

WIE WICHTIG IST DIE LEBENSQUALITÄT ALS ENDPUNKT IN PRAXIS UND KLINISCHER FORSCHUNG?

Sitz. Typ Fortbildung

Sitz. Titel MDS: Von der Diagnostik zur Therapie

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 **Hamburg**



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SEHR!!!



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OUTLINE



- INTRO / BEGRIFFE
- PRÄVALENZ & PRÄDIKTOREN
- RELEVANZ
- PRAKTISCHE TIPS
- ZUSAMMENFASSUNG

TERMINOLOGY

- ❑ **Performance status**
 - ❑ typical: WHO, Karnofsky.....
- ❑ **Patient-reported outcome(s) (PRO(s))**
 - ❑ reports directly patients' perception and perspective
 - ❑ typical: **Health-related quality of life (HRQoL)**, fatigue, depression, pain...
 - ❑ Score: **Patient-reported outcome measure(s) (PROM(s))**
- ❑ **Objective physical activities**
 - ❑ typical: gait speed, TUG (timed up and go)

HRQOL is appreciated by authorities

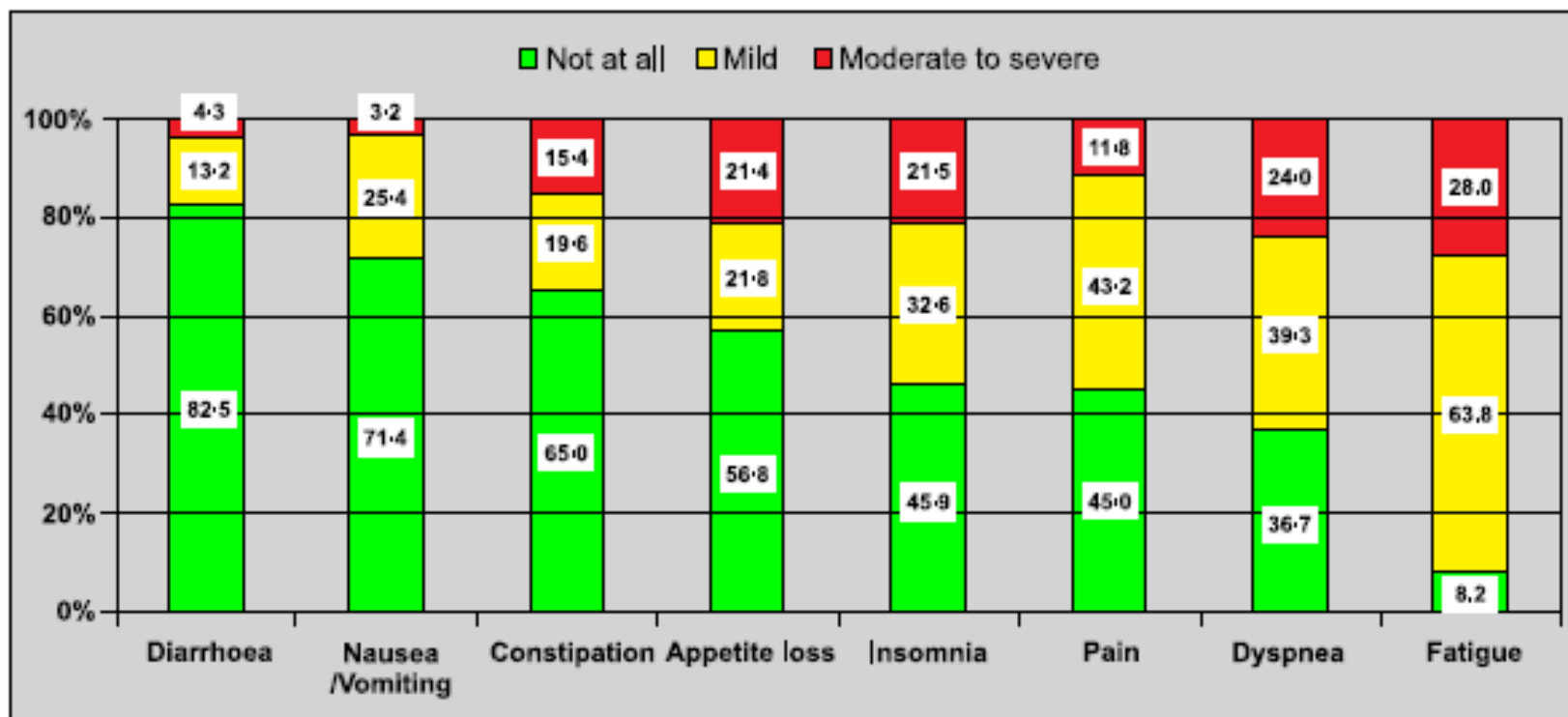
- **FDA: Clinical Outcome Assessments (COAs) to determine the treatment benefit of a drug; describes or reflects how a patient feels, functions, or survives.**
 - Clinician-reported outcomes (ClinRO)
 - Patient reported outcomes (PRO)
 - Performance outcome (PerO)
- **EMA 2016 guideline on the evaluation of anticancer medical products in man**
 - Appendix 2 focuses on the use of PROMs and HRQoL from a regulatory perspective.*the use of generally accepted instrument to estimate HRQoL or symptom control may provide valuable information.*

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Symptoms in patients with IPSS Int-2/High risk MDS

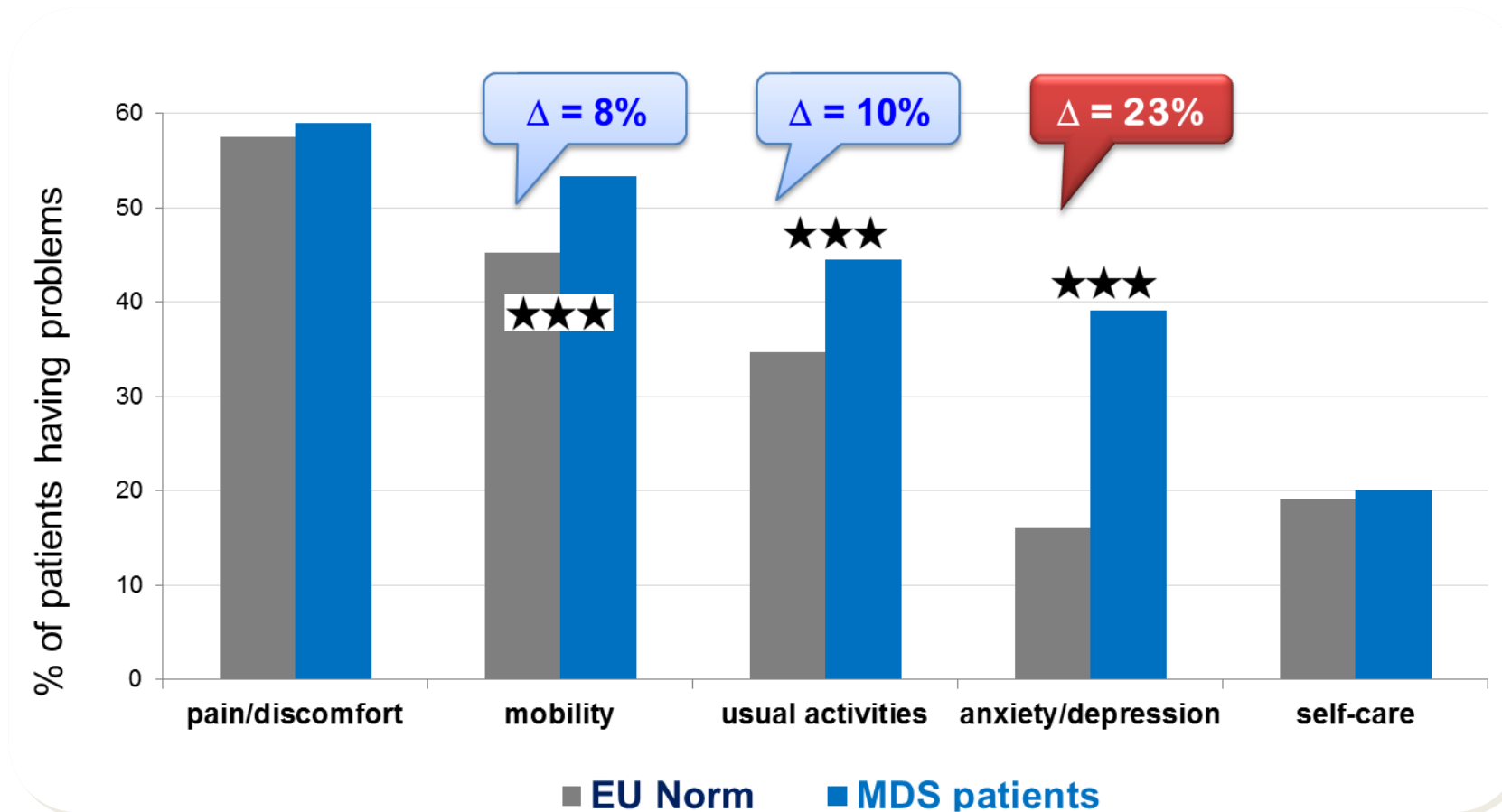


Pre-treatment Symptom Prevalence

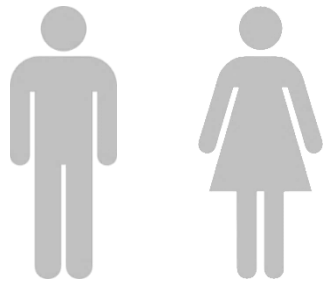
Symptom scale of the **EORTC QLQ-C30** has been scored on a (0–100) range (where a higher score means a worse outcome) and then rated as ‘Not at all’ (if score = 0), ‘Mild’ (if score >0 and <66) and ‘Moderate to severe’ (if score ≥66).

