UNIVERSITY OF BIRMINGHAM | INSTITUTE OF CARDIOVASCULAR SCIENCES

Universitäres Herz- und Gefäßzentrum Hamburg

Should unmet medical needs in the future take into account PED/PROs?

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Declaration of interests (past and current)

Consulting Fees/Honoraria

3M Medica
AstraZeneca
Bayer
Boehringer Ingelheim
Boston Scientific
Bristol Myer Squibb
Cardiome
Daiichi-Sankyo
Johnson & Johnson
MEDA Pharma
Medtronic
Merck
Otsuka
Pfizer
Sanofi Aventis
Servier
Siemens
Takeda

Research Grants

British Heart Foundation (BHF)
Accelerating Clinical Trials Network Canada
Dutch Heart Foundation
Else Kröner Fresenius Stiftung
European Union
Fondation Leducq
German Federal Ministry for Education and Research (BMBF)
German Heart Foundation (DHF)
German Research Foundation (DFG)
3M Medica
Cardiovascular Therapeutics
Daiichi Sankyo
Pfizer / BMS
MEDA Pharma
Medtronic
OMRON
Sanofi
St Jude Medical / Abbott

Patents (filed by University of Birmingham, transferred to UKE)

EP3120149A1
EP3172571B1

No personal fees or honoraria received from industry in the last five years

Long interest in what we now call patient-centred care and PROs

Yes of course!

…as long as the information is valid and reliable

…as long as the information is viewed in context

Yes of course!

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Unmet needs in cardiovascular diseases

Unmet Needs in CVD

- **Number one cause of mortality** in Europe
- Stagnation in **marketing authorisation approvals**
- Do not fit narrow **definitions** of **unmet medical need**
- Clinical trials are **large, lengthy** and **costly**
- Lack of **validated surrogate endpoints**

Call to Action

- Development of **patient-relevant surrogate and composite endpoints**
- Establishment of **patient-relevant, risk-based approach**
- More pragmatic definitions of **unmet medical need** and better alignment with disease burden
- Paradigm shift in **evidence generation**
- Use of **conditional marketing approvals**
- Trials designed to **support reimbursement decisions**
- Greater **public awareness** and **patient activism** **and** **greater advocacy by healthcare professionals**

ESC CRT position paper from Feb 2024 meeting. Szymanski P et al., submitted

Patient outcomes in clinical care: E Codman

Ernest Amory Codman, M.D., (1869-1940)

Surgeon, one of the first to introduce M&M conferences and to collect outcomes using 'end result cards'.

"We believe it is the duty of every hospital to establish a follow-up system, so that as far as possible, the result of every case will be available at all times for investigation by members of the staff, the trustees, or administration, or by other authorized investigators or statisticians."

https://commons.wikimedia.org/w/index.php?curid=10538769

Patient-reported indicators in clinical care: OECD

Patient-reported indicators measure whether people benefit from health care, not what their care providers do.

Patients report on **outcomes** that matter to them – whether treatment reduced their pain, for example, or if it helped them live more independently.

People also report on their **experience** of being treated – whether the treatment was properly explained, for example, or if they felt involved in decisions about their care.

Monitoring these indicators internationally will provide new tools to improve health care **policy** and **practice**.

OECD
BETTER POLICIES FOR BETTER LIVES

The EHRA scale, a simple patient-reported outcome in AF

Table 6 EHRA AF symptoms classification

	Symptom severity	Definition
EHRA I	'No symptoms'	
EHRA II	'Mild symptoms'	Normal daily activity not affected
EHRA III	'Severe symptoms'	Normal daily activity affected
EHRA IV	'Disabling symptoms'	Normal daily activity discontinued

Table I Modified EHRA (mEHRA) classification

mEHRA score	Symptoms	Description
1	None	
2a	Mild	Normal daily activity not affected, symptoms not troublesome to patient
2b	Moderate	Normal daily activity not affected but patient troubled by symptoms
3	Severe	Normal daily activity affected
4	Disabling	Normal daily activity discontinued

