PROCARD
Patient - Reported Outcomes in Cardiology Research Group

Patient-reported Outcomes Measures

CRT Plenary Meeting

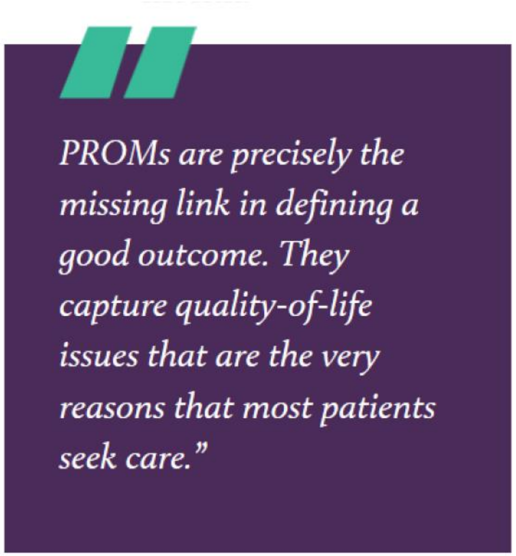
“Developing partnerships between patients, health professionals and the European Society of Cardiology”

Professor **Tone M Norekvål,** RN, MSc, PhD, FAHA, FESC
Haukeland University Hospital / University of Bergen
Norway

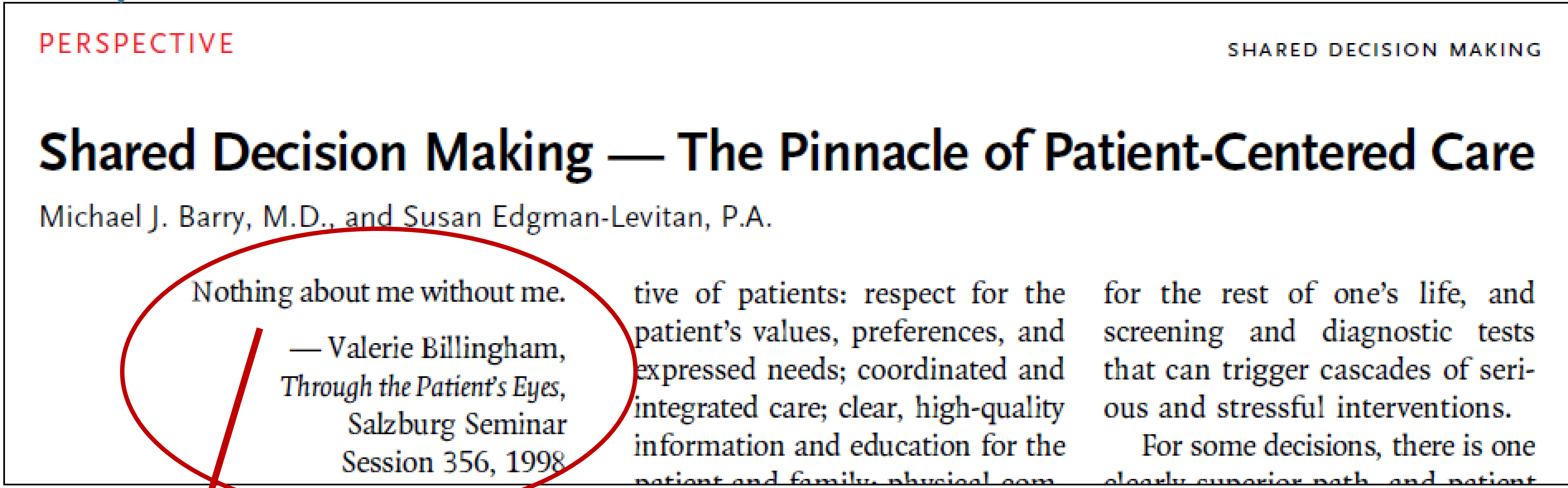
HELSE BERGEN
Haukeland University Hospital

Agenda

- Why patient-reported outcomes measures?
- Choice of tools and methods
- PROMS in:
  - Clinical trials
  - Registers
  - Clinical practice



PROMs are precisely the missing link in defining a good outcome. They capture quality-of-life issues that are the very reasons that most patients seek care.”