Symptom burden – EQ-5D

EU norm versus MDS patients

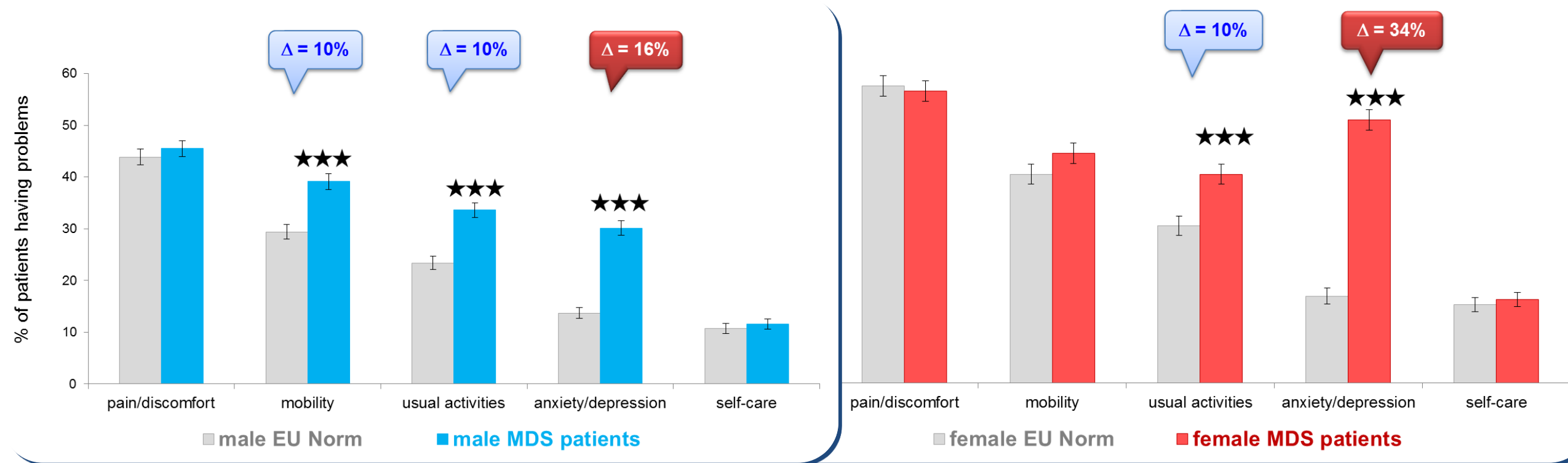


★★★★ $p < 0.001$, ★★★ $p < 0.01$, ★ $p < 0.05$



Symptom burden and sex – EQ-5D

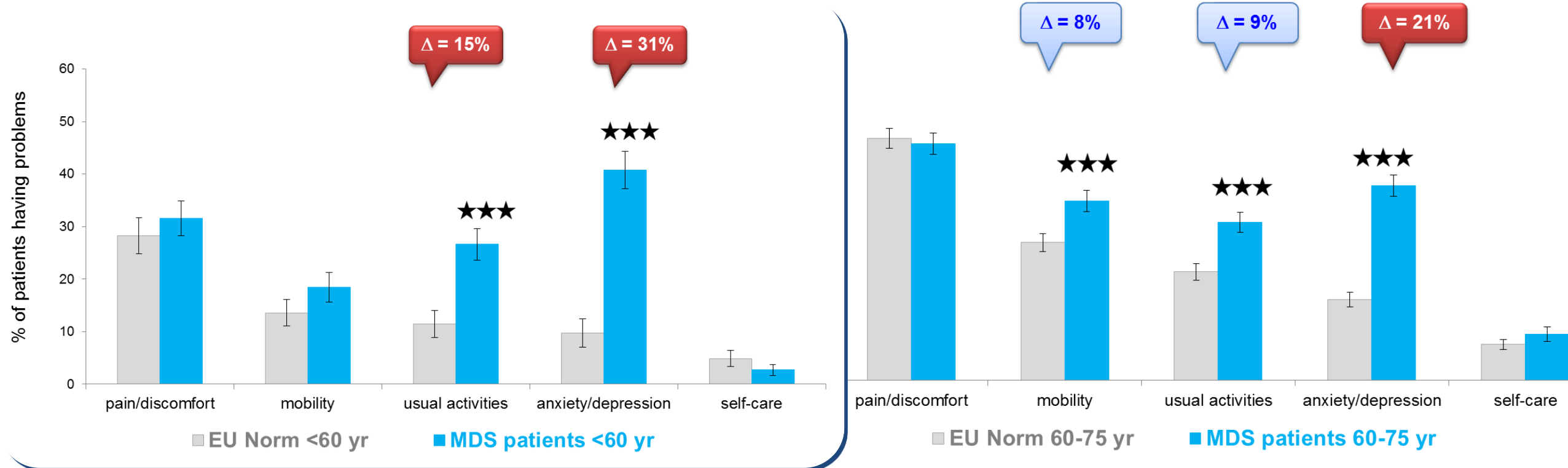
EU norm versus MDS patients



**** $p < 0.001$, ** $p < 0.01$, * $p < 0.05$

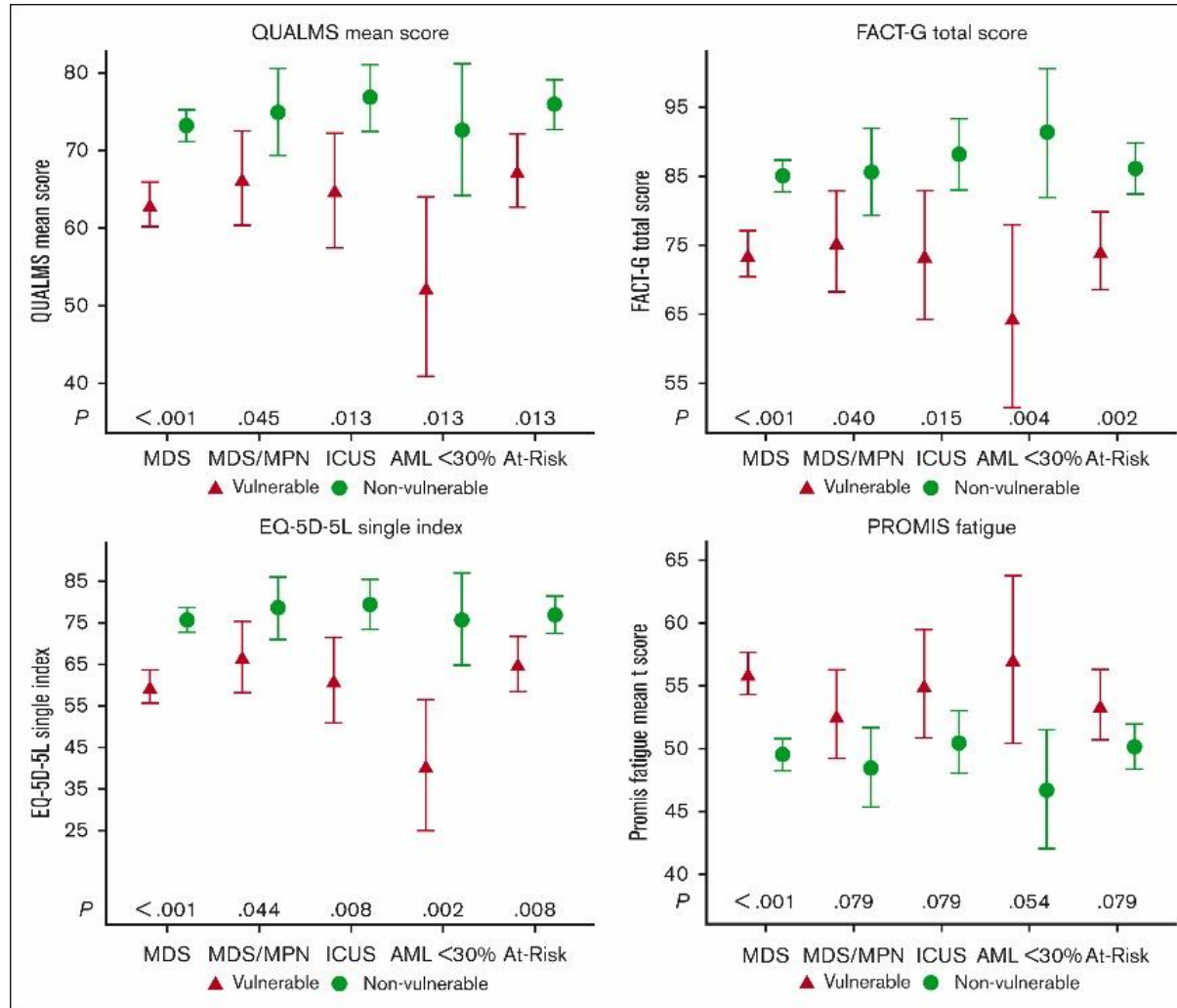
Symptom burden and age – EQ-5D

EU norm versus MDS patients



*** p < 0.001, ** p < 0.01, * p < 0.05

HRQoL and vulnerability among people with MDS - US national study



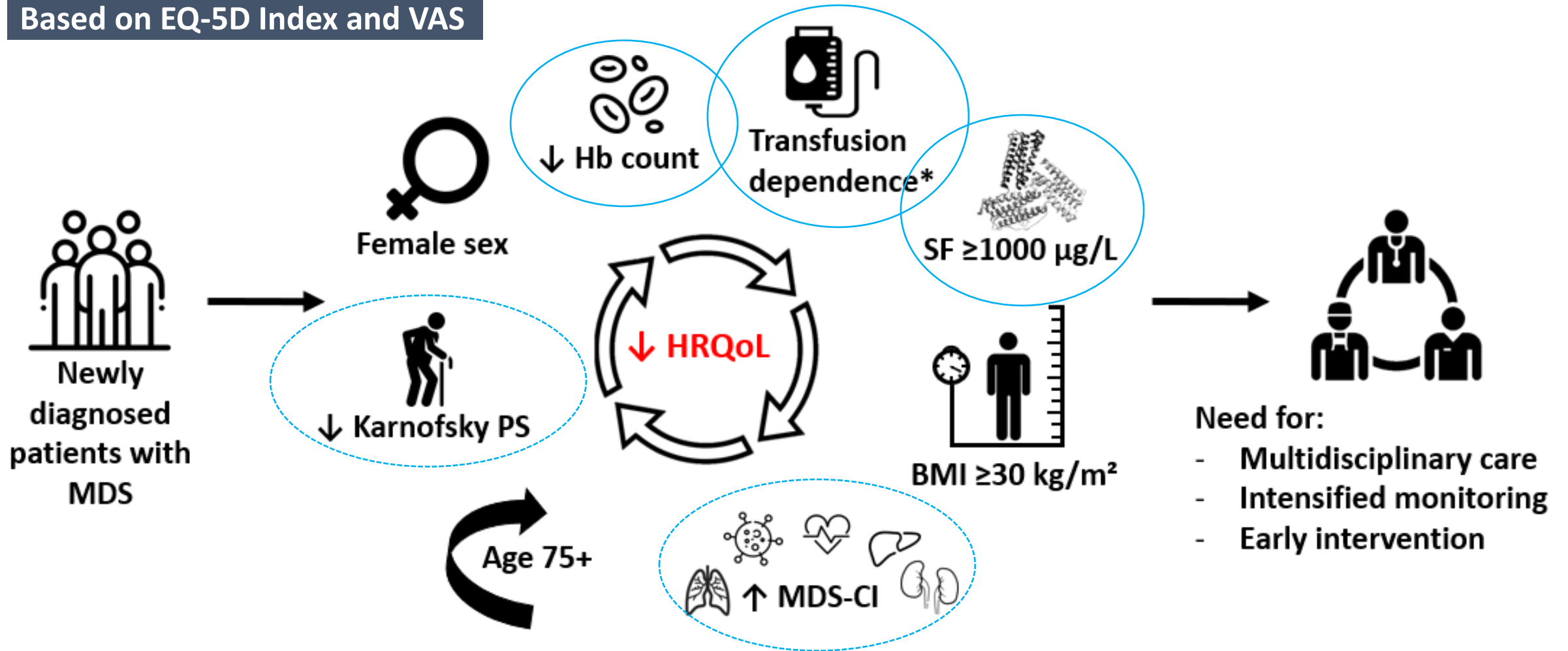
Impaired HRQoL is associated with vulnerability.....

Vulnerable defined as VES-13 \geq 3.