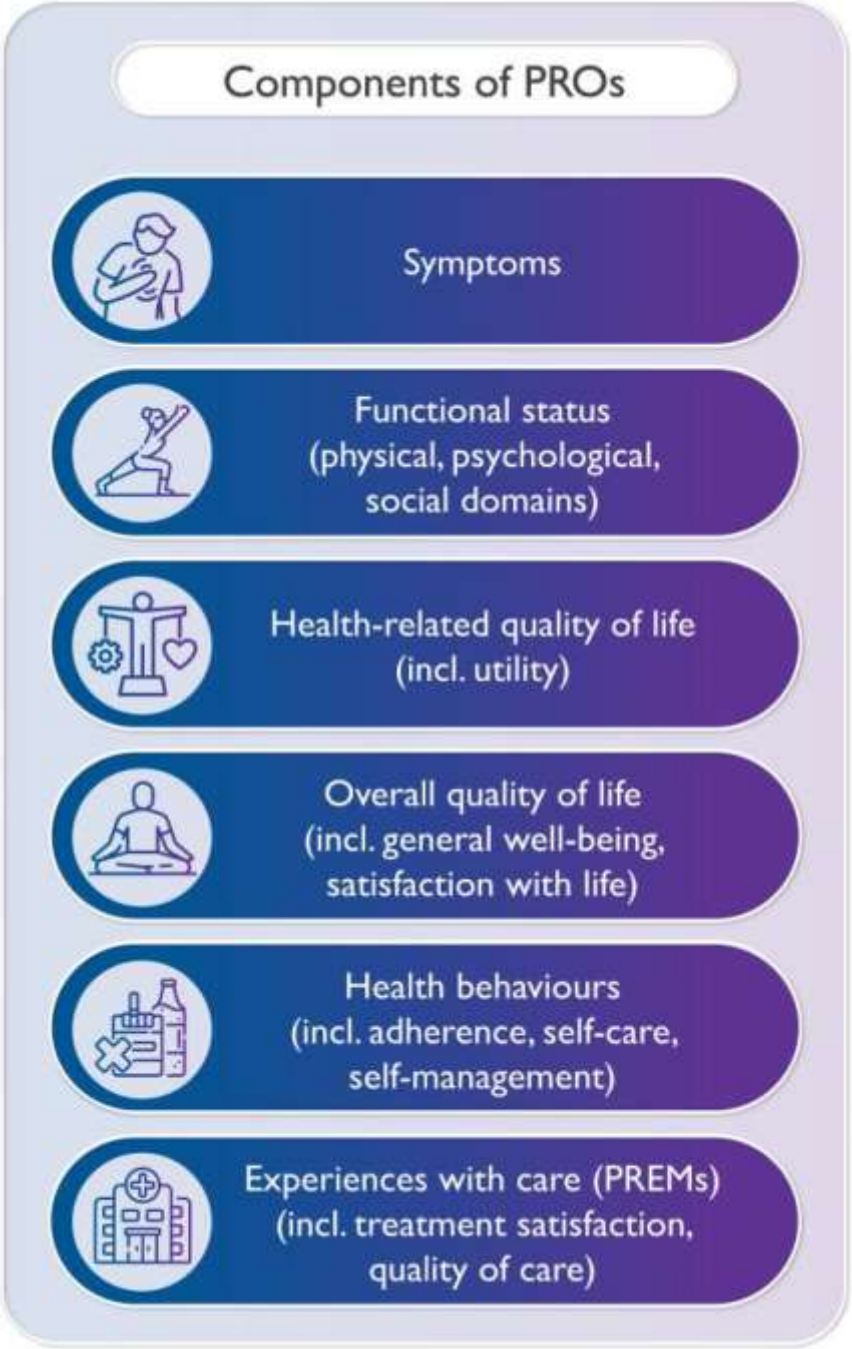
Underlined text represents the modification to the original descriptions of EHRA classes.

Kirchhof P, et al. *Eur Heart J* 28:2803-17 (2007)
The 2010 ESC atrial fibrillation guidelines. *Eur Heart J* 31:2369-429.(2010)
Wynn GJ, et al. *Europace* 16:965-72 (2014)
All ESC atrial fibrillation guidelines from 2016 onwards recommend the mEHRA score.

Many valid PRO and PRE instruments are available for cardiovascular diseases

PROs in quality monitoring and improvement

There is a growing awareness that PROs have a place in the evaluation of quality of care. This is rooted in the concept of value-based healthcare, which is defined as improving patient-relevant outcomes, relative to the cost per patient for achieving these improvements.[180] In this respect, PRO-based performance measures, also known as PRO-based quality indicators, are of key importance.[20] PRO-based performance measures entail an aggregation of information collected through PROMs or PREMs.[20,21] Data are aggregated for an accountable healthcare entity, such as a ward, a hospital, or a home care agency.[21] Performance measures are preferably expressed as ratios. An example is the percentage of patients with depressive feelings, as shown by a score of >9 on the Patient Health Questionnaire-9 items (PHQ9), who have a follow-up score of <5 at 6 months. The higher the percentage, the better the care that has been provided, because the goals of treatment and care have been reached. Quality indicators that are linked to ESC guidelines that encompass PROMs and PREMs[8,181,182] are particularly useful for monitoring the quality of care from patients' perspectives. It is important that performance measures are risk-adjusted.[183]



ACCNAP, ACVC, EAPCI, EAPC, HFA, EHRA, EACVI, ESC and ESC committees: PROs in clinical practice. Moons P, et al. *Eur Heart J* 44:3405-22.(2023)