PERSPECTIVE

SHARED DECISION MAKING

Shared Decision Making — The Pinnacle of Patient-Centered Care

Michael J. Barry, M.D., and Susan Edgman-Levitan, P.A.

> Nothing about me without me.
>
> — Valerie Billingham, *Through the Patient's Eyes,* Salzburg Seminar Session 356, 1998

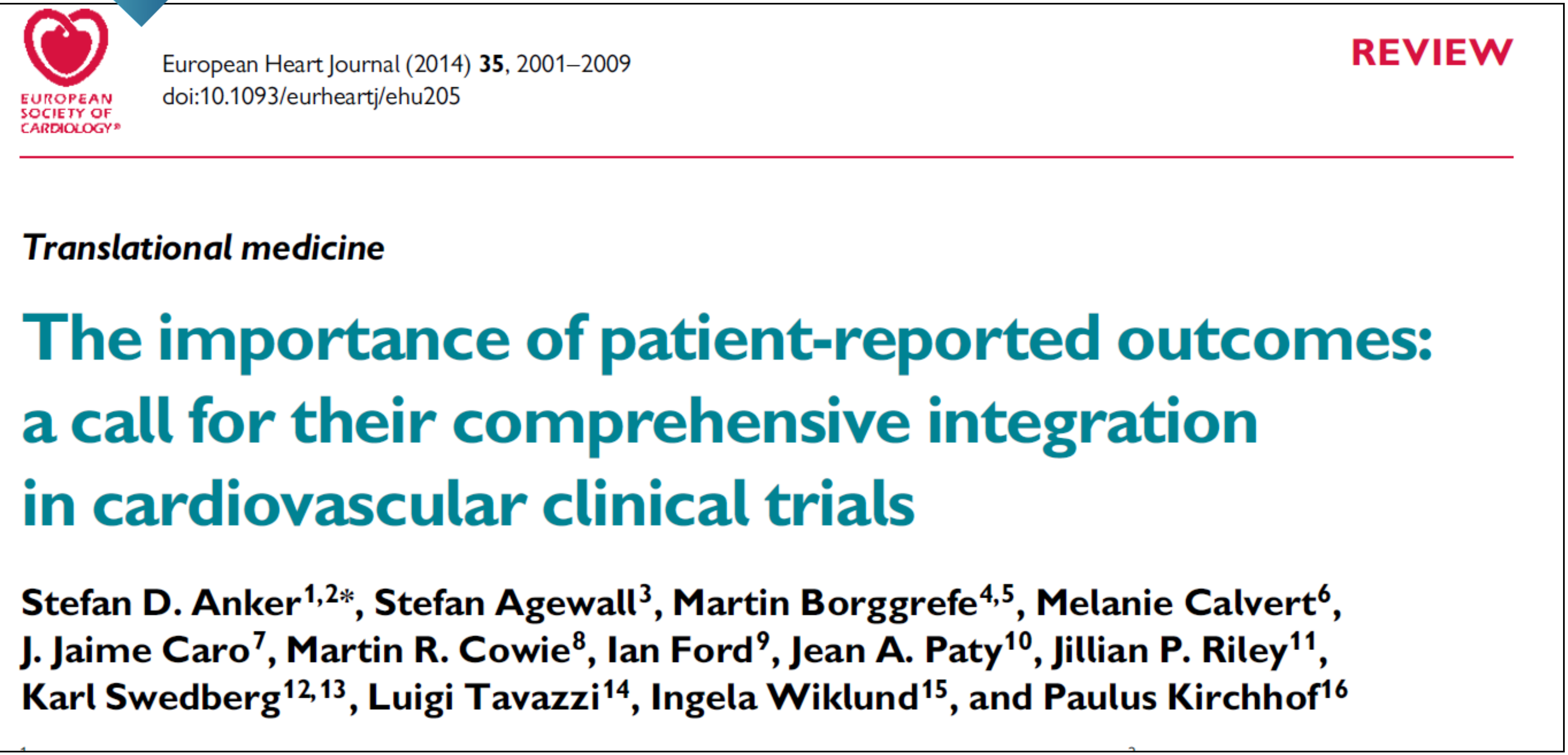
tive of patients: respect for the patient's values, preferences, and expressed needs; coordinated and integrated care; clear, high-quality information and education for the

for the rest of one's life, and screening and diagnostic tests that can trigger cascades of serious and stressful interventions.