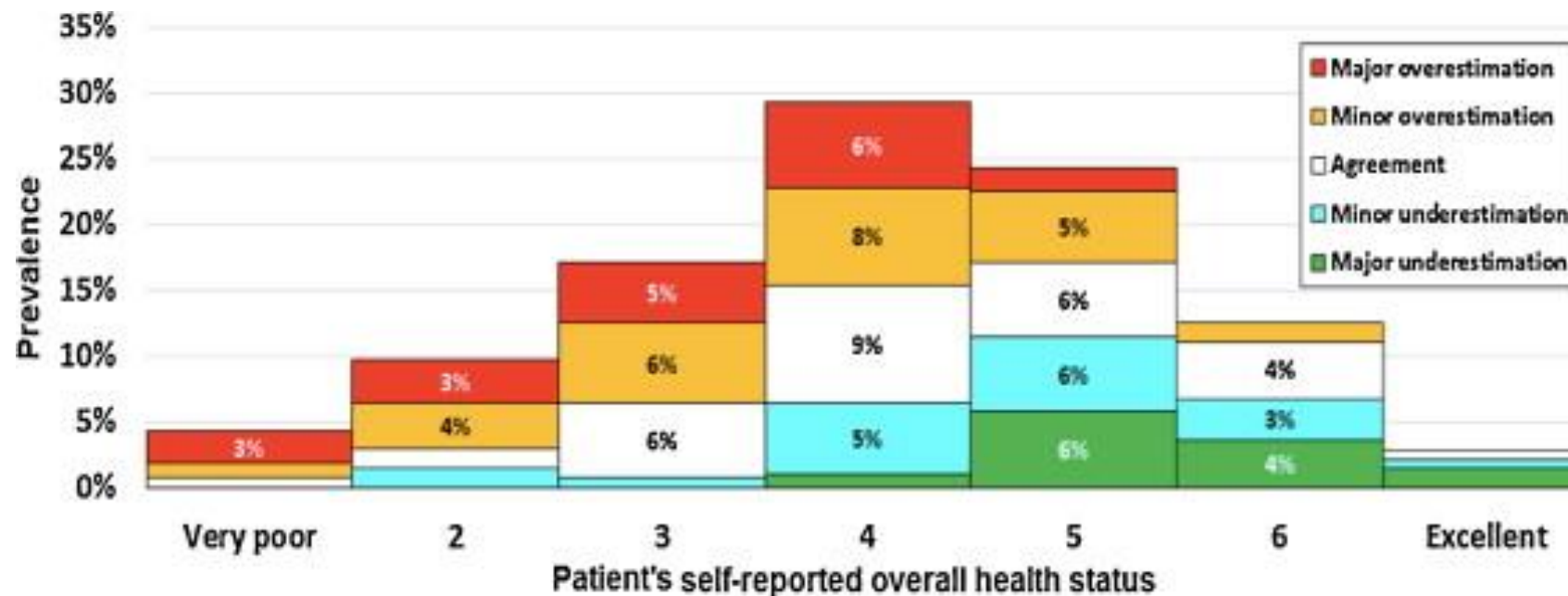
Predictors of impaired HRQoL

Based on EQ-5D Index and VAS



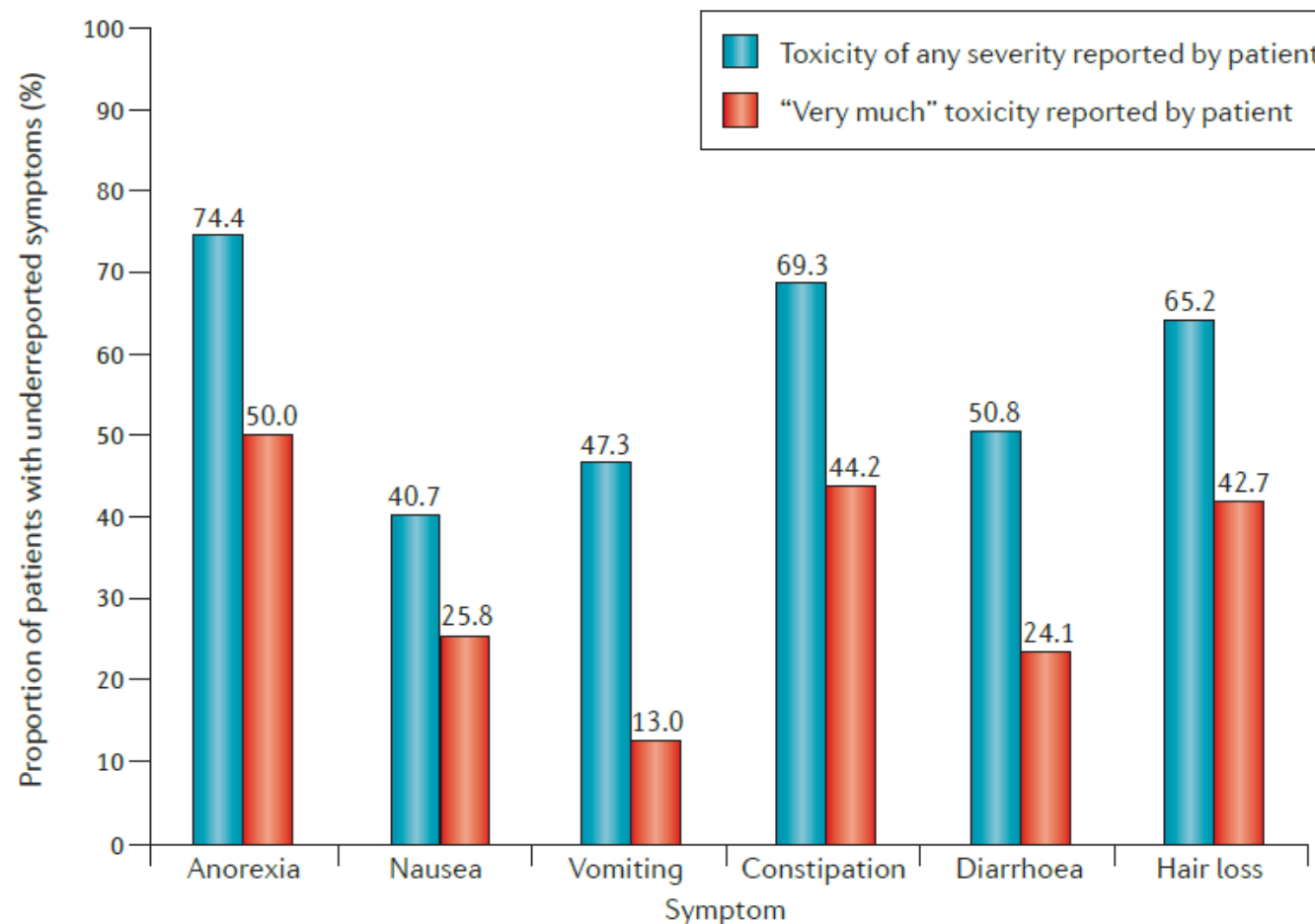
BMI - Body mass index; Hb - Hemoglobin; HRQoL - Health-related quality of life; Karnofsky PS - Karnofsky performance status; MDS-CI - MDS-specific comorbidity index; SF - Serum ferritin; ↓ - Low; ↑ - High. *At least one unit of red blood cells for a surveillance time of eight weeks before the HRQoL assessment.

Accuracy of physician assessment of treatment preferences and health status in MDS



- Based on EORTC QLQ C30
- Agreement on health status was found in 27.5% of cases.
- Physicians most frequently tended to overestimate health status of patients who reported low-level health status.

Underreporting of treatment-related toxicities by physicians, relative to patients



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HRQoL - SCORING

▣ Type of score

- ▣ Generic EQ-5D, EORTC-QLQ C30
- ▣ Symptom-specific FACIT-Fatigue scale, EORTC QLQ-FA12
- ▣ Disease-specific QUALMS, QoL-E

▣ Goal of analysis?

- ▣ Clinical setting?
- ▣ Working load for patients? No and complexity of items?
- ▣ Reference populations available?
- ▣ Cost-effectiveness analyses
- ▣ Validated?
- ▣ Costs, languages?

EORTC QLQ-C30



EORTC QLQ-C30 (version 3)

We are interested in some things about you and your health. Please answer all of the questions yourself by circling the number that best applies to you. There are no "right" or "wrong" answers. The information that you provide will remain strictly confidential.

Please fill in your initials:

Your birthdate (Day, Month, Year):

Today's date (Day, Month, Year):

31

	Not at All	A Little	Quite a Bit	Very Much
1. Do you have any trouble doing strenuous activities, like carrying a heavy shopping bag or a suitcase?	1	2	3	4
2. Do you have any trouble taking a <u>long</u> walk?	1	2	3	4
3. Do you have any trouble taking a <u>short</u> walk outside of the house?	1	2	3	4
4. Do you need to stay in bed or a chair during the day?	1	2	3	4
5. Do you need help with eating, dressing, washing yourself or using the toilet?	1	2	3	4

During the past week:

	Not at All	A Little	Quite a Bit	Very Much
6. Were you limited in doing either your work or other daily activities?	1	2	3	4
7. Were you limited in pursuing your hobbies or other leisure time activities?	1	2	3	4
8. Were you short of breath?	1	2	3	4
9. Have you had pain?	1	2	3	4
10. Did you need to rest?	1	2	3	4
11. Have you had trouble sleeping?	1	2	3	4
12. Have you felt weak?	1	2	3	4
13. Have you lacked appetite?	1	2	3	4
14. Have you felt nauseated?	1	2	3	4
15. Have you vomited?	1	2	3	4
16. Have you been constipated?	1	2	3	4

During the past week:

	Not at All	A Little	Quite a Bit	Very Much
17. Have you had diarrhea?	1	2	3	4
18. Were you tired?	1	2	3	4
19. Did pain interfere with your daily activities?	1	2	3	4
20. Have you had difficulty in concentrating on things, like reading a newspaper or watching television?	1	2	3	4
21. Did you feel tense?	1	2	3	4
22. Did you worry?	1	2	3	4
23. Did you feel irritable?	1	2	3	4
24. Did you feel depressed?	1	2	3	4
25. Have you had difficulty remembering things?	1	2	3	4
26. Has your physical condition or medical treatment interfered with your <u>family</u> life?	1	2	3	4
27. Has your physical condition or medical treatment interfered with your <u>social</u> activities?	1	2	3	4
28. Has your physical condition or medical treatment caused you financial difficulties?	1	2	3	4

For the following questions please circle the number between 1 and 7 that best applies to you

29. How would you rate your overall health during the past week?

1 2 3 4 5 6 7

Very poor

Excellent

30. How would you rate your overall quality of life during the past week?

1 2 3 4 5 6 7

Very poor

Excellent



EQ-5D-3L and VAS questionnaire

Your own health state today	Your own health state today