Weiterqualifikation 2024

PRE to improve patient experience at the UKE

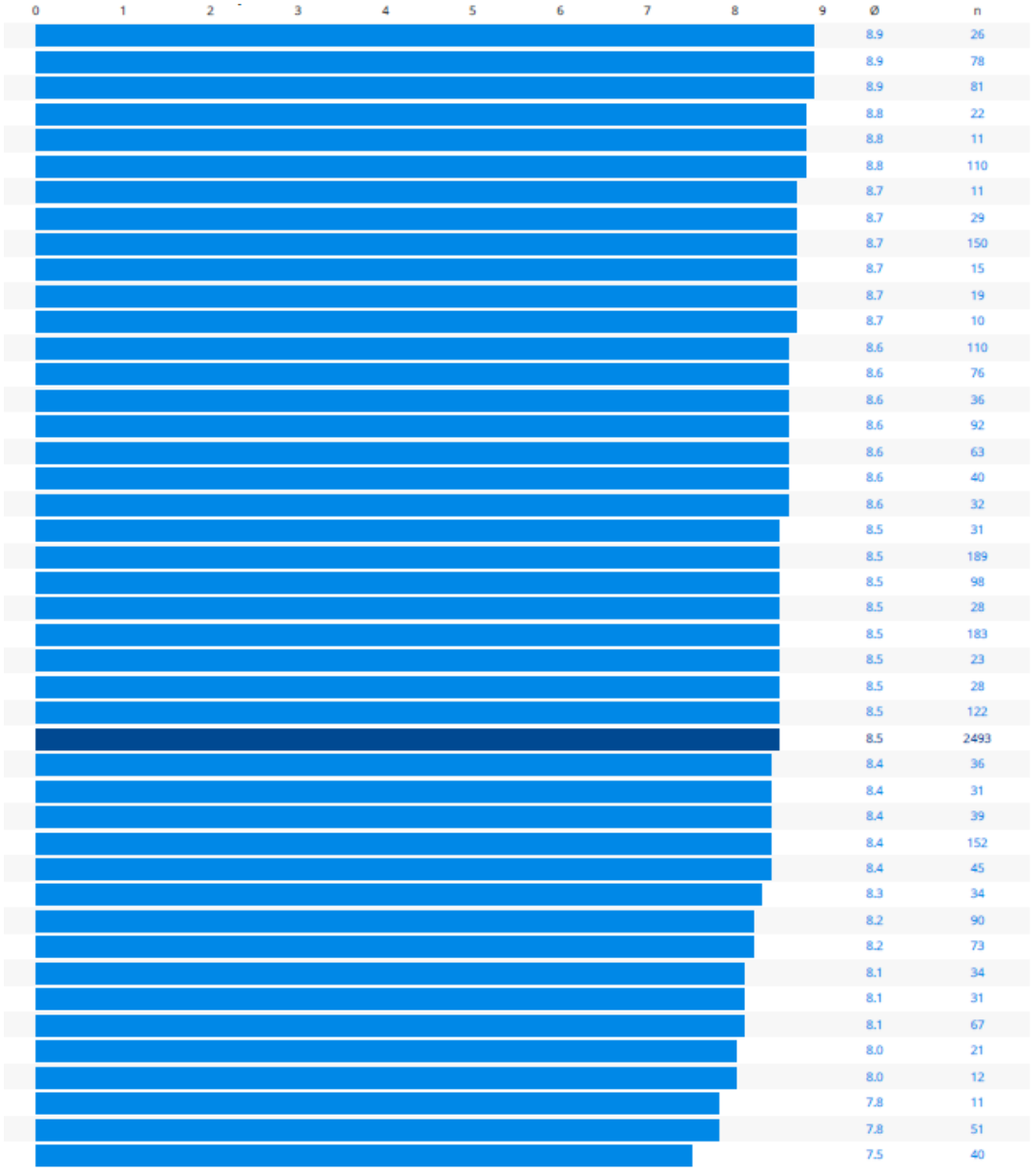
Ongoing patient survey of key patient needs with annual report for each department / ward

1. Physicians and nurses are trustworthy
2. I get clear information on health status
3. I am treated with respect and dignity
4. I am free of pain
5. Staff is friendly, approachable, and available