For some decisions, there is one

- *«Nothing about me without me»*

(Barry MJ & Edgman-Levitan S, NEJM 2012: 366; 9.)



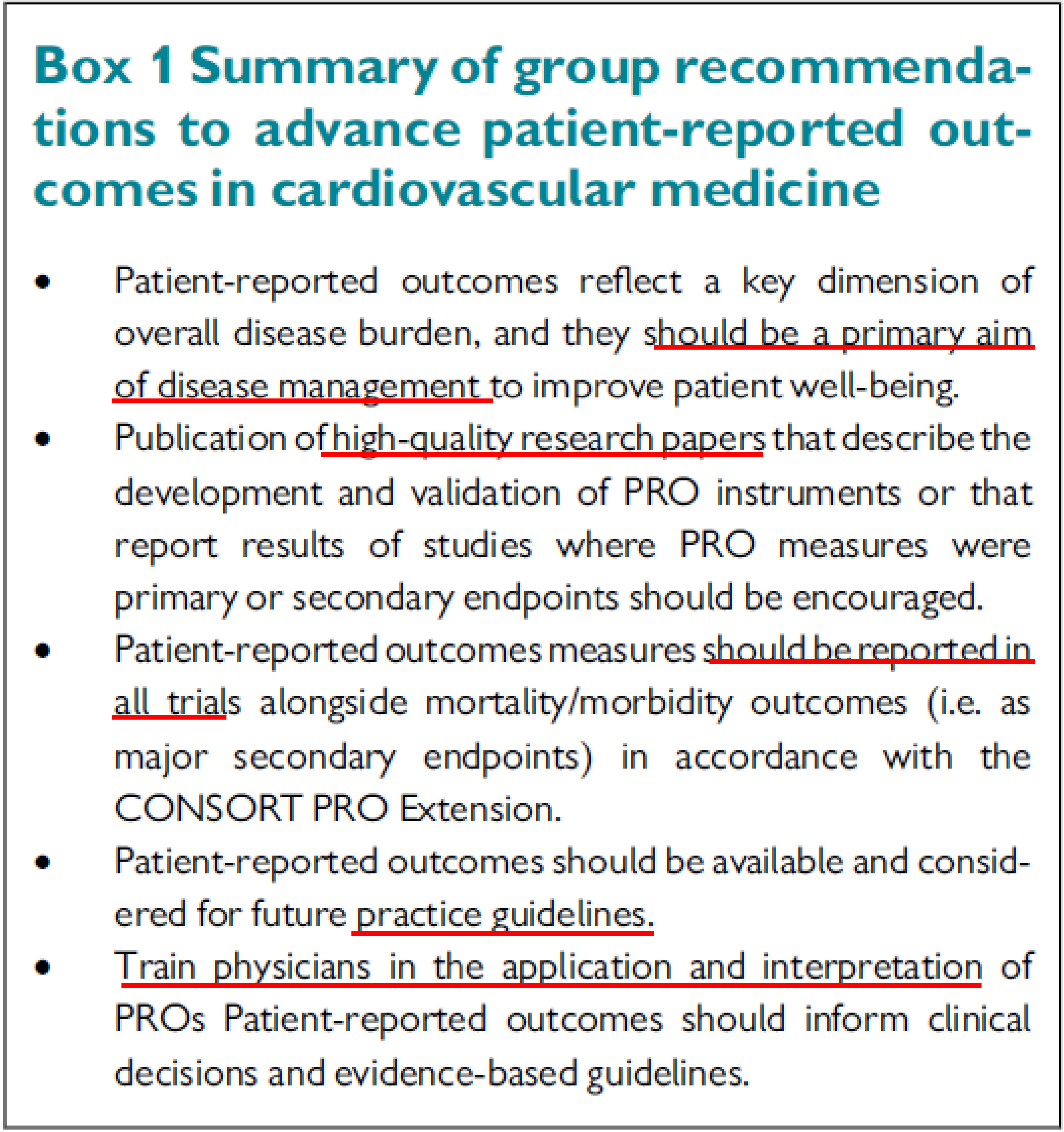
European Heart Journal (2014) **35**, 2001–2009
doi:10.1093/eurheartj/ehu205