<p>By placing a tick in one box in each group below, please indicate which statement best describes your own health state today. Do not tick more than one box in each group.</p> <p>Mobility I have no problems in walking about <input type="checkbox"/> I have some problems in walking about <input type="checkbox"/> I am confined to bed <input type="checkbox"/></p> <p>Self-care I have no problems with self-care <input type="checkbox"/> I have some problems washing and dressing myself <input type="checkbox"/> I am unable to wash and dress myself <input type="checkbox"/></p> <p>Usual activities (eg. work, study, housework, family or leisure activities) I have no problems with performing my usual activities <input type="checkbox"/> I have some problems with performing my usual activities <input type="checkbox"/> I am unable to perform my usual activities <input type="checkbox"/></p> <p>Pain/discomfort I have no pain or discomfort <input type="checkbox"/> I have moderate pain or discomfort <input type="checkbox"/> I have extreme pain or discomfort <input type="checkbox"/></p> <p>Anxiety/depression I am not anxious or depressed <input type="checkbox"/> I am moderately anxious or depressed <input type="checkbox"/> I am extremely anxious or depressed <input type="checkbox"/></p>	<p>To help people say how good or bad a health state is, we have drawn a scale (rather like a thermometer) on which the best state you can imagine is marked 100 and the worst state you can imagine is marked 0.</p> <p>We would like you to indicate on this scale how good or bad your own health is today, in your opinion. Please do this by drawing a line from the box below to whichever point on the scale indicates how good or bad your health state is.</p> <p>Best imaginable health state</p> <p>100 90 80 70 60 50 40 30 20 10 0</p> <p>Worst imaginable health state</p> <p>Your own health state today</p>

QUality of Life in MDS Scale (QUALMS)



The QUALMS

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The Quality of Life in Myelodysplasia Scale

Patients often have different experiences over the course of their illness; however, please limit your responses to your experience **over the past week only**. The information you provide will remain strictly confidential.

During the past week, how often...	Never	Rarely	Sometimes	Often	Always
1 ...did you feel as though there was a lack of clear information about your disease?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2 ...have you felt there was limited emotional support available for patients with MDS beyond their families?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3 ...did you feel as though you couldn't do anything about your disease?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4 ...did you feel the course of your disease was unpredictable?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
5 ...did you have difficulty explaining MDS to your friends or family?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
6 ...did you have trouble concentrating?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
7 ...have you considered changing long-term plans due to health concerns?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
8 ...have you experienced shortness of breath?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
9 ...did low energy levels cause you to change your schedule?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
10 ...did you feel as though your life was organized around medical appointments?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
11 ...have you felt a sense of hopelessness?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
12 ...have you been worried about getting an infection?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
13 ...have you had sufficient energy for routine tasks?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
14 ...were you afraid of dying?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
15 ...did you feel angry about your diagnosis?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
16 ...were you worried about bleeding?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
17 ...did you feel a sense of gratitude for a part of life that you took for granted before?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
18 ...did you feel nauseated?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
19 ...did you worry about your MDS progressing or developing into leukemia?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
20 ...did you take into account that you might be fatigued when planning your activities?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

For the following questions, please again mark the answer choice that best represents your experiences and feelings **over the past week**. The information you provide will remain strictly confidential.

