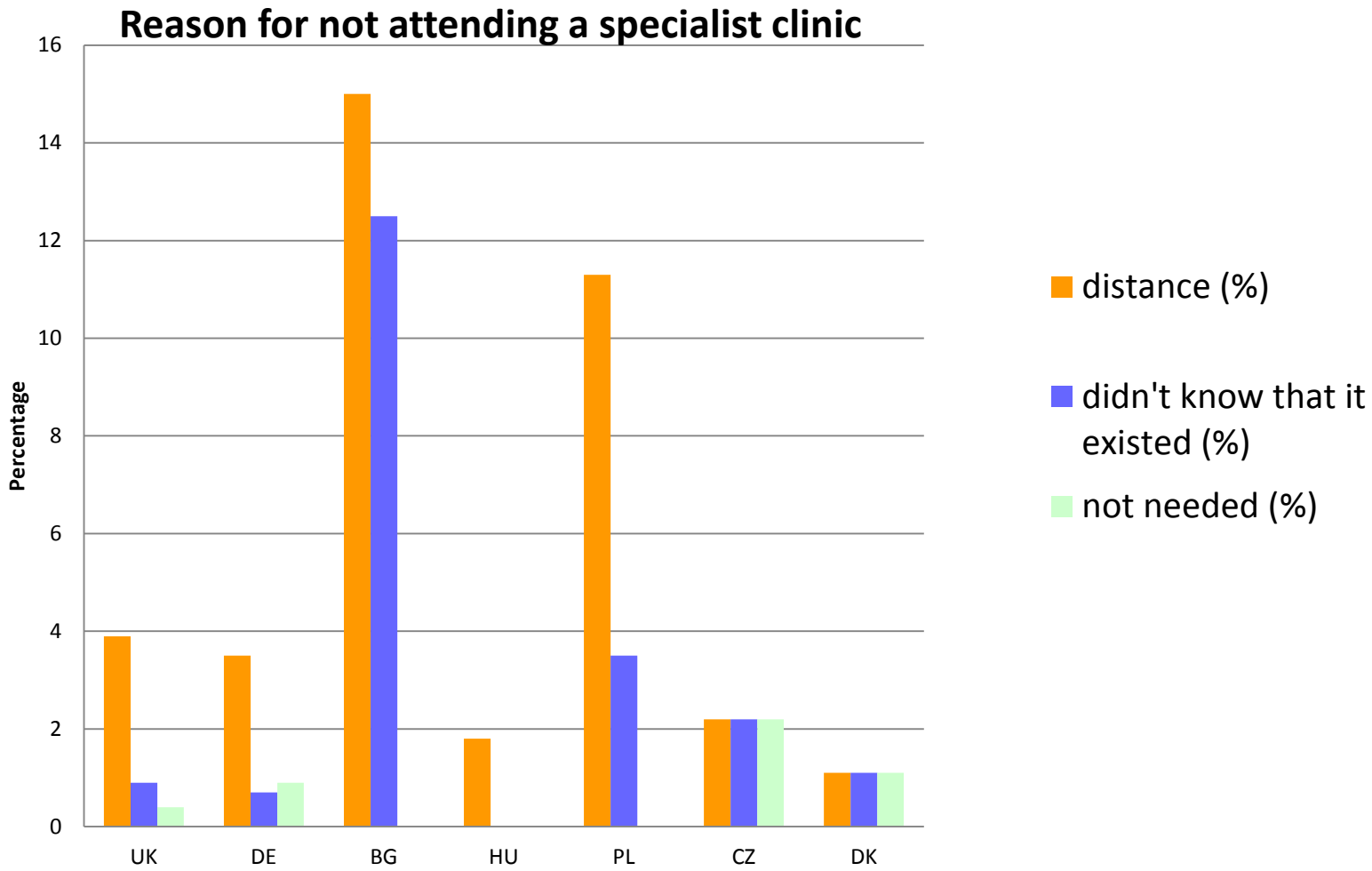
Attendance of Neuromuscular Center



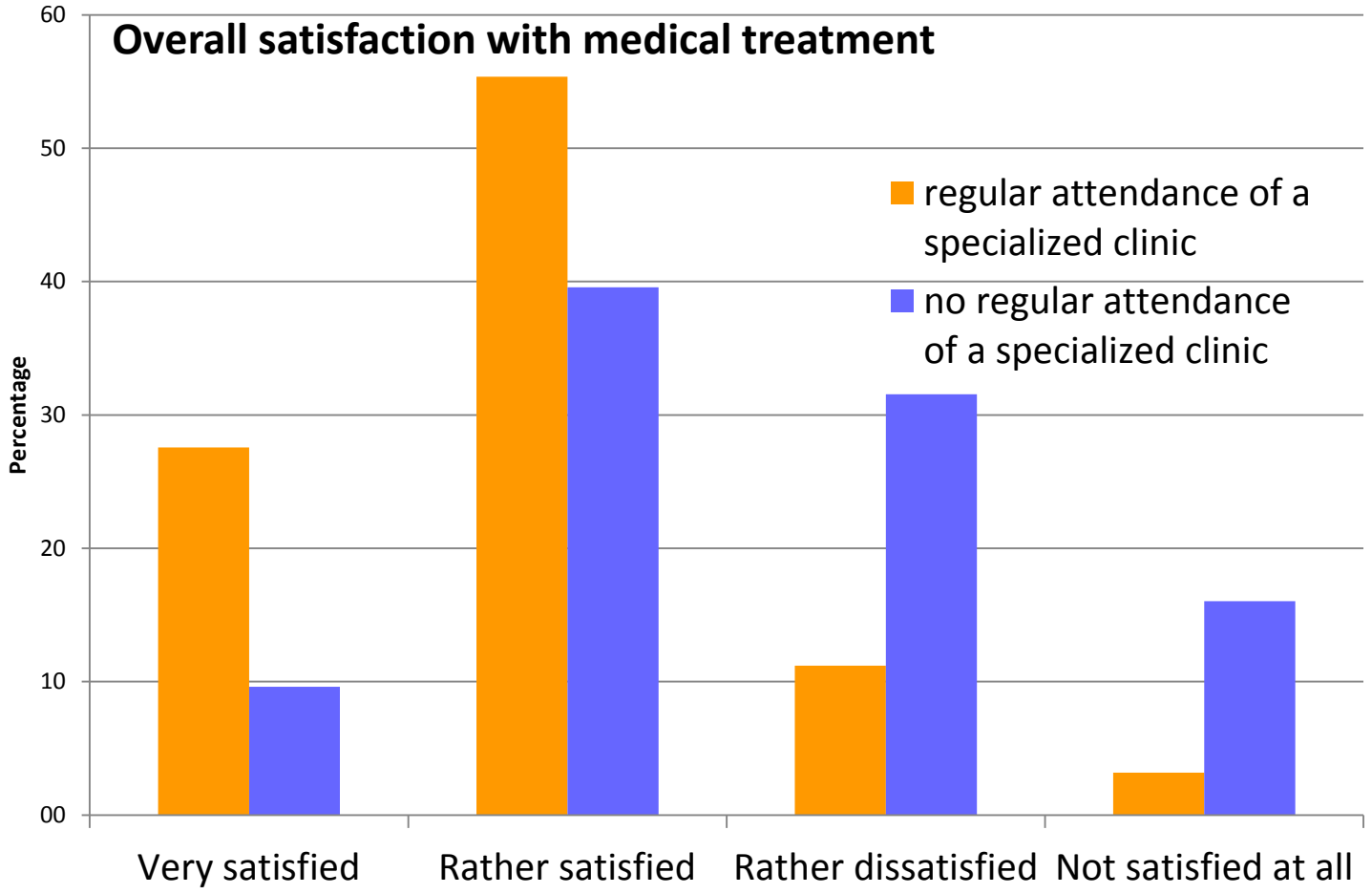
Attendance of Neuromuscular Center



Attendance of Neuromuscular Center



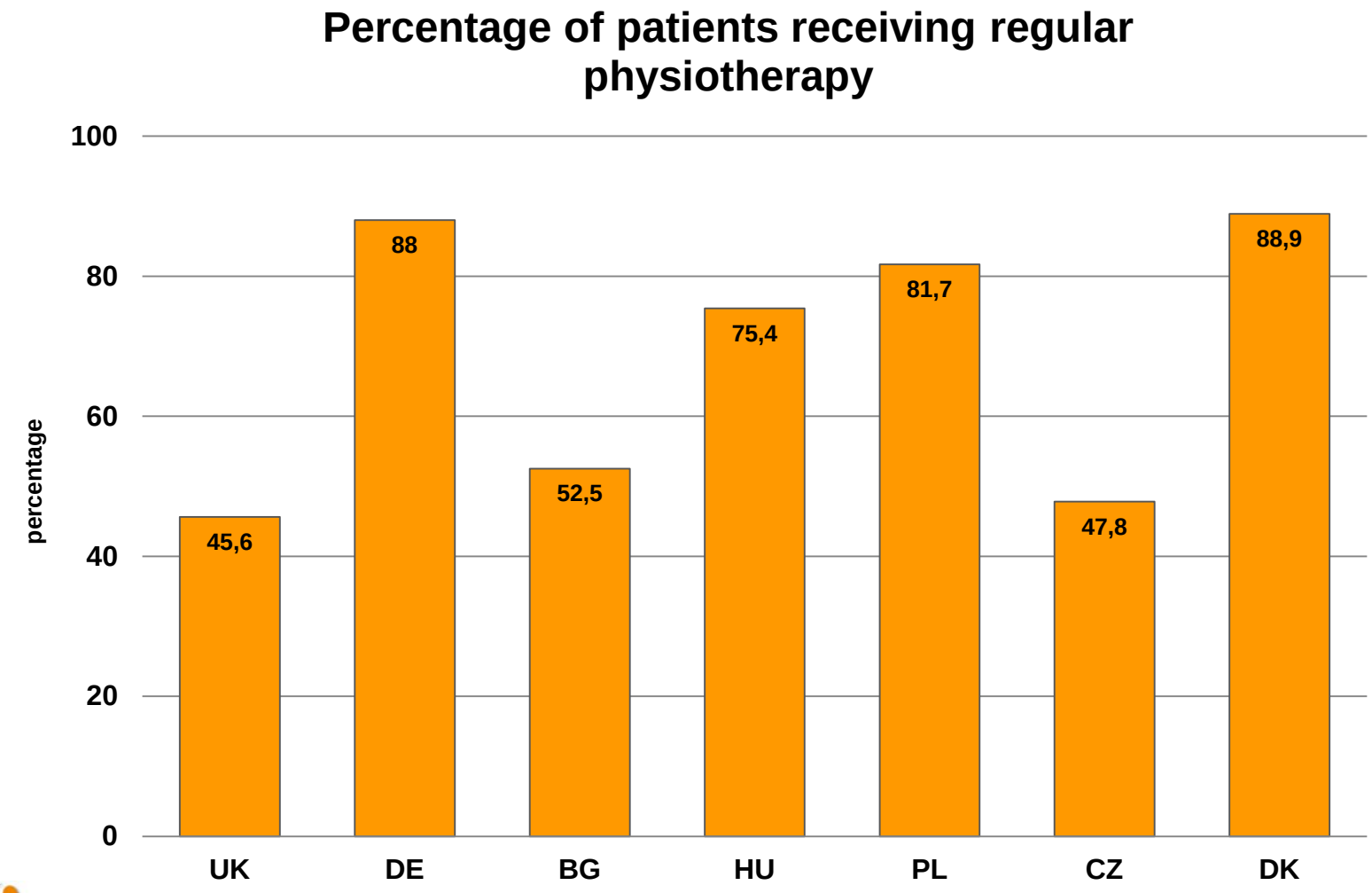