Cleanliness

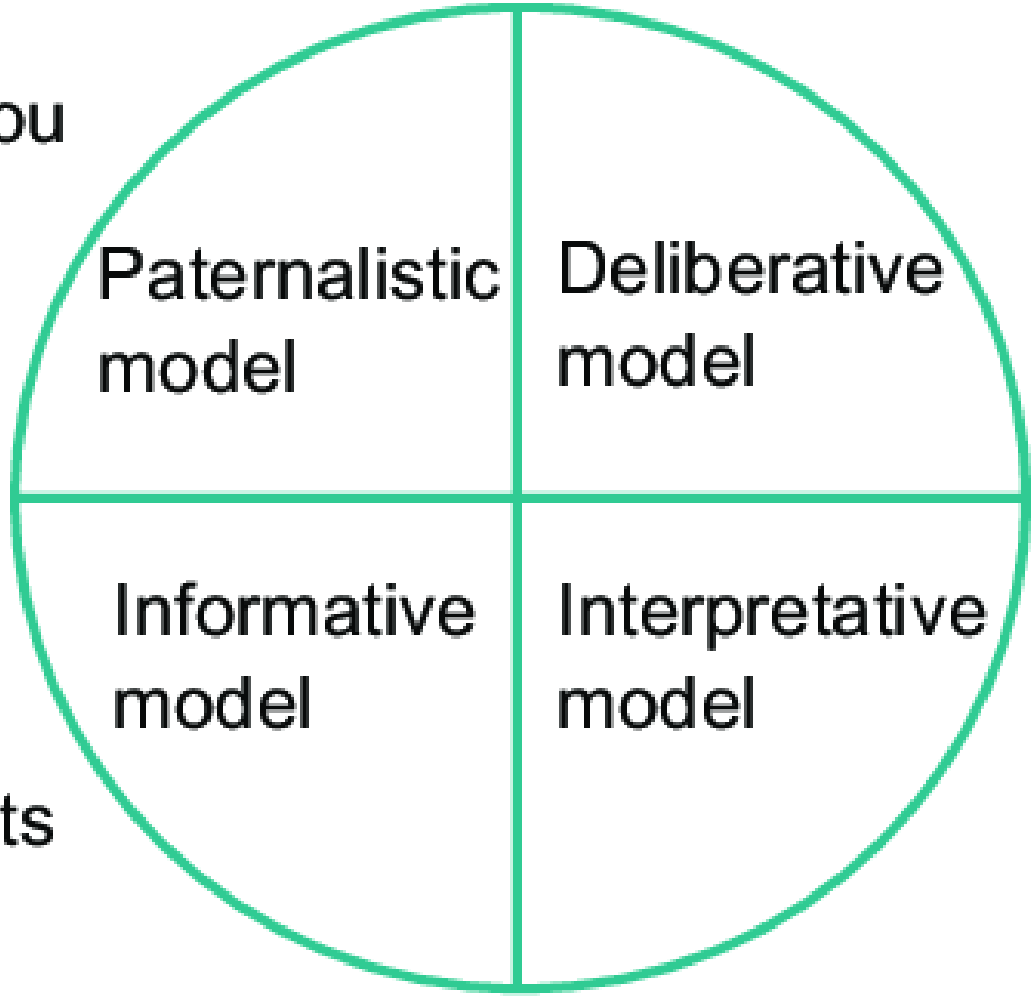
Short waiting times

Good food



PRO and PED can improve rapport of patients and health care professionals

I decide for you



In addition,
I tell you
my preferences

I give you facts

I give you facts
and help you
to find
your preferences

Yes of course!

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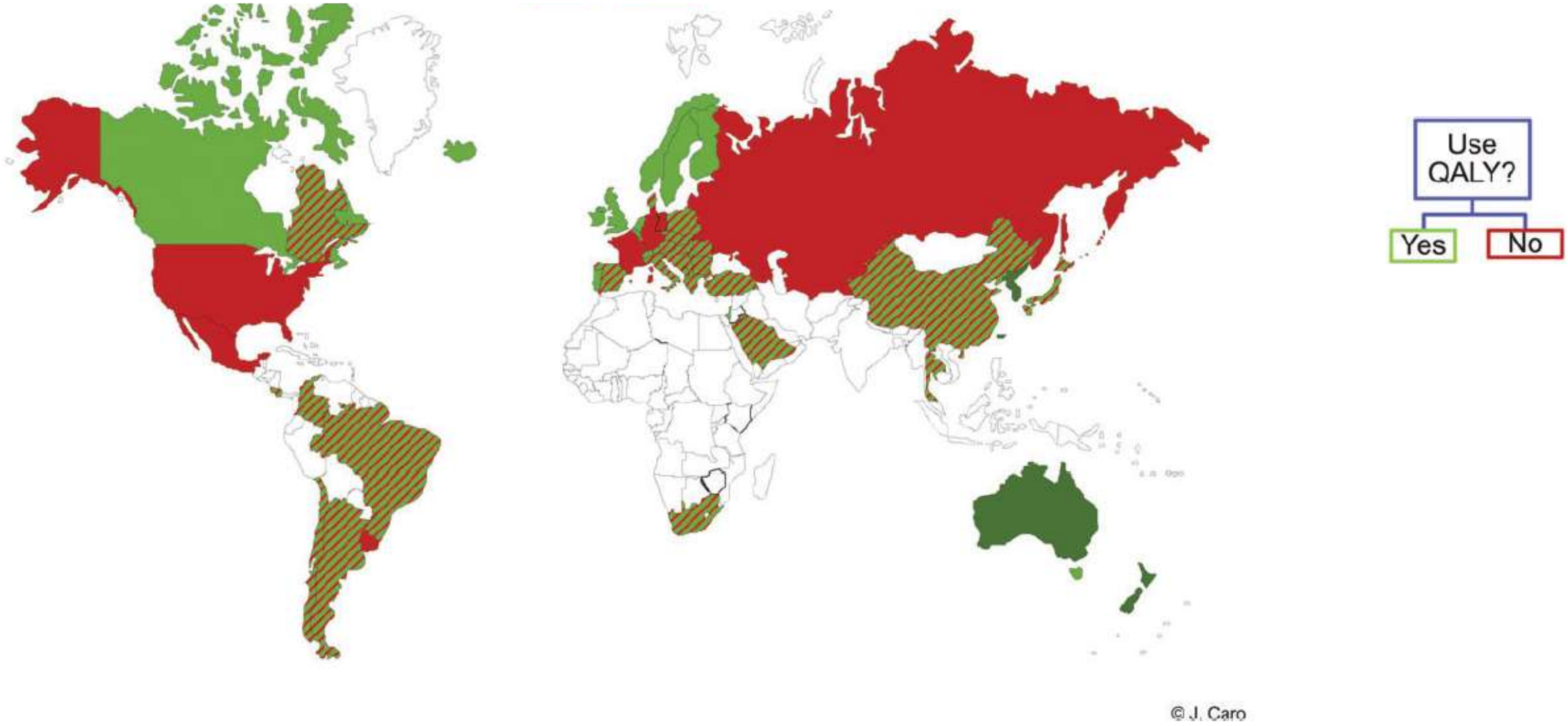
…as long as the information is viewed in context

Use of quality of life-adjusted life years (QALY) 2014



ESC CRT position paper on patient-reported outcomes in research. Anker SD, et al. *Eur Heart J* 35:2001-9.(2014)

Yes as long as the data are valid and reliable

How are you today?