During the past week, how often...	Never	Rarely	Sometimes	Often	Always
21 ...were you concerned that your MDS caused a financial burden for you or your family?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
22 ...did you feel your family relationships were strained by your disease?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
23 ...have you felt weak?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
24 ...have you been too tired to take on the responsibilities you used to have?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
25 ...did you worry about becoming a burden to your friends or family?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
26 ...were you unable to participate in activities you are used to doing?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
27 ...have you felt anxious about test or lab results?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
28 ...did you avoid crowds because of fear of getting an infection?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
29 ...did you find yourself grateful for tomorrow?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
30 ...did you feel you were able to find quality information about MDS treatments?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
31 ...were you concerned about bruising?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
32 ...did you feel as though there were a lack of concrete answers about what will happen with your MDS?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
33 ...did you experience a change in bowel habits?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

For the following questions, you may select "not applicable" if the question does not apply to you.

During the past week, how often...	Never	Rarely	Sometimes	Often	Always
34 ...were you afraid of losing your job? (check here <input type="checkbox"/> if not applicable because you are unemployed/retired)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
35 ...did you feel too tired to drive? (check here <input type="checkbox"/> if not applicable because you do not drive)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
36 ...were you afraid to have sex due to your blood counts? (check here <input type="checkbox"/> if not applicable because you are not currently sexually active)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
37 ...were you afraid that your MDS treatment would stop working? (check here <input type="checkbox"/> if not applicable because you are not currently being treated)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
38 ...have you been too tired to take care of a family member or loved one? (check here <input type="checkbox"/> if not applicable because you are not providing such care)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>



PRO domains covered by QUALMS (Quality of Life in MDS Scale)

Domain measured	Scale abbreviation	n. of items	Items*
Physical burden	QUALMS-P	14	<ul style="list-style-type: none"> Too tired for prior responsibilities Low energy change schedule Weak Unable participate in activities Take into account might be fatigued Worry about becoming burden Felt hopelessness Change in bowels Shortness of breath Change long-term plans due to health Trouble concentrating Life organized around medical Nauseated Energy for routine tasks
Emotional Burden	QUALMS-E	11	<ul style="list-style-type: none"> Could not do anything about disease Disease unpredictable Lack of concrete answers No clear information Afraid of dying Difficulty explaining MDS to others Worry progressing/leukemia Anxious about tests or lab results Angry about diagnosis Worried infection Limited emotional support available
Benefit finding	QUALMS-BF	3	<ul style="list-style-type: none"> Grateful for tomorrow Get quality information Gratitude when prior took for granted
Single optional items	N/A	5	<ul style="list-style-type: none"> Family relationships strained Bruising Avoid crowds Worried bleeding Concerned financial burden

* The items are reported as in Table 2 from Abel et al. (Haematologica. 2016;101(6):781-788).

Differences in the scales of the QUALMS by MDS-CI, Karnofsky performance status, Anemia, and RBC transfusions

	MDS-Comorbidity Index					Karnofsky performance status					Anemia ^a					RBC transfusions ^b				
	Low (n=143)		Int./High (n=94)			< 90 (n=110)		≥ 90 (n=91)			Anemic patients (n=218)		Non-Anemic patients (n=35)			No (n=166)		Yes (n=68)		
QUALMS Scale	Mean	SD	Mean	SD	P	Mean	SD	Mean	SD	P	Mean	SD	Mean	SD	P	Mean	SD	Mean	SD	P
QUALMS-P	66.6	21.3	58.6	21.9	0.006	54.6	19.4	75.8	17.9	<0.001	60.5	21.8	77.7	16.5	<0.001	68.8	20.4	49.6	18.4	<0.001
QUALMS-E	72.8	17.7	65.8	21.3	0.014	67.0	18.7	75.3	19.3	0.001	68.1	19.4	78.5	17.9	0.010	72.2	19.0	63.5	19.5	0.002
QUALMS-BF	50.8	26.5	48.8	24.3	0.483	50.9	23.2	49.4	29.7	0.829	51.3	25.0	43.8	29.9	0.243	47.9	25.9	57.4	21.0	0.014
QUALMS Total	69.1	14.8	62.2	17.6	0.006	61.8	15.0	74.2	14.5	<0.001	64.6	16.4	76.3	12.7	0.001	69.8	15.3	57.6	15.1	<0.001

a WHO definition: female Hb <12.0 g/dL, male Hb <13.0 g/dL; Mean and (SD) of Hb levels were 9.5 g/dL (1.8) and 13.7 g/dL (1.1), for anemic and non-anemic patients, respectively.

b Denotes whether the patient has received any RCBs within one year from the baseline QUALMS assessment.

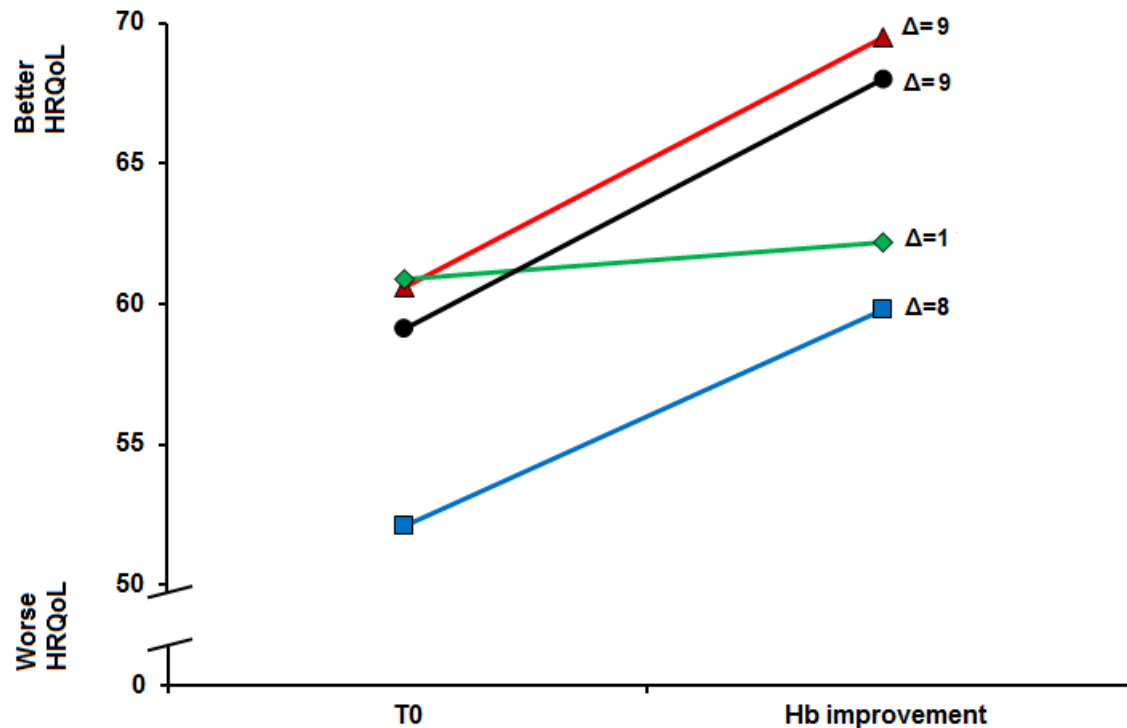
Abbreviations: MDS-CI = MDS-comorbidity index; Int. = intermediate; RBC = red blood cell; QUALMS-P = physical burden; QUALMS-E = emotional burden; QUALMS-BF = benefit finding. SD; standard deviation.

Responsiveness to change of the QUALMS by hemoglobin improvements



■ QUALMS-P ▲ QUALMS-E ◆ QUALMS-BF ● Total score

Hb improvement



Responsiveness to change of the QUALMS by meaningful improvement in Hb values (≥ 1.5 g/dL) from baseline (only for patients with a baseline Hb level < 11 g/dL) ($n = 30$).

Abbreviations:

QUALMS-P = physical burden;

QUALMS-E = emotional burden;

QUALMS-BF = benefit finding.