Attendance of Neuromuscular Center



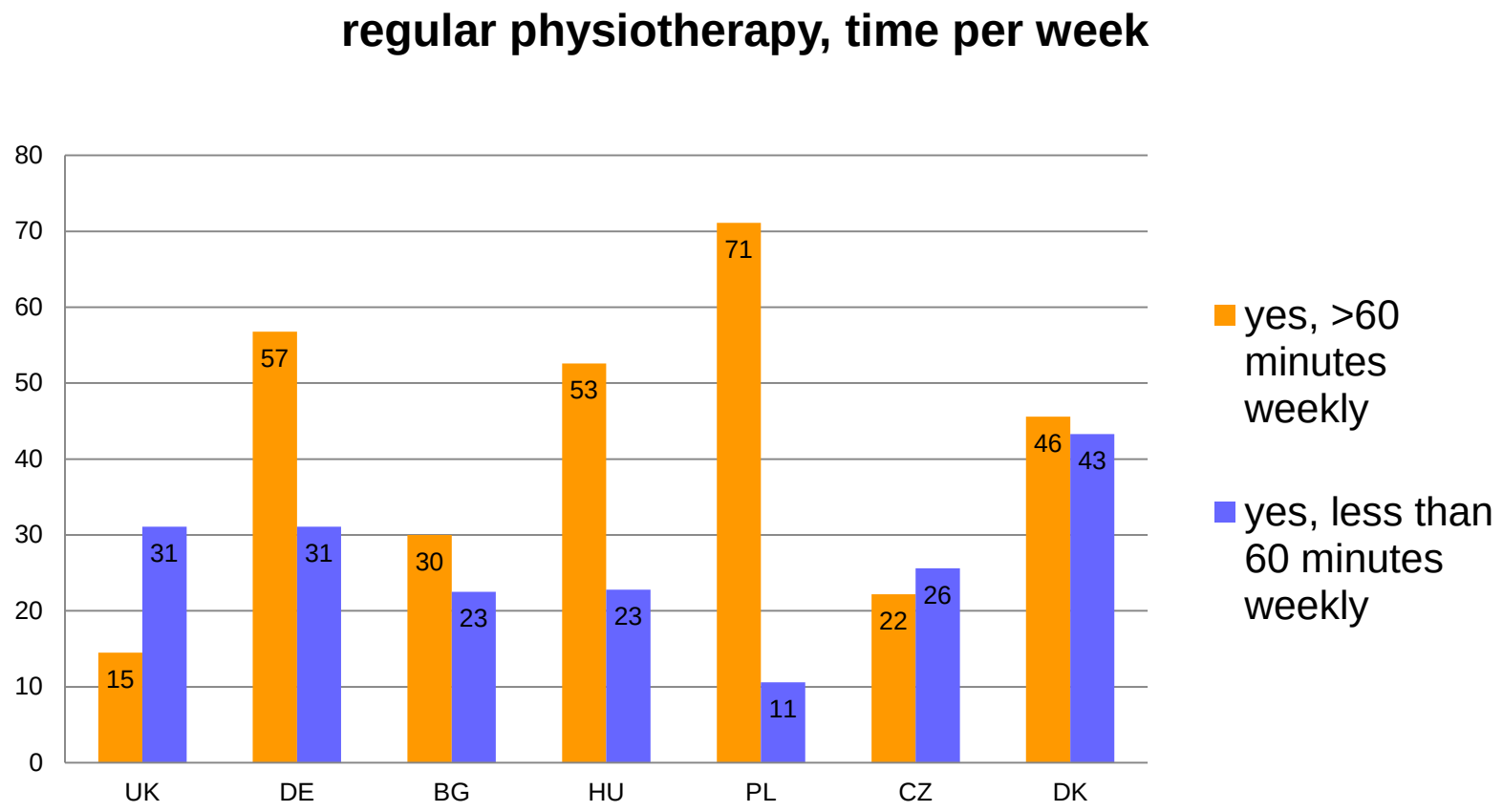
RESULTS

Physiotherapy

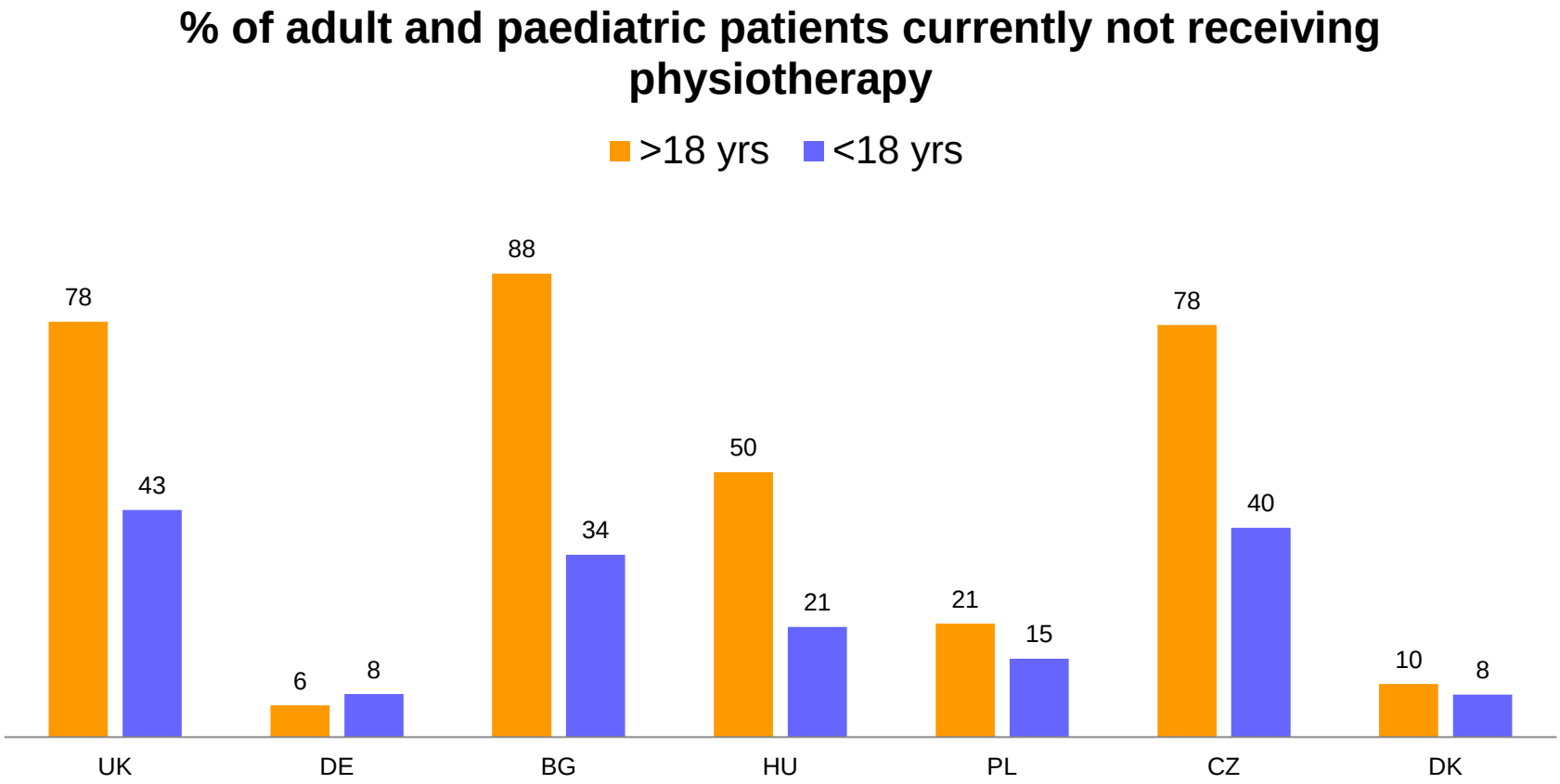
Physiotherapy



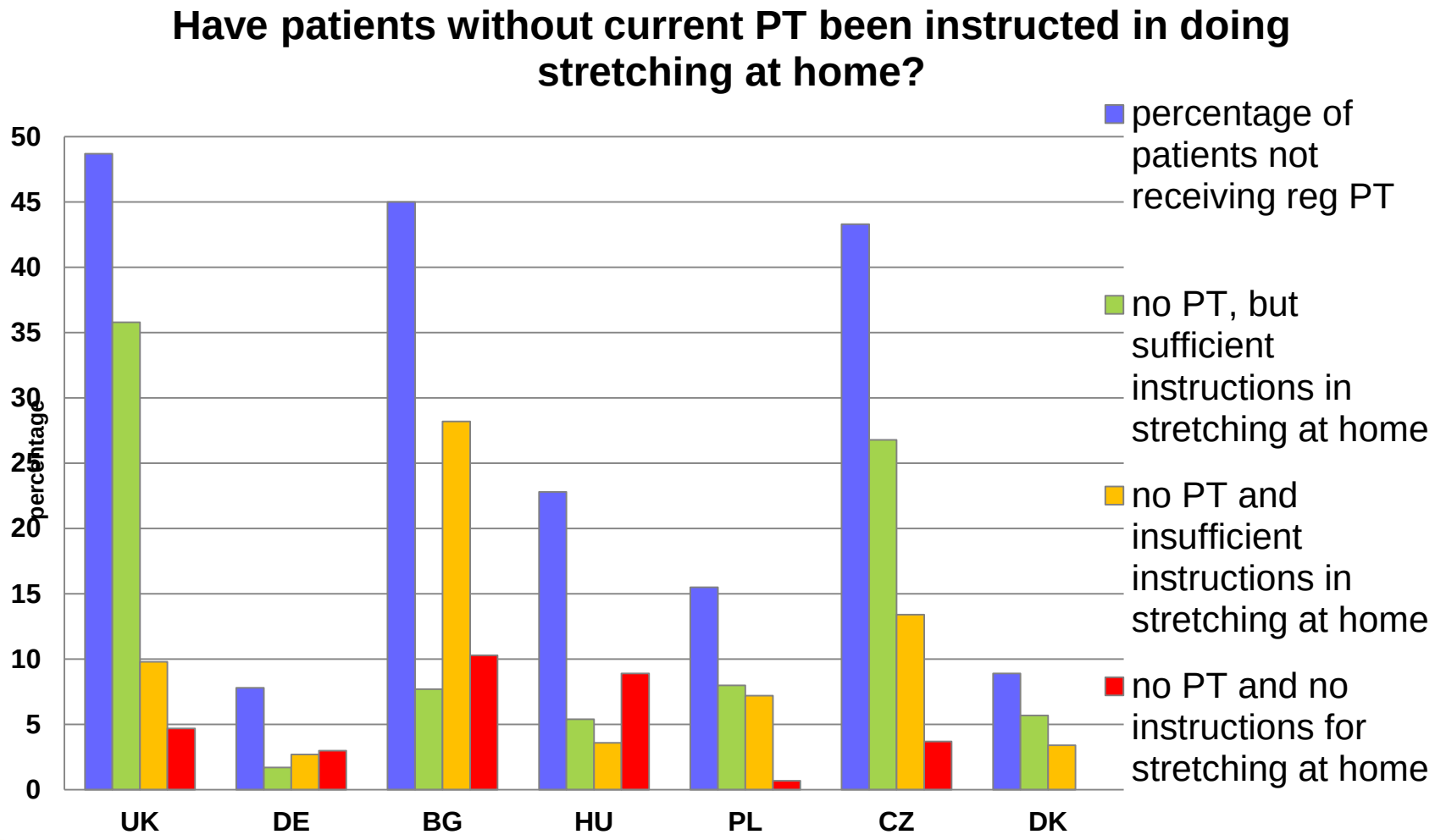
Physiotherapy



Physiotherapy