REVIEW

Translational medicine

The importance of patient-reported outcomes: a call for their comprehensive integration in cardiovascular clinical trials

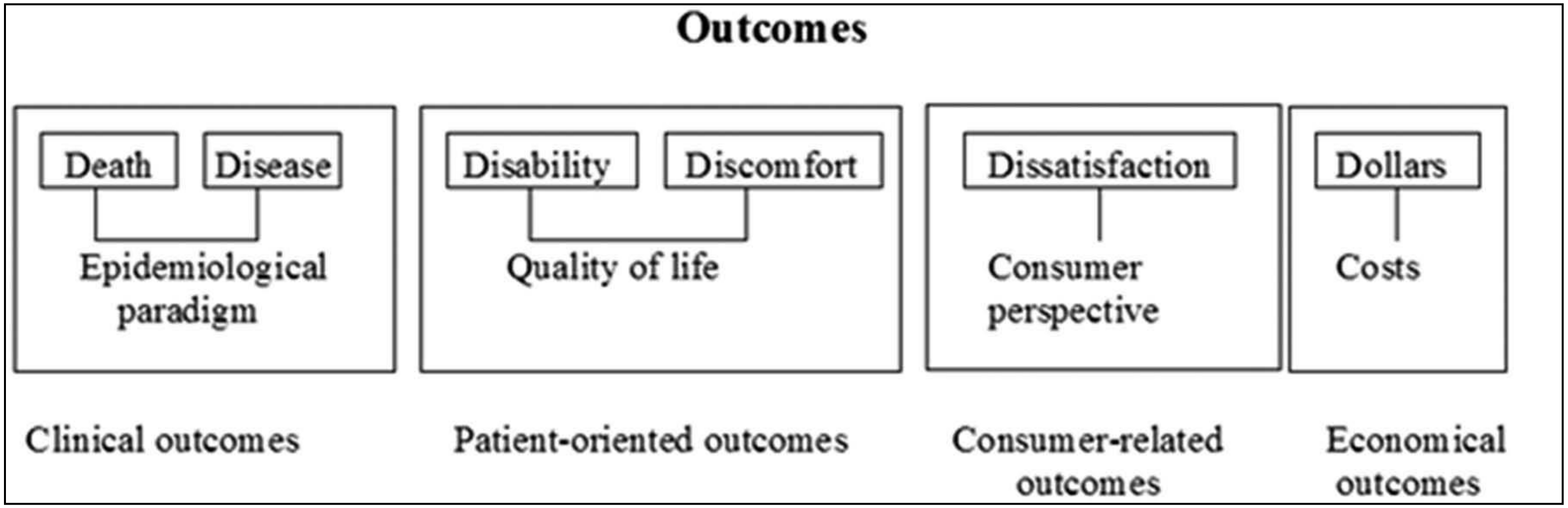
Stefan D. Anker[1,2]*, Stefan Agewall[3], Martin Borggrefe[4,5], Melanie Calvert[6], J. Jaime Caro[7], Martin R. Cowie[8], Ian Ford[9], Jean A. Paty[10], Jillian P. Riley[11], Karl Swedberg[12,13], Luigi Tavazzi[14], Ingela Wiklund[15], and Paulus Kirchhof[16]



Box 1 Summary of group recommendations to advance patient-reported outcomes in cardiovascular medicine

- Patient-reported outcomes reflect a key dimension of overall disease burden, and they should be a primary aim of disease management to improve patient well-being.
- Publication of high-quality research papers that describe the development and validation of PRO instruments or that report results of studies where PRO measures were primary or secondary endpoints should be encouraged.
- Patient-reported outcomes measures should be reported in all trials alongside mortality/morbidity outcomes (i.e. as major secondary endpoints) in accordance with the CONSORT PRO Extension.
- Patient-reported outcomes should be available and considered for future practice guidelines.
- Train physicians in the application and interpretation of PROs Patient-reported outcomes should inform clinical decisions and evidence-based guidelines.