NCI Patient Reported Outcomes version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE™)



- PRO-CTCAE Item Library includes
 - 124 items representing
 - 78 symptomatic toxicities drawn from the CTCAE
- More than 30 languages available
- PRO-CTCAE should be used in conjunction with CTCAE

Patient-Reported Outcomes version Of The Common Terminology Criteria For Adverse Events (PRO-CTCAE™) QUICK GUIDE TO THE ITEM LIBRARY*

Oral	Respiratory	Neurological	Sleep/Wake	Sexual
Dry mouth S	Shortness of breath SI	Numbness & tingling SI	Insomnia SI	Achieve and maintain erection S
Difficulty swallowing S	Cough SI	Dizziness SI	Fatigue SI	Ejaculation F
Mouth/throat sores SI	Wheezing S	Visual/Perceptual	Mood	Decreased libido S
Cracking at the corners of the mouth (cheilosis/cheilitis) S	Cardio/Circulatory	Blurred vision SI	Anxious FSI	Delayed orgasm P
Voice quality changes P	Swelling FSI	Flashing lights P	Discouraged FSI	Unable to have orgasm P
Hoarseness S	Heart palpitations FS	Visual floaters P	Sad FSI	Pain w/sexual intercourse S
Gastrointestinal	Cutaneous	Watery eyes SI	Genitourinary	Miscellaneous
Taste changes S	Rash P	Ringing in ears S	Irregular periods/vaginal bleeding P	Breast swelling and tenderness S
Decreased appetite SI	Skin dryness S	Attention/Memory	Missed expected menstrual period P	Bruising P
Nausea FS	Acne S	Concentration SI	Vaginal discharge A	Chills FS
Vomiting FS	Hair loss A	Memory SI	Vaginal dryness S	Increased sweating FS
Heartburn FS	Itching S	Pain	Painful urination S	Decreased sweating P
Gas P	Hives P	General pain FSI	Urinary urgency FI	Hot flashes FS
Bloating FS	Hand-foot syndrome S	Headache FSI	Urinary frequency FI	Nosebleed FS
Hiccups FS	Nail loss P	Muscle pain FSI	Change in usual urine color P	Pain and swelling at injection site P
Constipation S	Nail ridging P	Joint pain FSI	Urinary incontinence FI	Body odor S
Diarrhea F	Nail discoloration P			
Abdominal pain FSI	Sensitivity to sunlight P			
Fecal incontinence FI	Bed/pressure sores P			
	Radiation skin reaction S			
	Skin darkening P			
	Stretch marks P			

Attributes	
F: Frequency	I: Interference
S: Severity	P: Presence/Absence
A: Amount	

*Complete library of items available at: <https://healthcaredelivery.cancer.gov/pro-ctcae>

Version date: 3/11/2020

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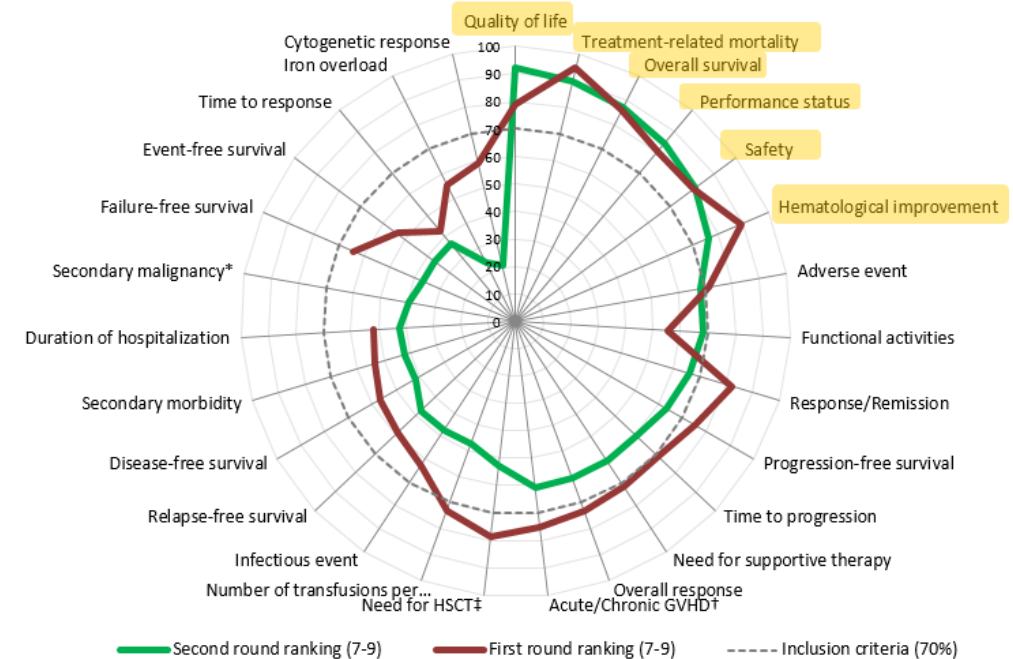
First Core Outcome Set for MDS

research paper

Development of a core outcome set for myelodysplastic syndromes – a Delphi study from the EUMDS Registry Group

Newly developed core outcome set in MDS (MDS-COS):

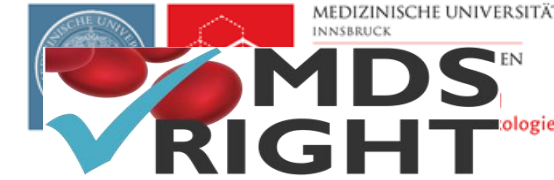
- health-related quality of life (HRQoL)
- treatment-related mortality
- overall survival
- performance status
- safety improvement
- haematological improvement



Ranking of outcomes based on Delphi survey (Round 1: n=56, 2: n=38 EUMDS-experts)

Core outcome set (COS)...agreed standardized set of outcomes that should be measured and reported, as a minimum, in all clinical trials in specific areas (COMET Initiative)

First Core Outcome Set for MDS



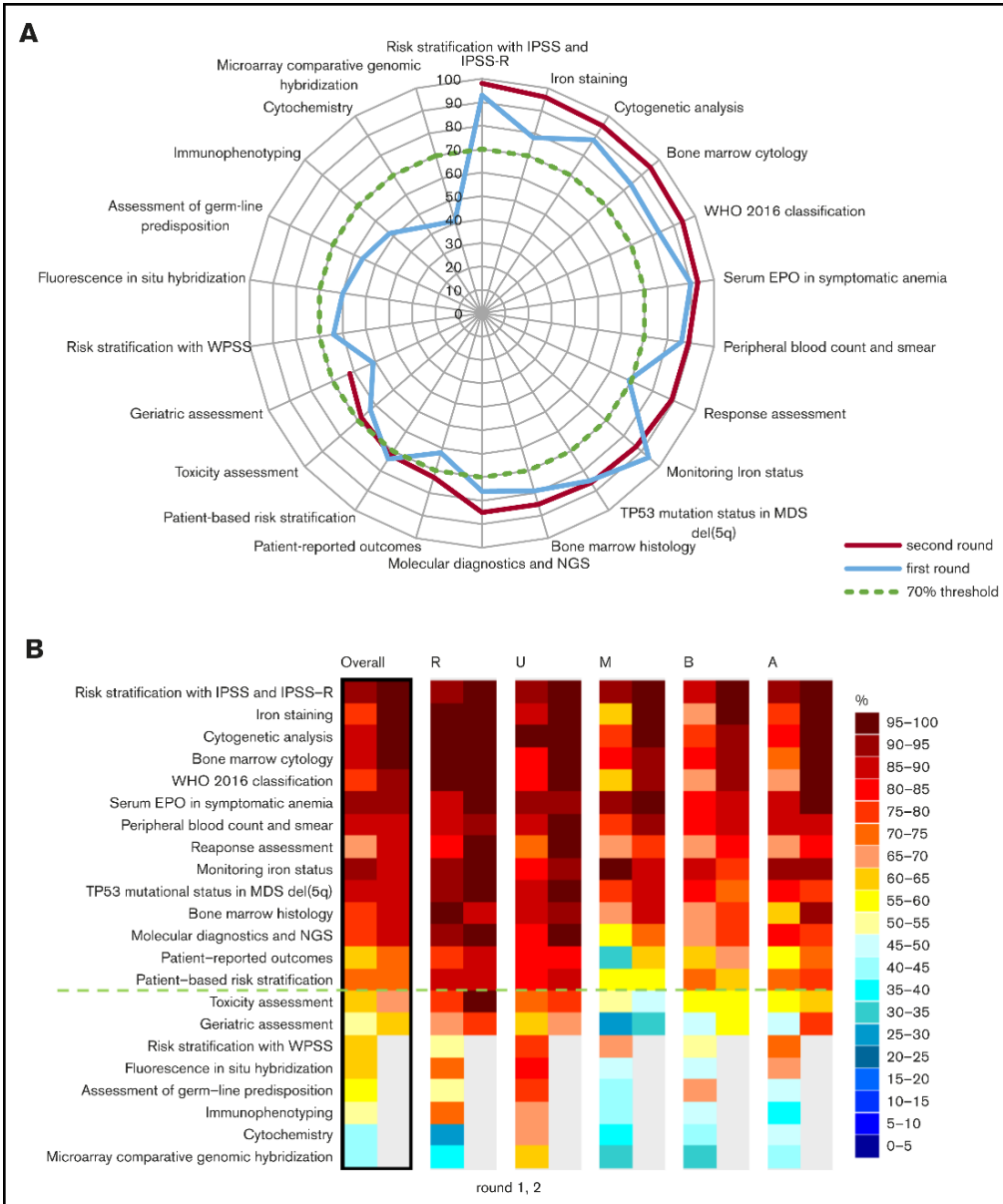
research paper

Development of a core outcome set for myelodysplastic syndromes – a Delphi study from the EUMDS Registry Group

Table 1. Application of outcomes: results from the first Delphi round.