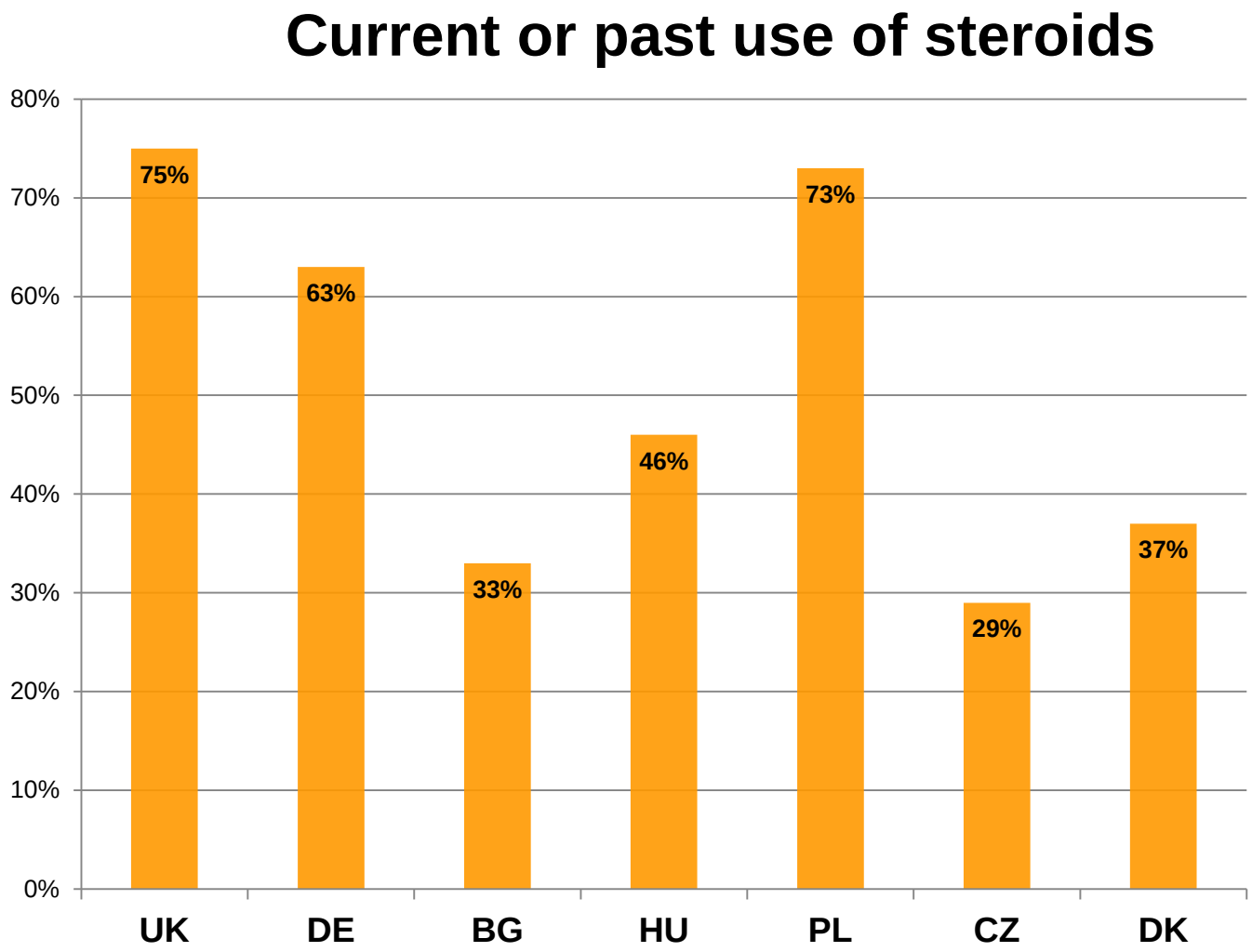
Physiotherapy



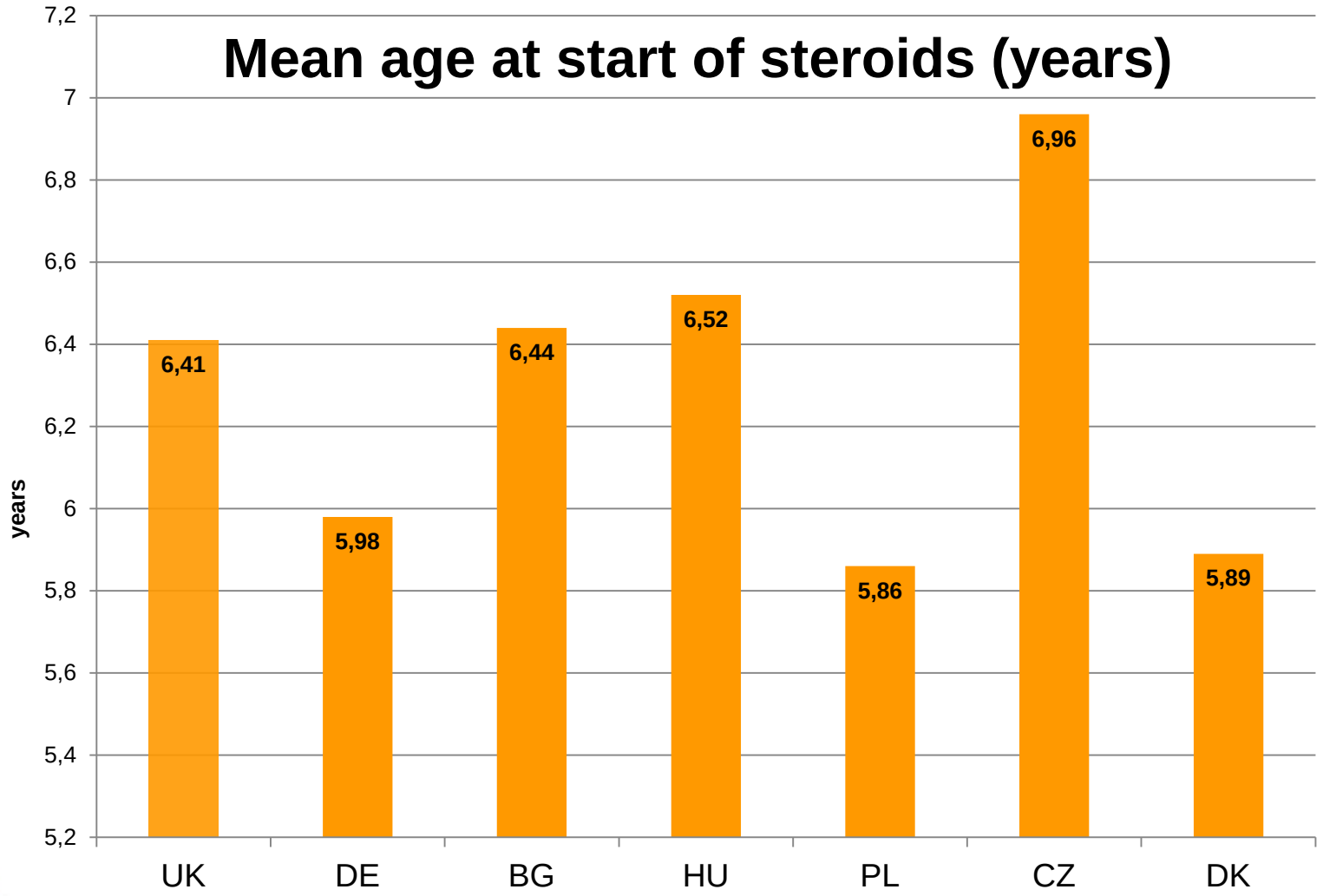
RESULTS

STEROIDS

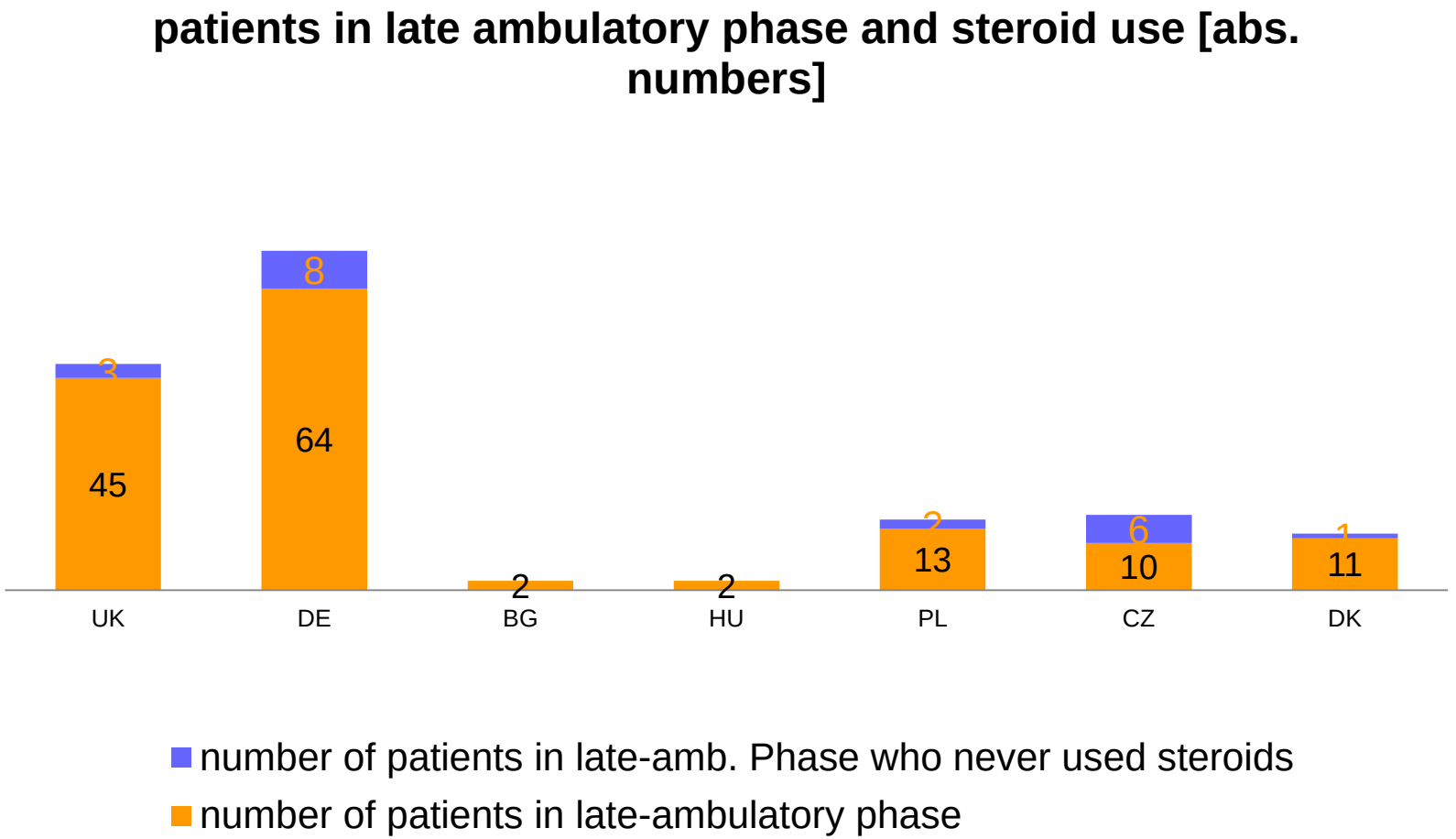
Steroid use


Current or past use of steroids
80%
70%
60%
50%
40%
30%
20%
10%
0%
75%
63%