Thank you, very good (I am polite)

Thank you, very good (but don´t ask me about last week)

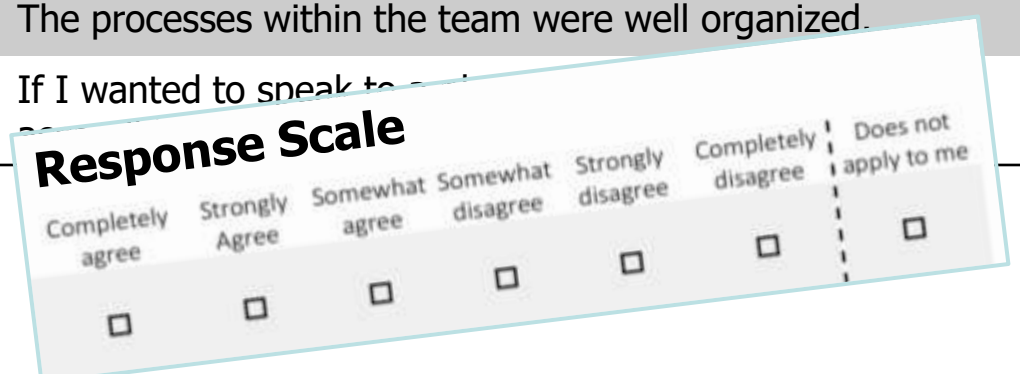
So-and-so (I got a speeding ticket on my way here)

No answer (I hate the place / you, therefore I will not answer)

Je ne comprends rien

An UKE patient experience questionnaire (EPAT-16)

1.	The healthcare professionals were sensitive (for example they addressed my feelings, showed understanding, or empathized with my situation).
2.	I trusted my healthcare professionals.
3.	My wishes, needs and expectations were asked and taken into account in the treatment.
4.	My entire personal life was taken into account during the treatment (for example, job, family and friends, partnership and sexuality, culture and religion, age, or financial circumstances).
5.	I was given enough time to describe my concerns and my situation (for example, medical history or current symptoms).
6.	I was asked if I use or would like to use additional services (for example, support groups, counseling, health courses, complementary and alternative medicine, or spiritual support/pastoral care).
7.	The processes within the team were well organized.
8.	If I wanted to speak to a



9.	It was discussed with me whether follow-up appointments would be useful (for example, for aftercare or further treatment).
10.	I was encouraged to speak up if I noticed inconsistencies in my treatment.
11.	I received information about my condition from my healthcare professionals (for example, causes, symptoms, effects or course).
12.	I was an equal partner with my healthcare professionals (for example, in making decisions or sharing information).
13.	I was informed about the options for involving my family members in the treatment (for example, accompanying to appointments, participating in conversations, or assisting with medication intake). .
14.	I was encouraged to improve my health by changing my behavior (for example, through diet, exercise, reducing tobacco or alcohol). .
15.	When I had pain, I was helped quickly.
16.	The healthcare professionals addressed my fears and concerns (for example, by showing understanding and providing encouragement).

Christalle E, et al. *BMJ Qual Saf*.(2024)
Christalle E, et al. *Health Expect* 25:1529-38.(2022)