Potential MDS core outcomes	IPSS-R risk group			*	Therapy			*	Clinical setting			*
	0–3 (Very) Low	>3–4.5 Intermediate	>4.5 (Very) High		Supportive	Disease-modifying	HSCT		Clinical study	Registry	Daily practice	
Health-related quality of life	41 (34.5)	38 (31.9)	40 (33.6)		37 (35.9)	37 (35.9)	29 (28.2)		30 (33.7)	21 (23.6)	38 (42.7)	*
Treatment-related mortality	25 (23.4)	34 (31.8)	48 (44.9)	*	11 (11.8)	37 (39.8)	45 (48.4)	*	33 (41.8)	20 (25.3)	26 (32.9)	*
Overall survival	28 (25.5)	35 (31.8)	47 (42.7)	*	14 (14.3)	41 (41.8)	43 (43.9)	*	34 (38.6)	27 (30.7)	27 (30.7)	
Performance status	38 (29.5)	45 (34.9)	46 (35.7)	*	23 (20.9)	41 (37.3)	46 (41.8)	*	38 (39.2)	24 (24.7)	35 (36.1)	*
Safety	46 (35.9)	42 (32.8)	40 (31.3)		24 (24.0)	41 (41.0)	35 (35.0)	*	38 (43.2)	18 (20.5)	32 (36.4)	*
Haematological improvement	40 (32.8)	43 (35.2)	39 (32.0)		21 (26.3)	40 (50.0)	19 (23.8)	*	31 (35.6)	22 (25.3)	34 (39.1)	*
Adverse event	40 (32.8)	42 (34.4)	40 (32.8)		23 (22.5)	42 (41.2)	37 (36.3)	*	38 (45.2)	18 (21.4)	28 (33.3)	*

Guideline-based indicators for patients with MDS



RATING PERFORMANCE – DIAGNOSIS

- PROs reached required threshold for acceptance only after rechallenge in the second round.
- Underlies ongoing controversies and the requirement for further improvements in standardization, practicability and validation of the clinical utility.

PROs as defined by MDS-specialists and by patients



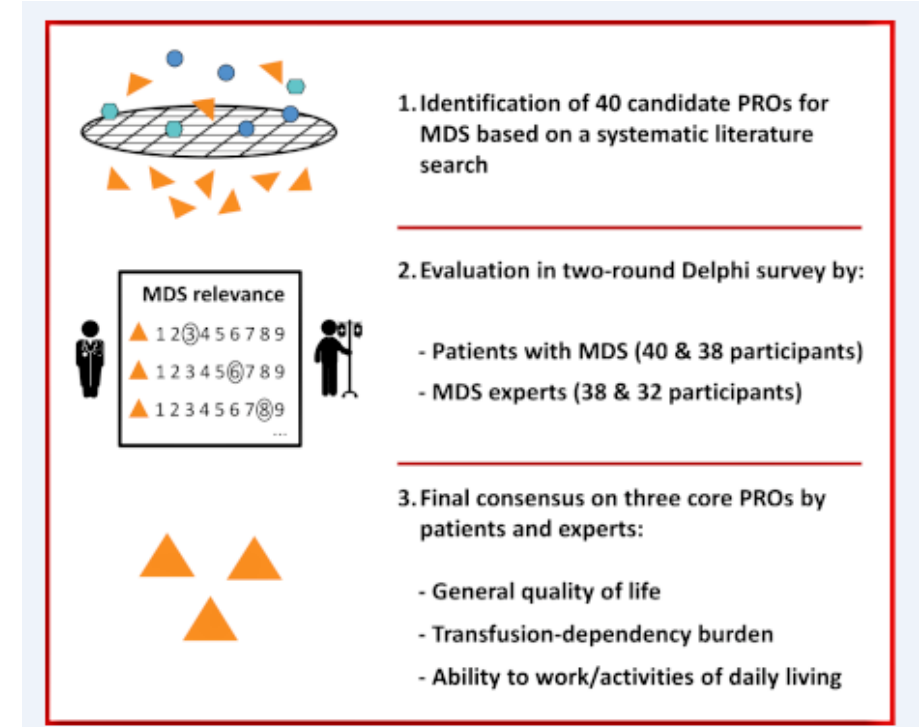
Stojkov I et al., Blood Adv. 2021 Sep 7

Core set of patient-reported outcomes for MDS: an EUMDS Delphi study involving patients and hematologists

Stojkov I, Conrads-Frank A, Rochau UG, Koinig KA, Arvandi M, Puntcher S, van Marrewijk CJ, Fenaux P, Symeonidis A, Chermat F, Garelius HKG, Bowen DT, Mittelman M, Mora E, de Witte TM, Efficace F, Siebert U, Stauder R.

Core patient-reported outcomes (PROs) defined by patients and experts:

- General quality of life
- Transfusion-dependency burden
- Ability to work/activities of daily living



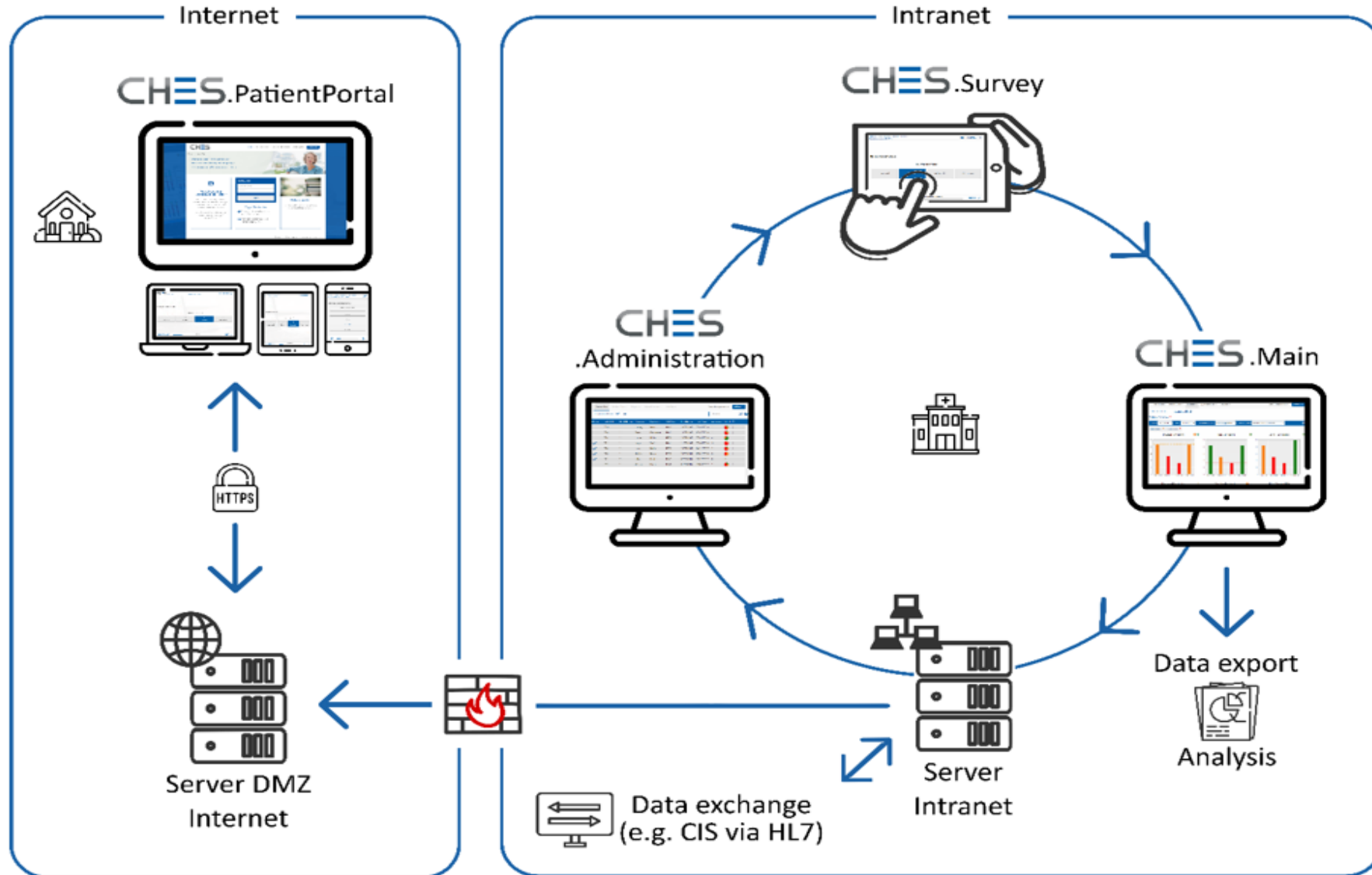
Ranking of outcomes based on Delphi survey