33%
46%
73%
29%
37%
UK
DE
BG
HU
PL
CZ
DK

Steroid use

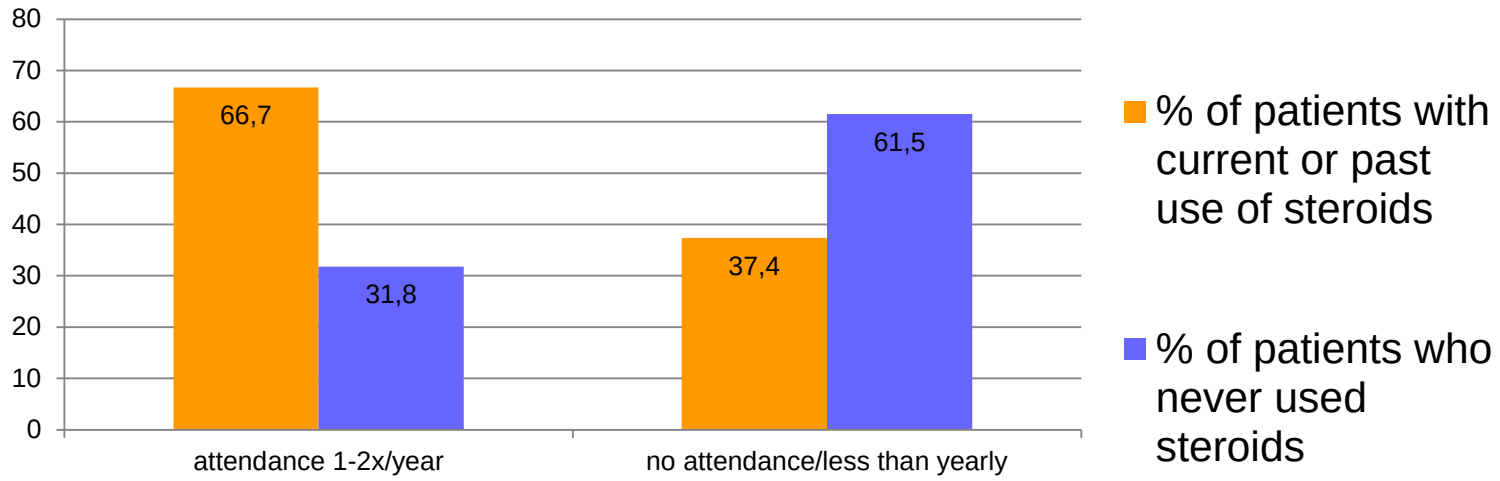

Mean age at start of steroids (years)
years
7,2
7
6,8
6,6
6,4
6,2
6
5,8
5,6
5,4
5,2
6,41
5,98
6,44
6,52
5,86
6,96
5,89
UK
DE
BG
HU
PL
CZ
DK