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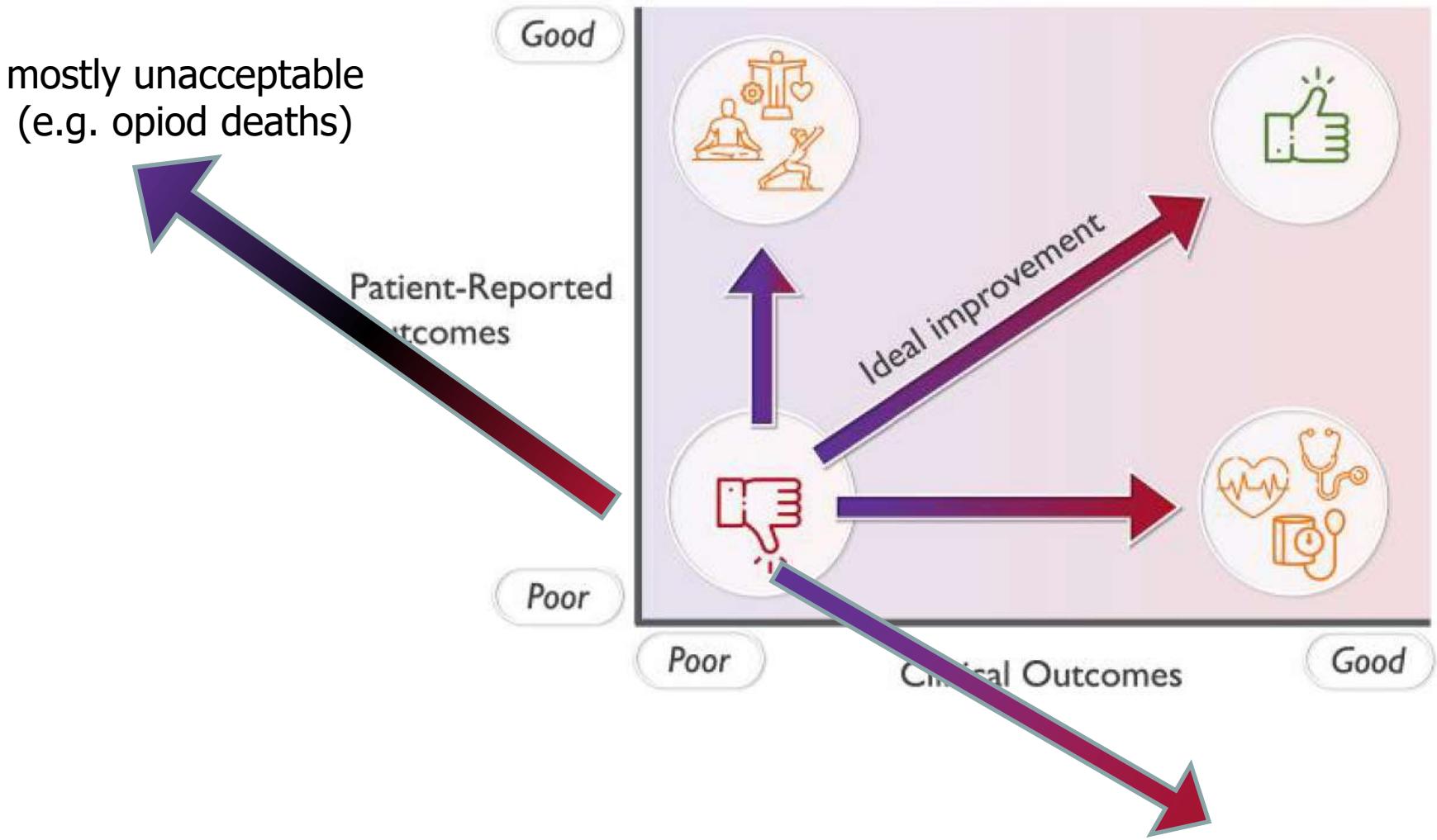
Patient well-being (measured by PRO/PRE/PED) in context