OUTLINE



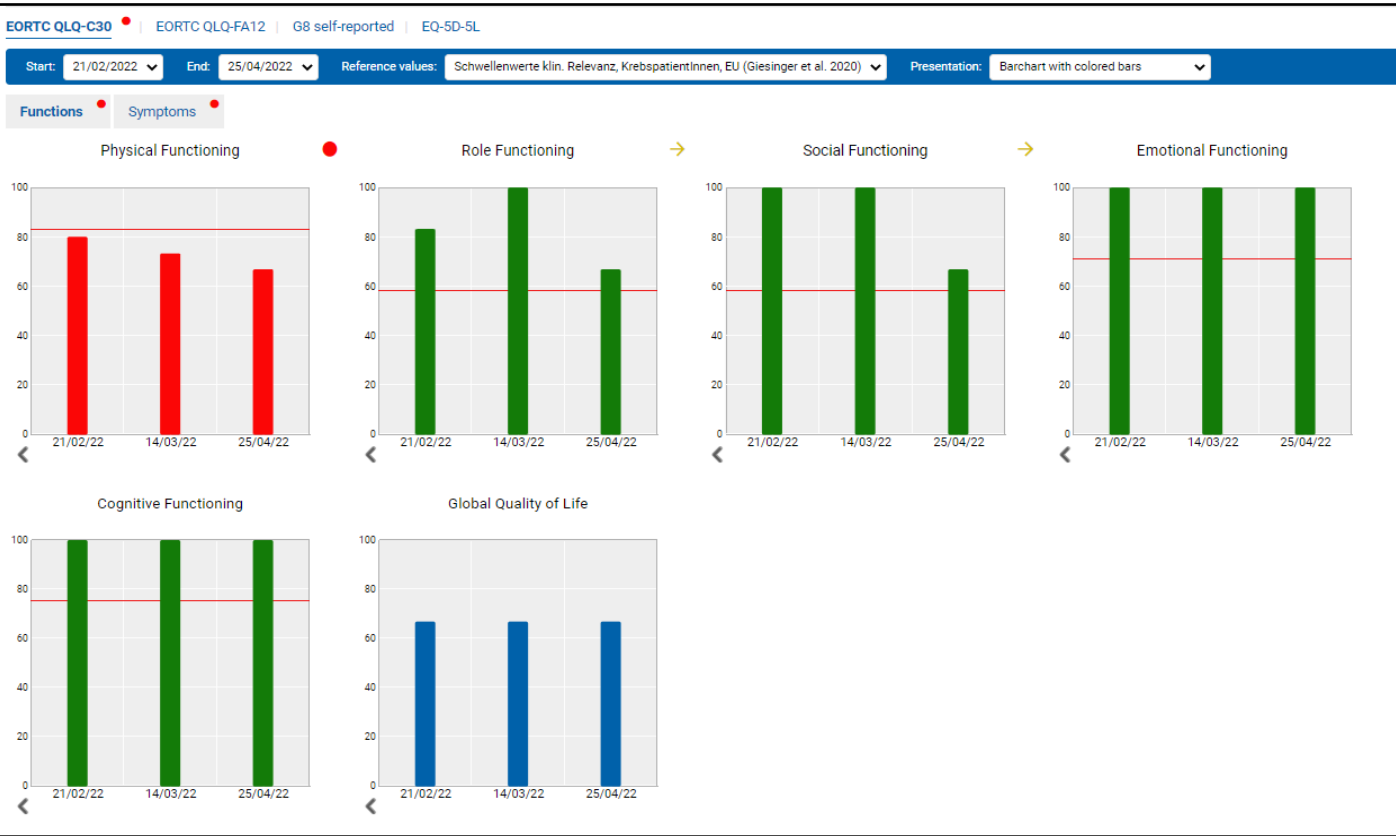
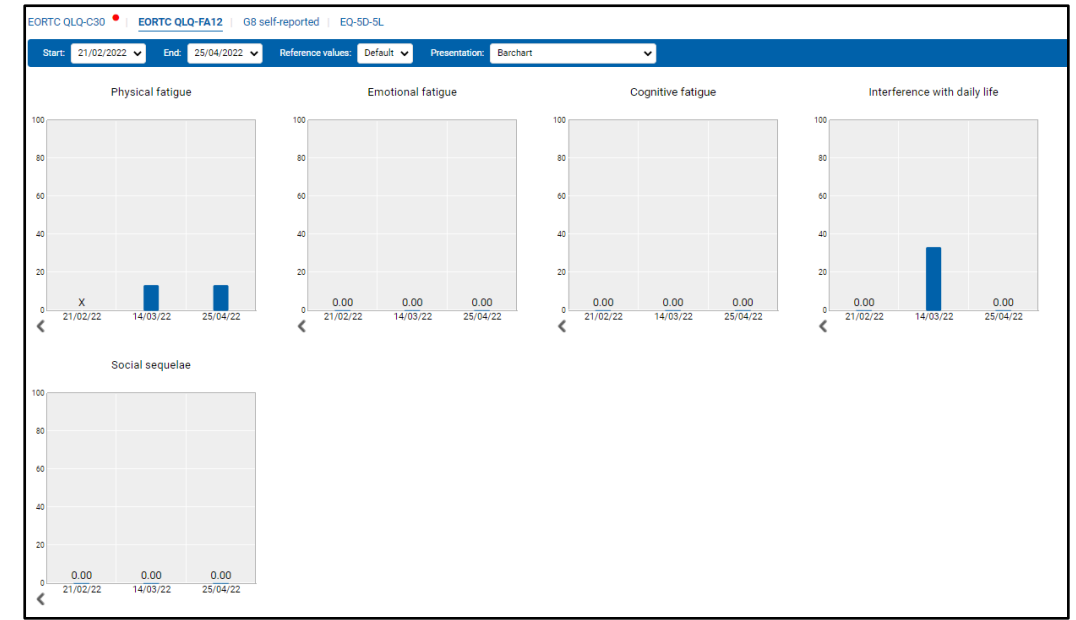
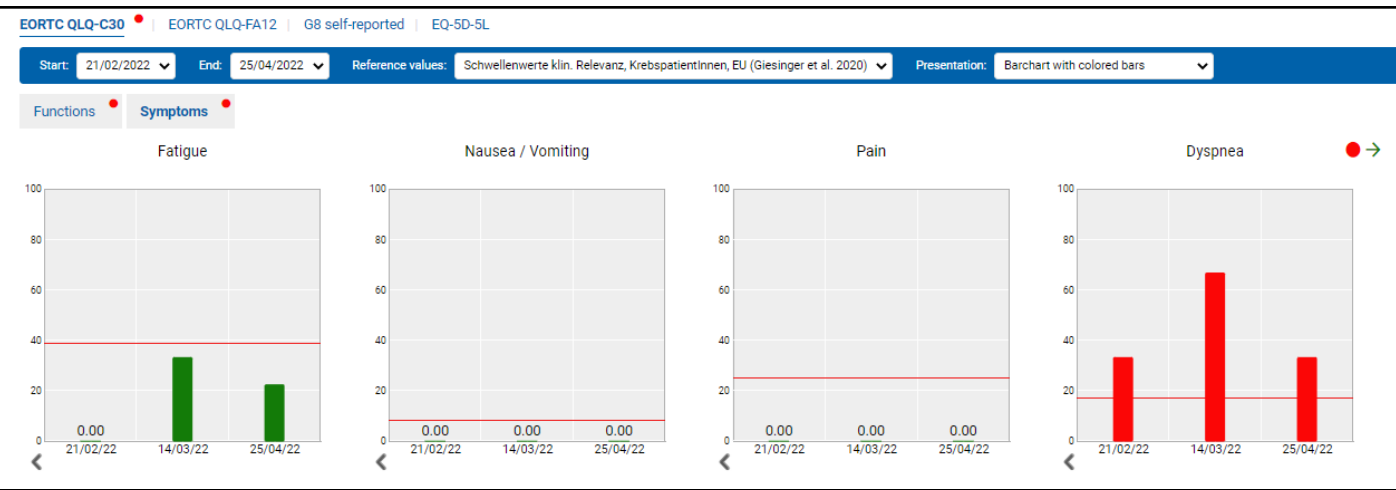
- INTRO / BEGRIFFE
- PRÄVALENZ & PRÄDIKTOREN / DETERMINANTEN
- SCORES / PROMs
- RELEVANZ
- PRAKTISCHE TIPS
- ZUSAMMENFASSUNG

IT Infrastructure, Softwaretool CHES



- Evaluation Software Development (ESD)
- Computer-based Health Evaluation System (CHES)

<https://ches.pro/>



ADVANTAGES

- *Frequent & remote assessments*
- *Longitudinal evaluation*

- Evaluation Software Development (ESD)
 - Computer-based Health Evaluation System (CHES)
- Available at: <https://ches.pro/> Accessed May 2022

ZUSAMMENFASSUNG - CONCLUSIONS

HRQoL ist wichtig

- Patient empowerment; partizipative Entscheidungsfindung; Patientenbetreuung
- Feedback basierend auf PROMs erhöht die Motivation von Patient:innen und Behandler:innen
- Endpunkt auch für Zulassungsbehörden
- Evidenz für Benefit in diversen Tumorentitäten

HÜRDEN

- Zeit
- Ressourcen
- Score?
- Interpretation und Auswertung



WIE WICHTIG IST DIE LEBENSQUALITÄT ALS ENDPUNKT IN PRAXIS UND KLINISCHER FORSCHUNG?

Sitz. Typ Fortbildung

Sitz. Titel **MDS: Von der Diagnostik zur
Therapie**

16-10-2023, 08:00-09:30



JAHRESTAGUNG

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