The 6Ds model of health outcomes

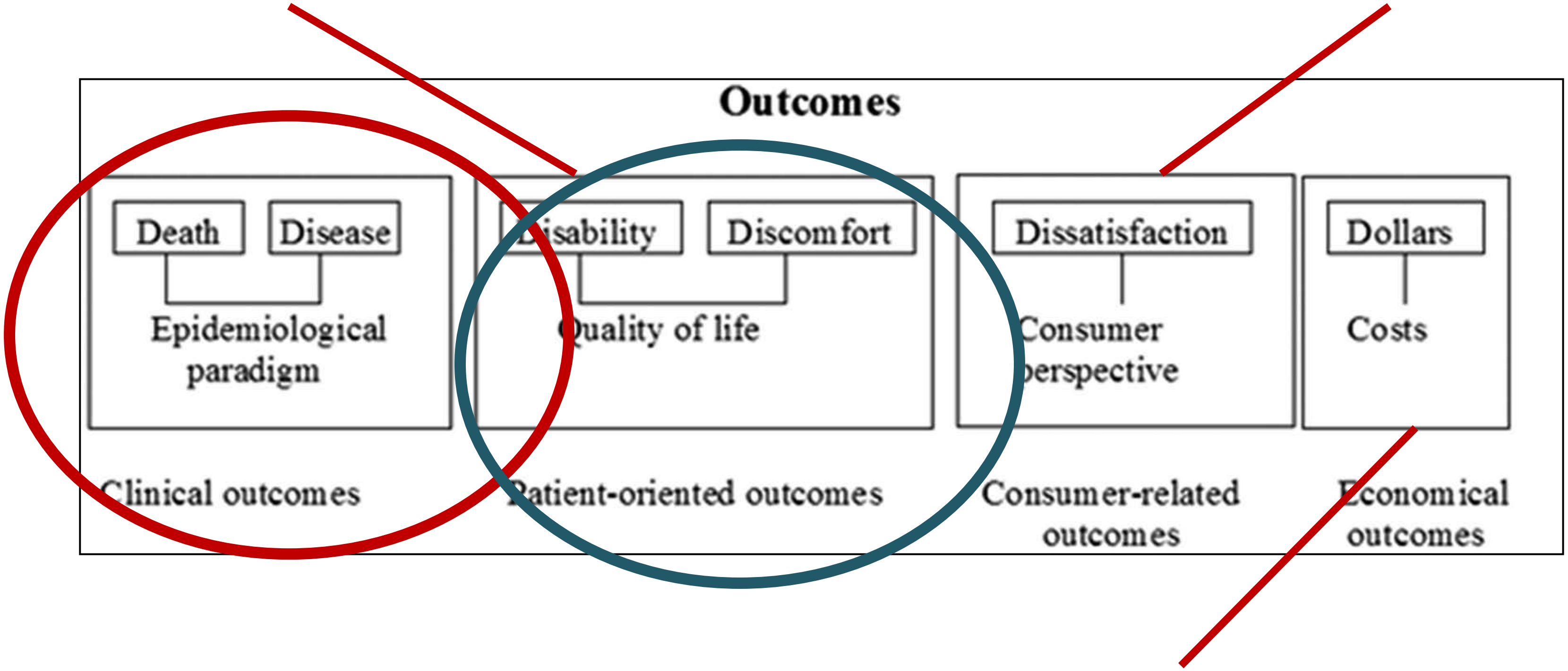


(Radosevich DM and Werni TLK. *A practical guidebook for implementing, analyzing, and reporting outcomes measurement*. Health Outcomes Institute, 1996, p. 1–10.)