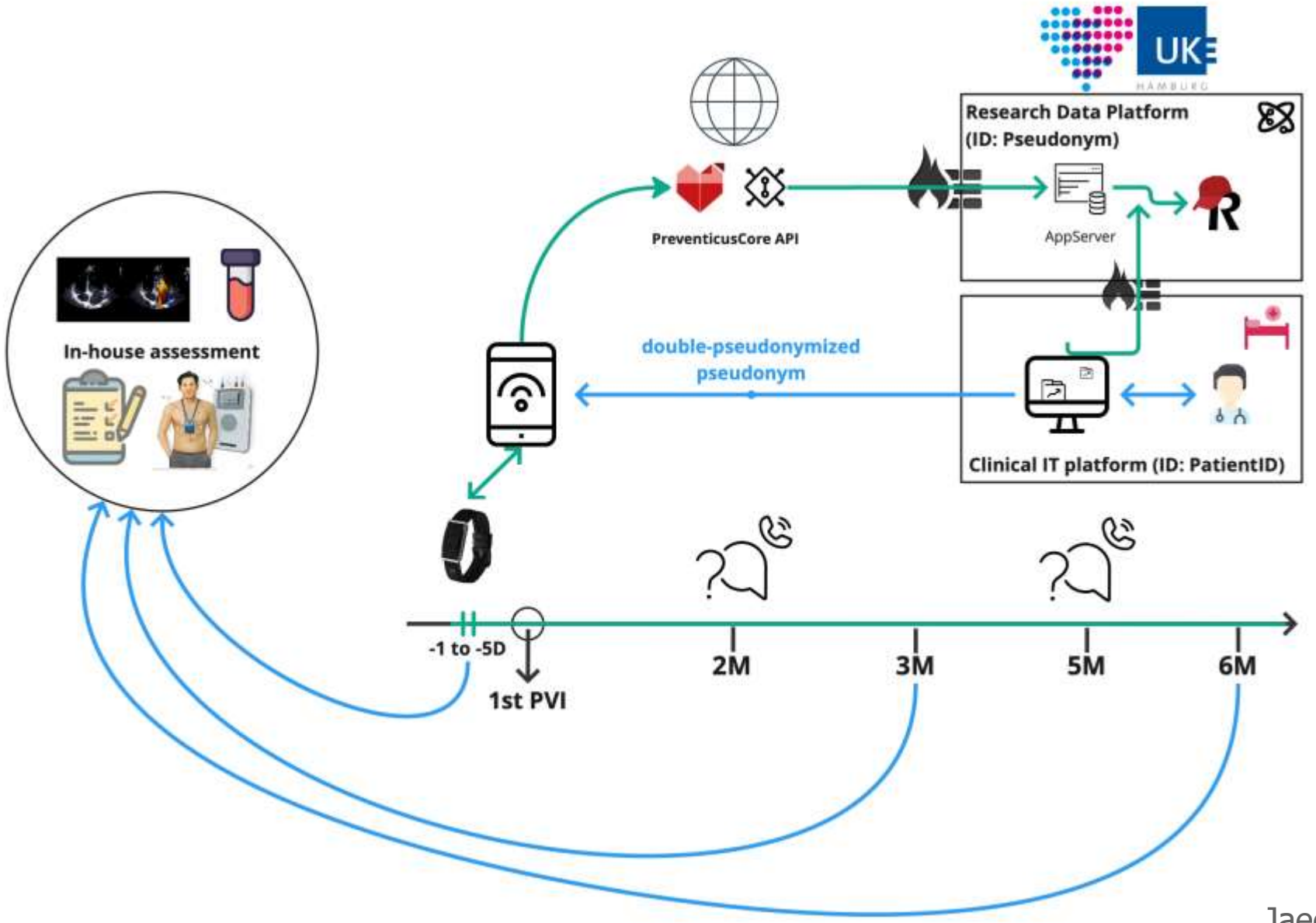


modified from Moons P, et al. *Eur Heart J* 44:3405-22.(2023)

The future is digital

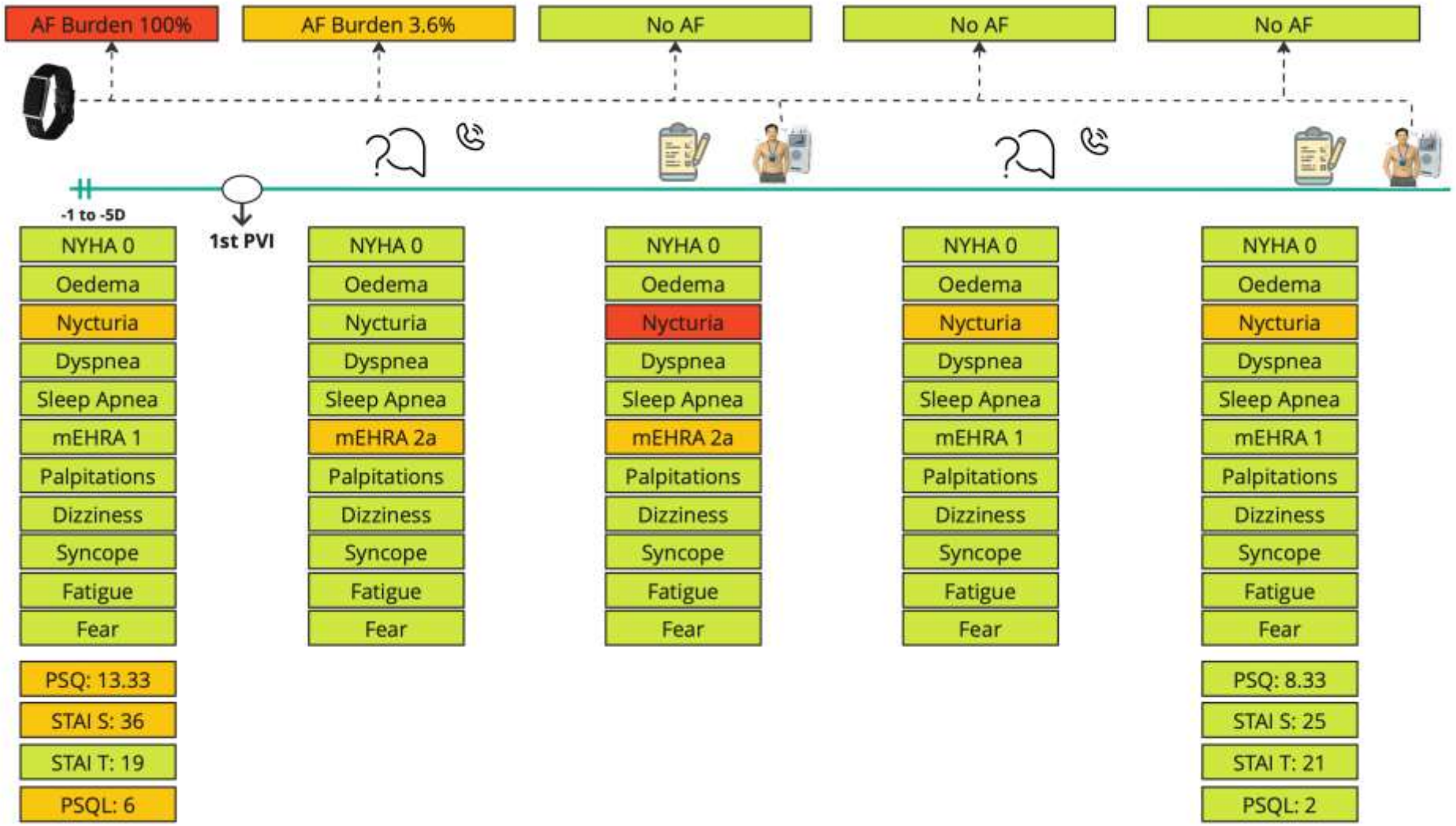
ePRO / ePED

I Wear AF Trial: Blended follow-up of patients with atrial fibrillation



Jaeckle S, Obergassel J, work in progress
wearable rhythm monitoring adapted from Fabritz L et al, *Eur Heart J Digit Health* 3:610-625.(2022)