Steroid use

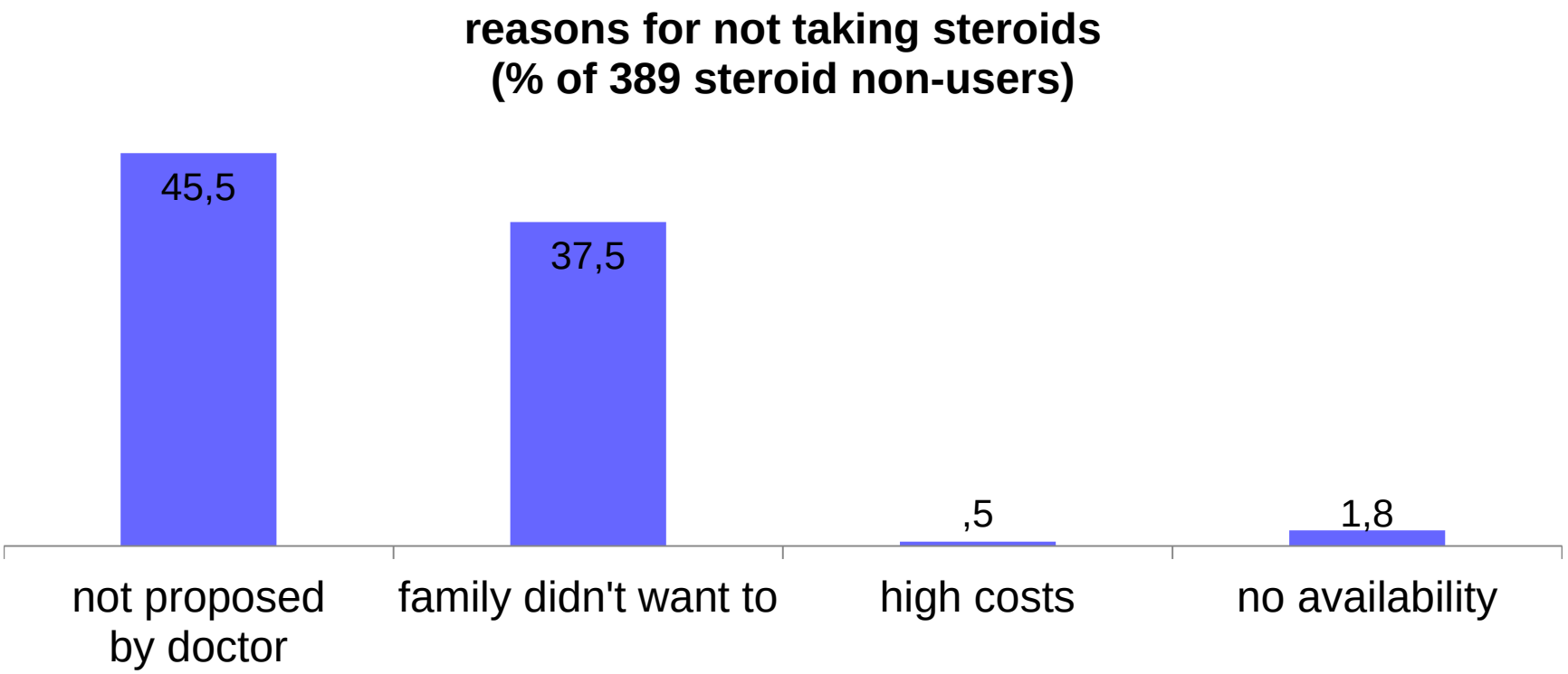

patients in late ambulatory phase and steroid use [abs. numbers]
3
45
8
64
2
2
2
13
6
10
1
11
UK
DE
BG
HU
PL
CZ
DK
number of patients in late-amb. Phase who never used steroids
number of patients in late-ambulatory phase

Steroid use

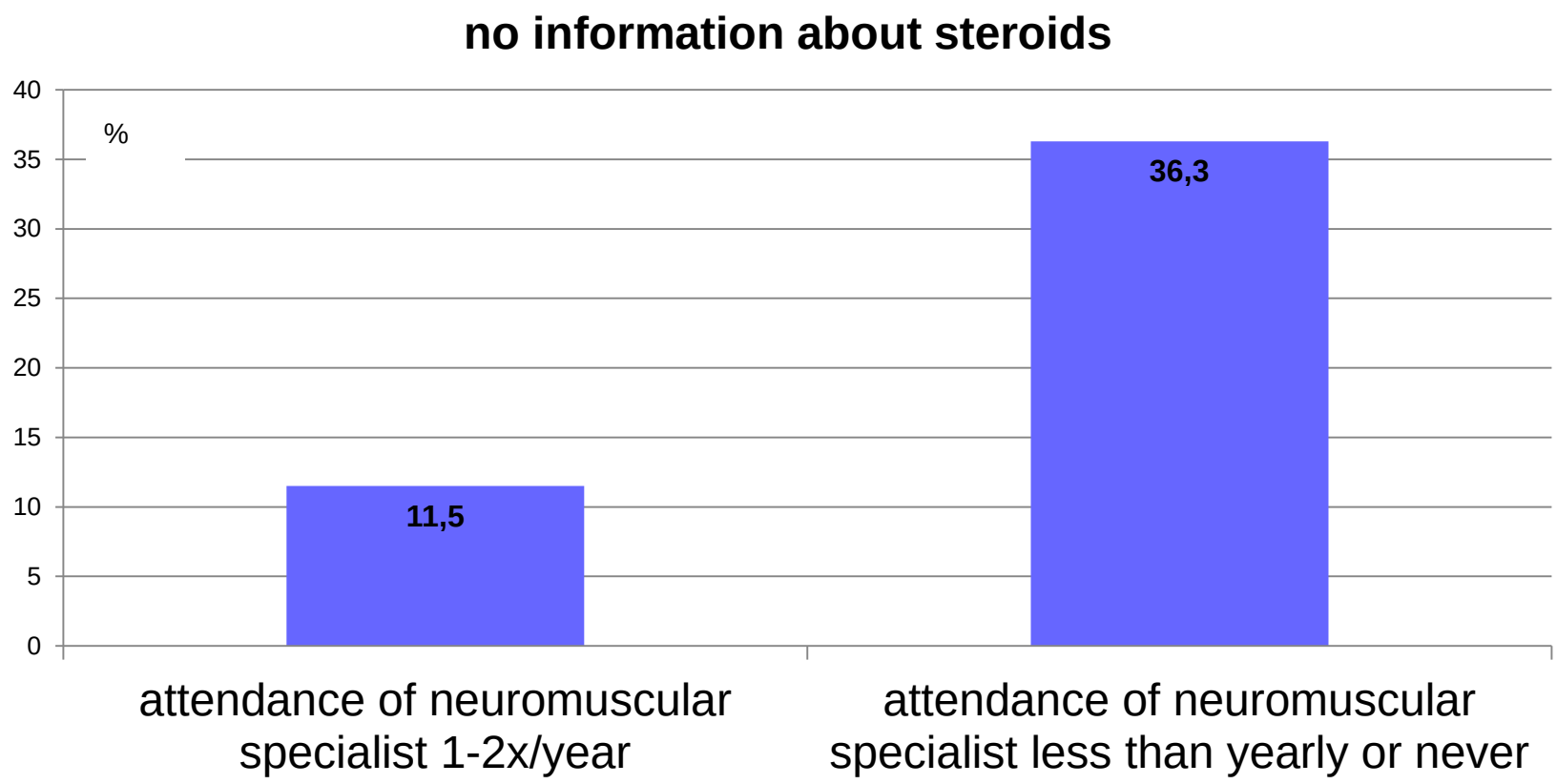
Attendance at neuromuscular centre and % of patients with or without steroid treatment