I Wear AF trial: PRO improvement in a patient with asymptomatic atrial fibrillation (anxiety, stress sleep quality)



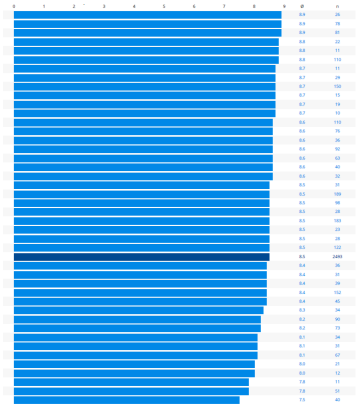
Jaeckle S, Obergassel J, work in progress

For the emerging role of AF burden see Becher N, et al. *Eur Heart J* 45:2824-2838 (2024) doi: 10.1093/eurheartj/ehae373.

Summary: PRO / PED describe unmet medical needs

PREs identify medical needs at the UKE

- valid and reliable instruments
- representative populations
- seen in context
- The future is digital



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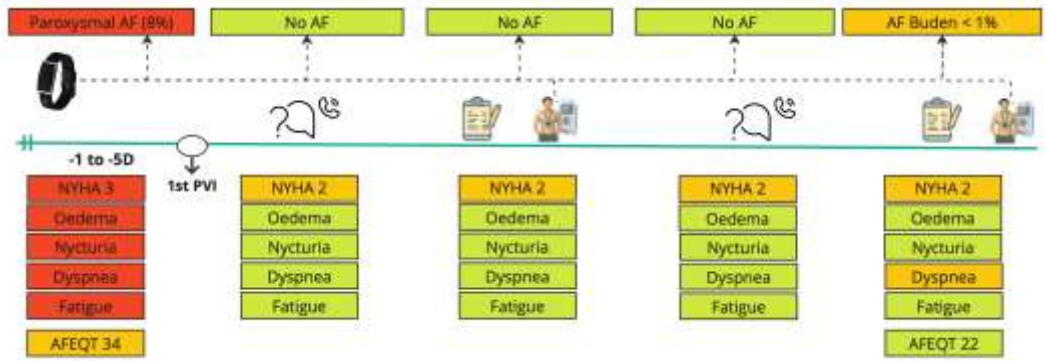
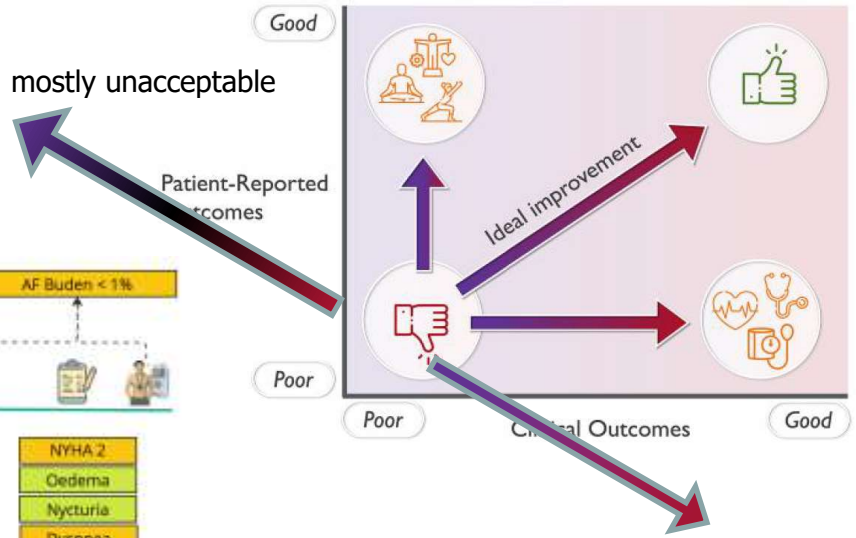
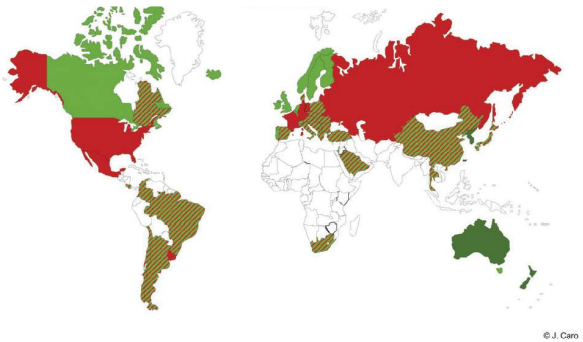
A patient with asymptomatic AF





may be acceptable in severe illness
(end of life care)



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