Steroid use

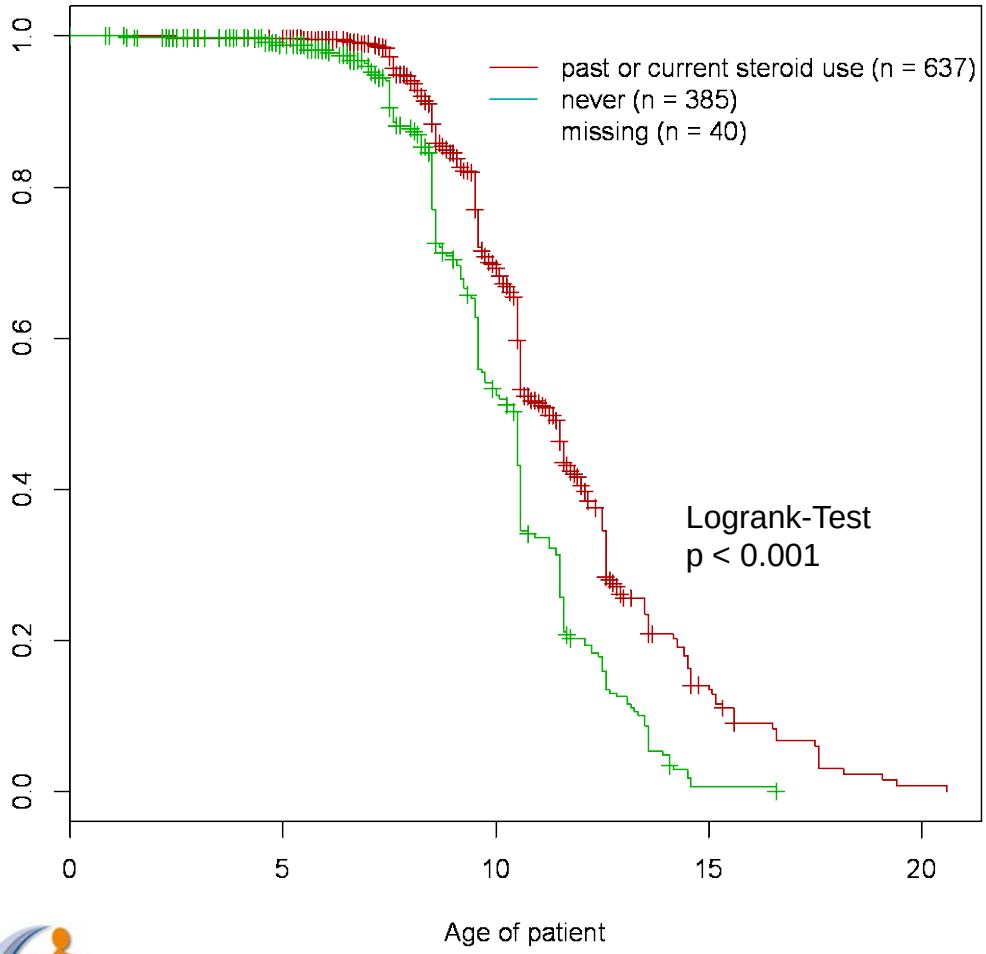

reasons for not taking steroids
(% of 389 steroid non-users)
45,5
37,5
,5
1,8
not proposed by doctor
family didn't want to
high costs
no availability

Steroid use


no information about steroids
%
40
35
30
25
20
15
10
5
0
11,5
36,3
attendance of neuromuscular specialist 1-2x/year
attendance of neuromuscular specialist less than yearly or never

Steroid use

Kaplan−Meier curves of time to loss of ambulation by steroid use



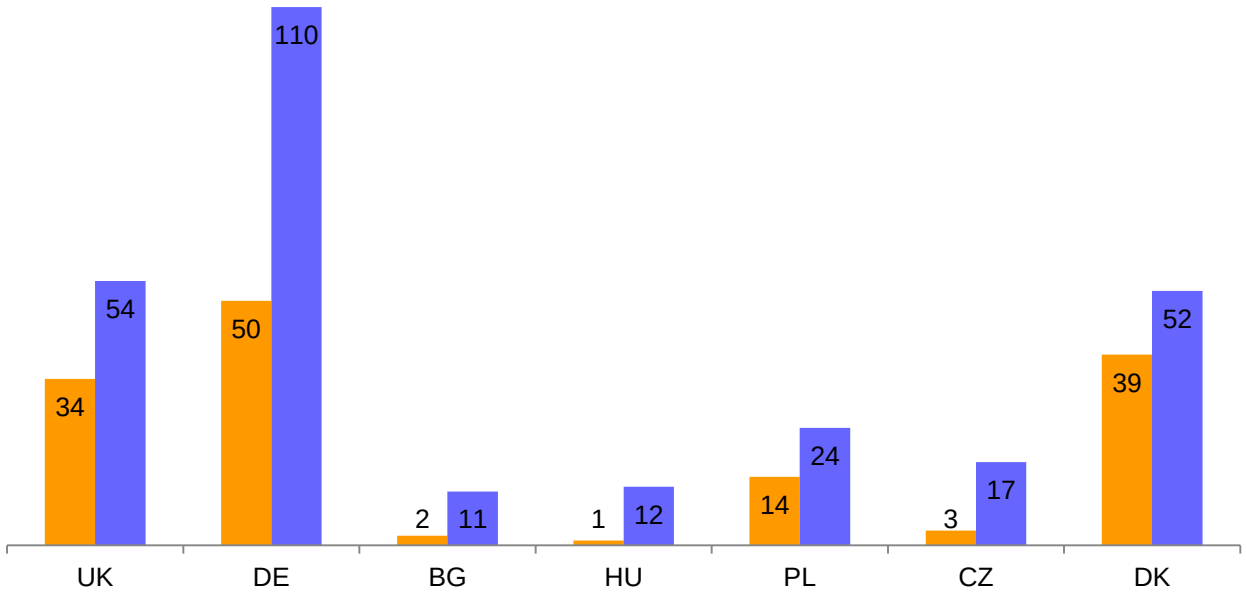
	mean age at loss of ambulation	SD	range
current or past use	10,65	2,52	2,5-20,6
steroids never used	10,05	2,16	1,3-16,6

RESULTS

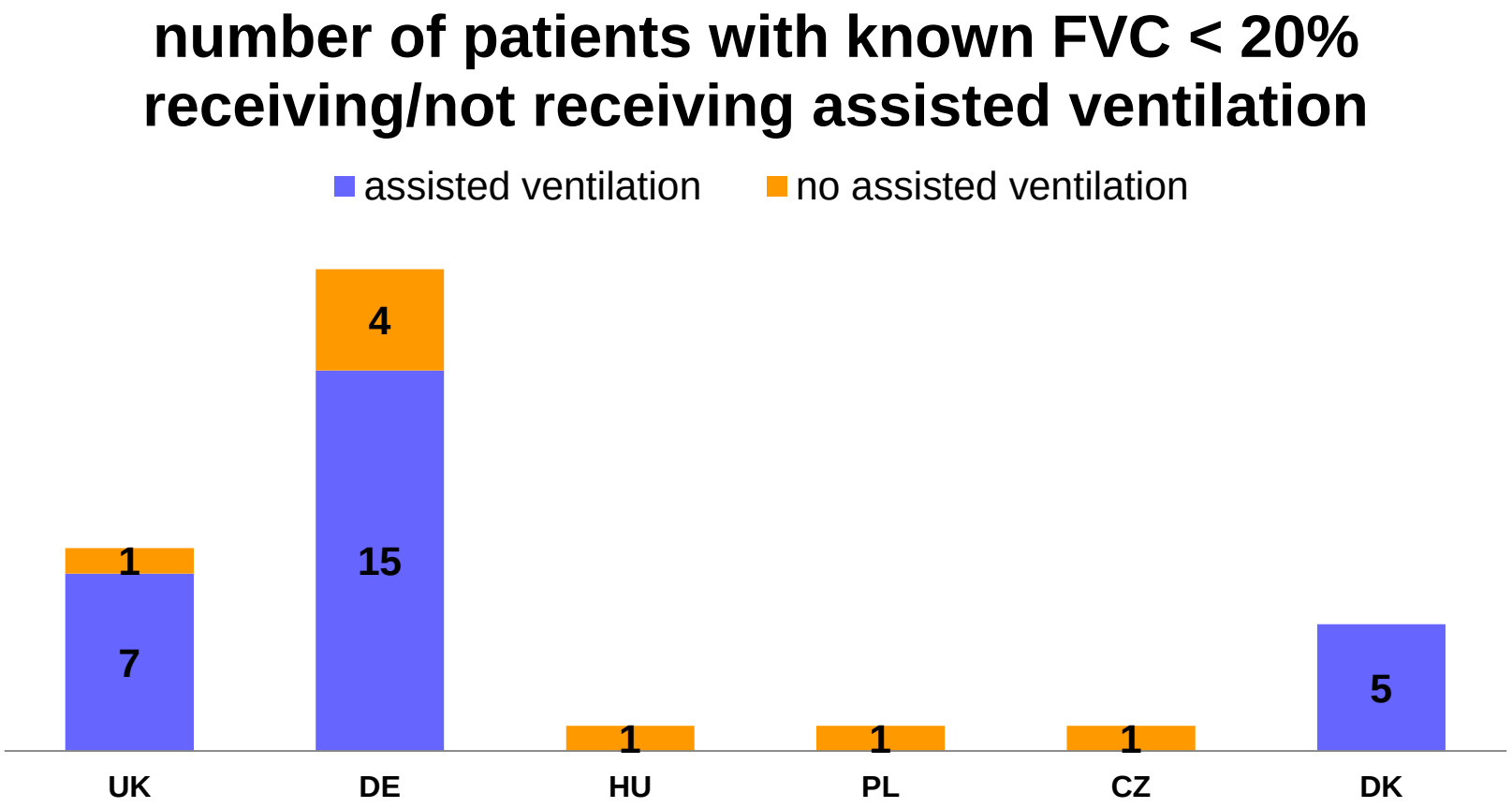
RESPIRATORY CARE

Respiratory care

number of patients aged ≥16 receiving any kind of ventilation



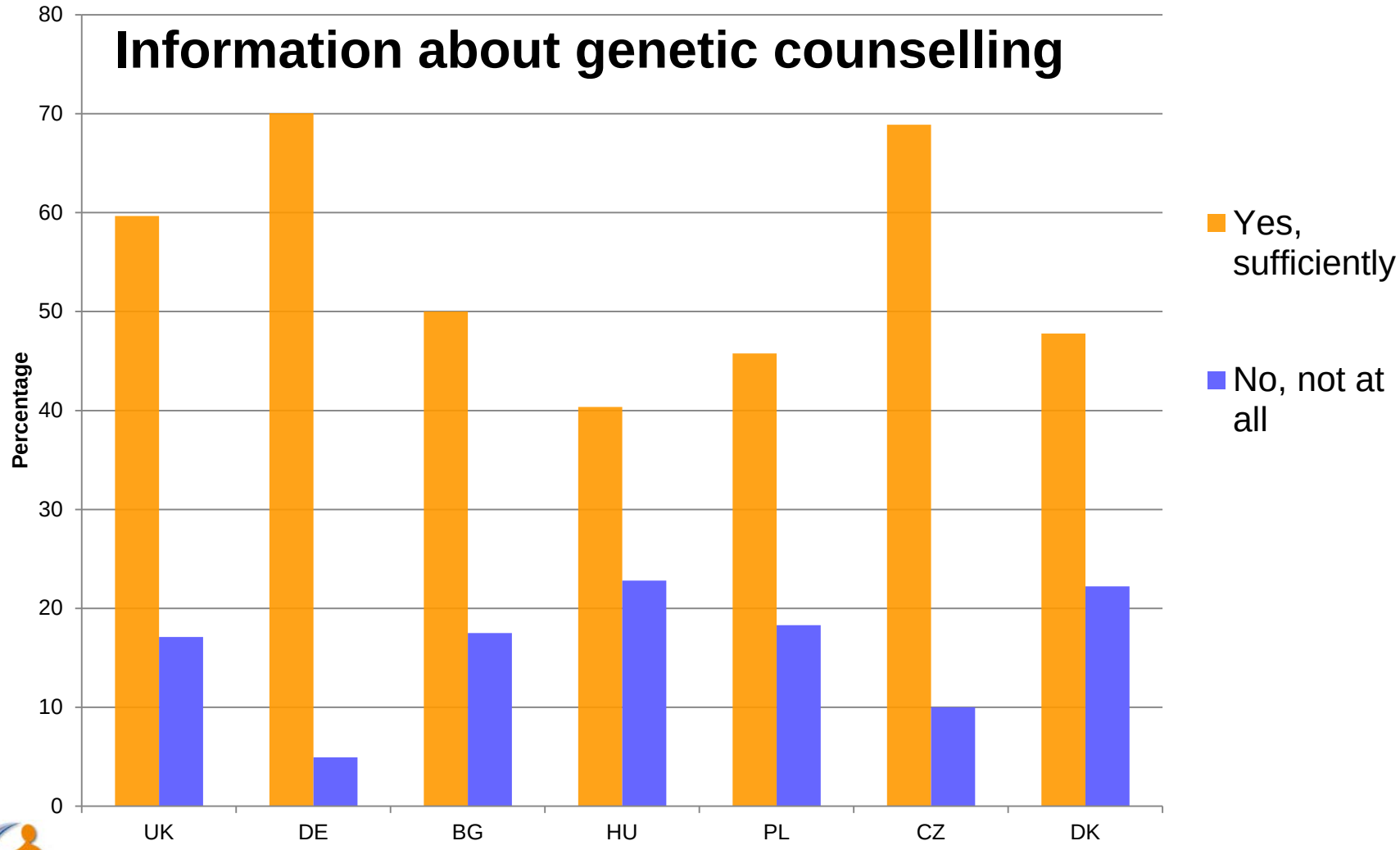
Respiratory care



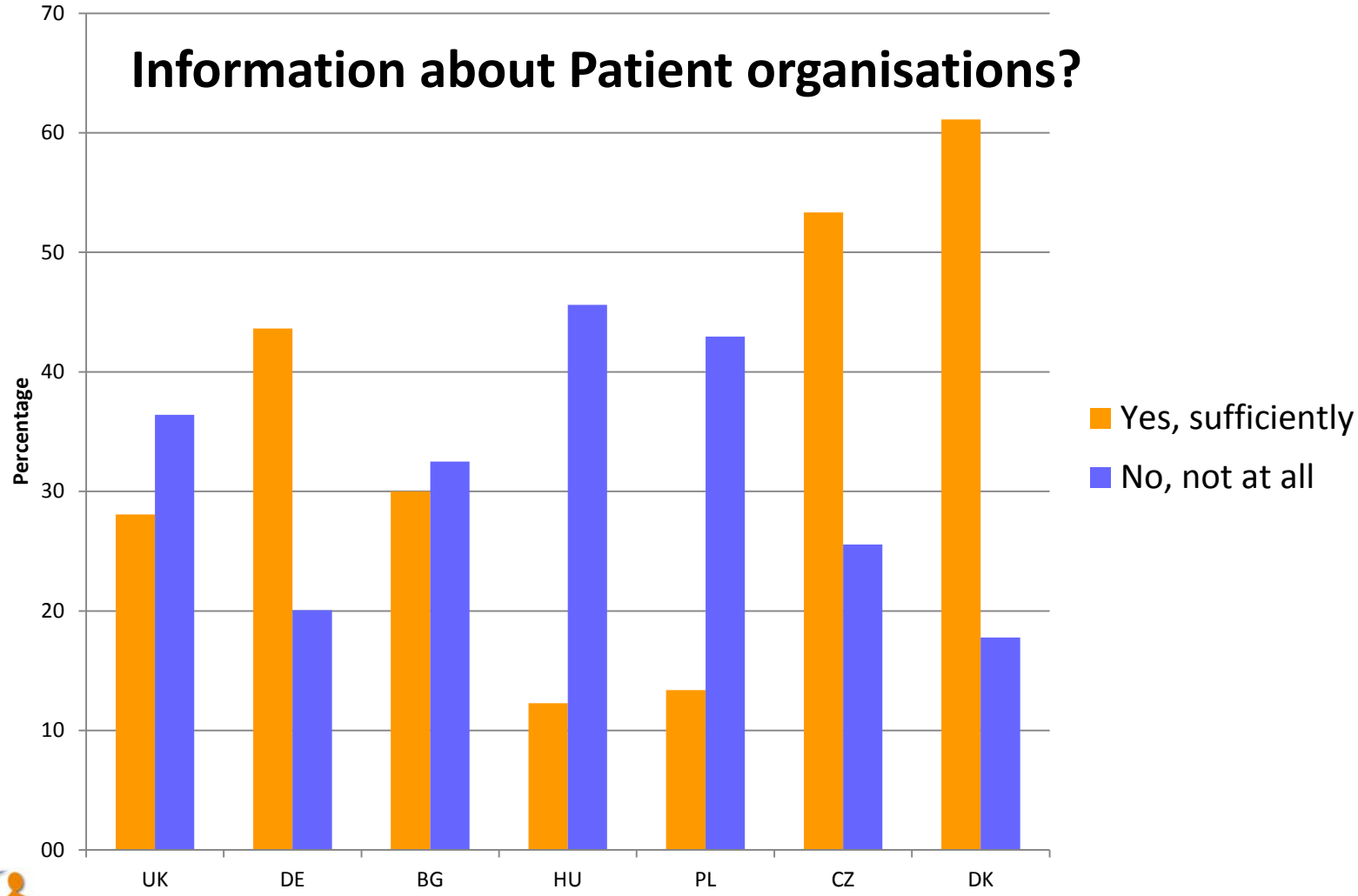
RESULTS

INFORMATION

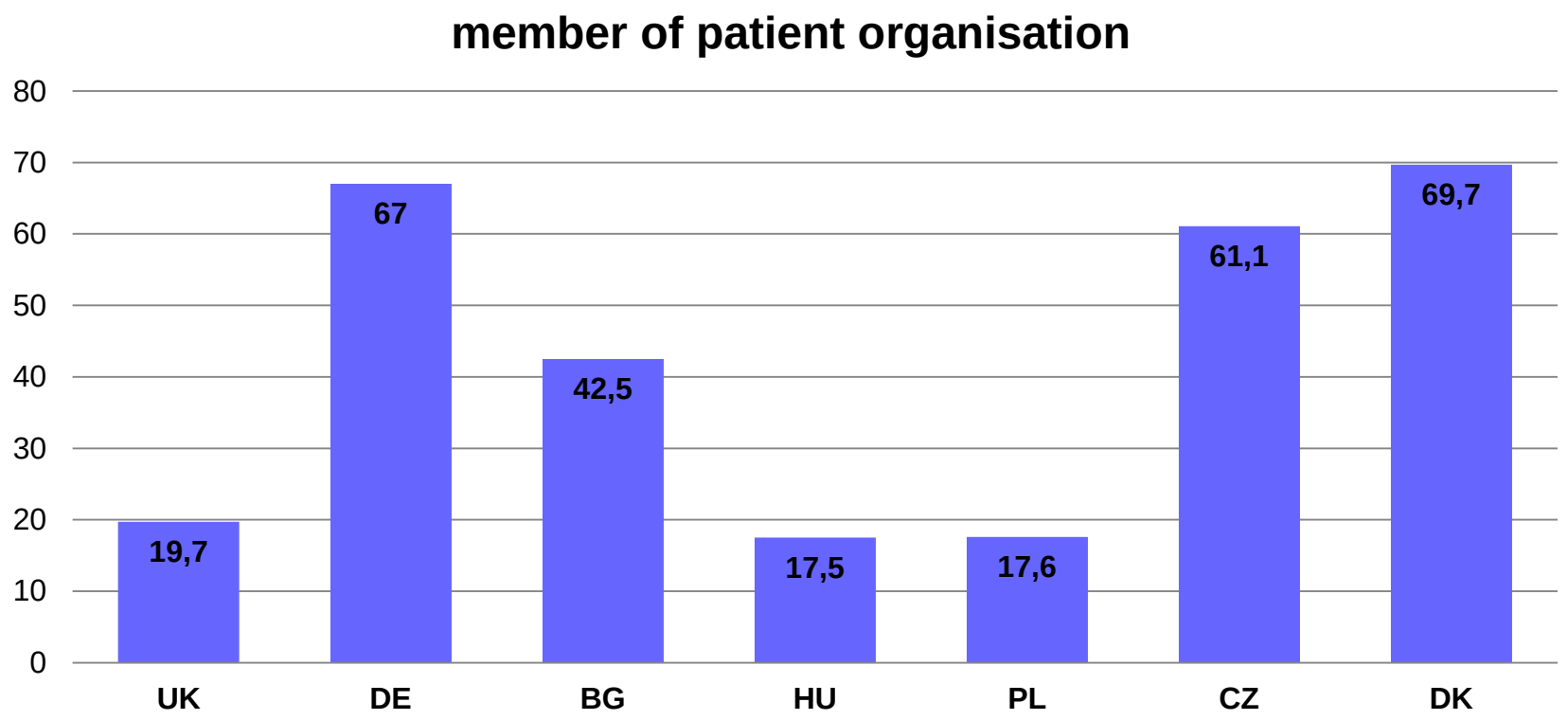
Has a medical professional ever talked to you about…



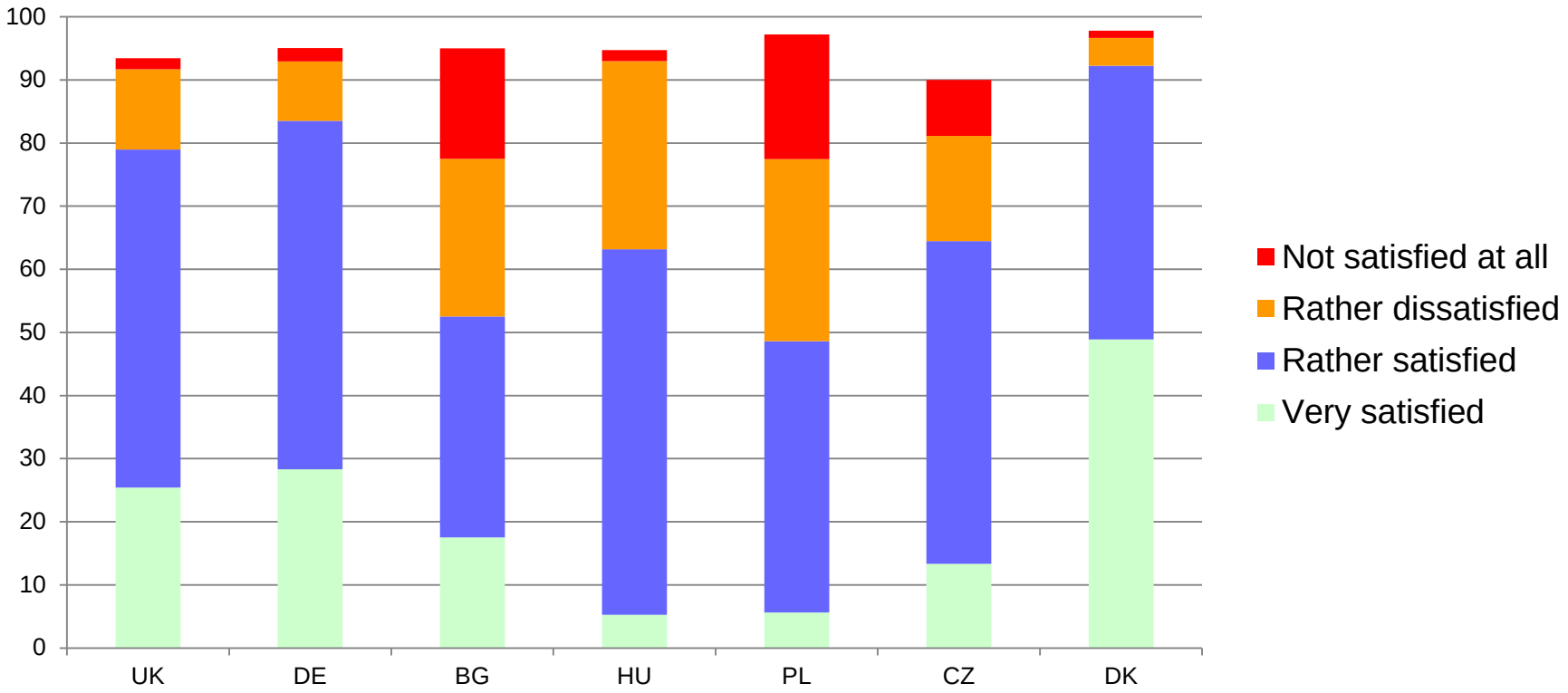
Has a medical professional ever talked to you about…



Patient organisations

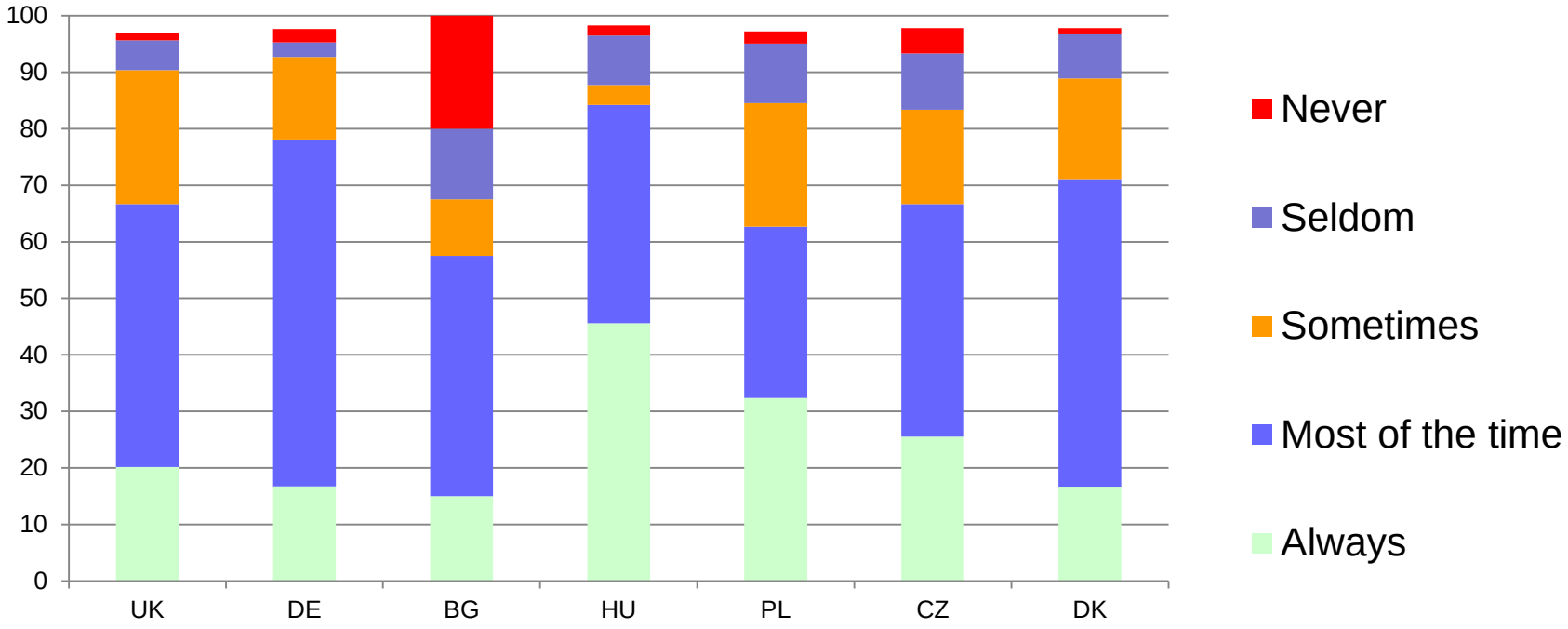


Satisfaction with medical treatment



Society

Are you viewed on equal terms by other citizens in your local community?



Key findings

- Loss of ambulation has been delayed through current care practice including steroid treatment
- Most but not all patients are offered steroid treatment
- The number of non-ambulatory and adult patients is constantly increasing
- Adult patients receive less physiotherapy and are often not seen at a neuromuscular centre
- Patients seen at neuromuscular centers receive more information and treatment according to recommendations

CARE-NMD

Thank you!

Kick-off meeting Luxembourg 2010

CARE-NMD

Executive Agency for Health and Consumers

TREAT-NMD Neuromuscular Network