The 6Ds model of health outcomes

PROM

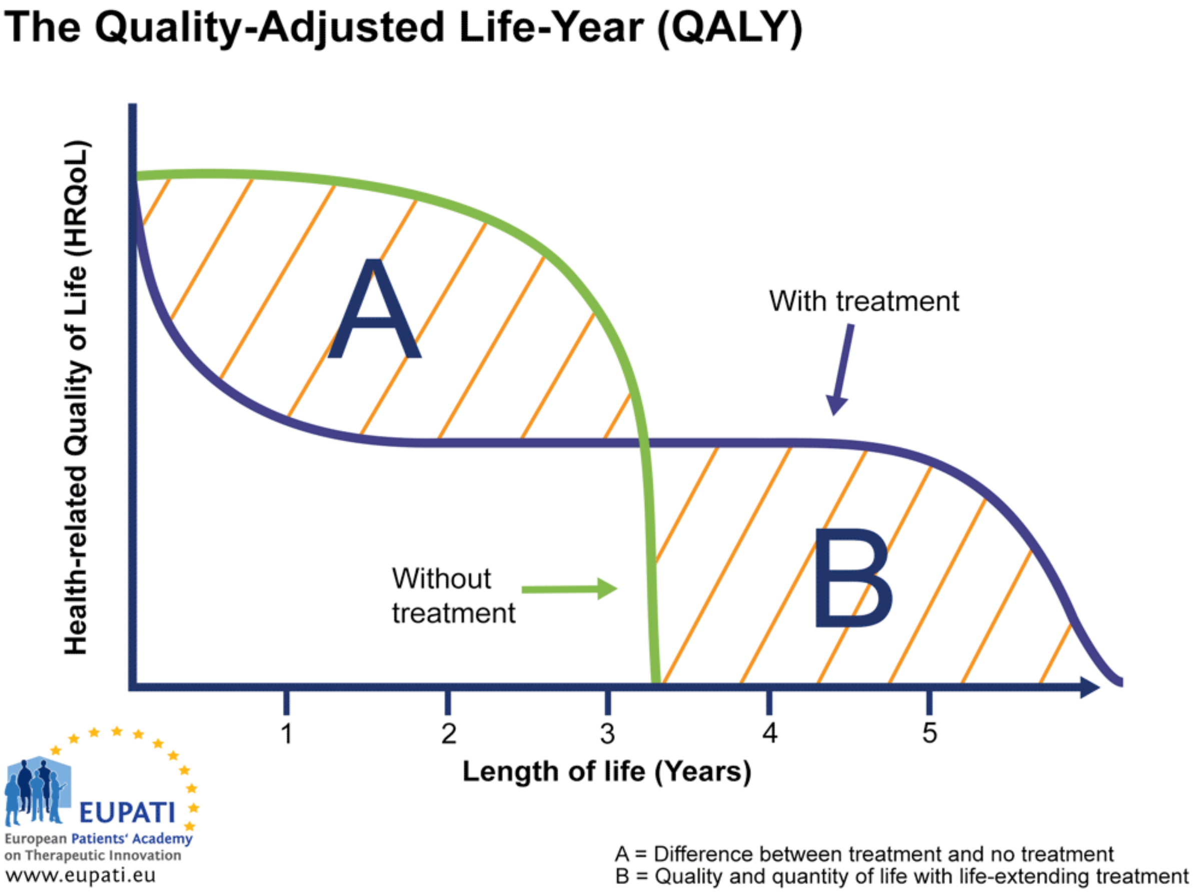
PREM



QUALYS

(Radosevich DM and Werni TLK. *A practical guidebook for implementing, analyzing, and reporting outcomes measurement*. Health Outcomes Institute, 1996, p. 1–10.)

QUALY



The Quality-Adjusted Life-Year (QALY)

A = Difference between treatment and no treatment
B = Quality and quantity of life with life-extending treatment

QALY: attempts to represent the impact a therapy has on the length of life while also taking into account any changes in the health-related quality of life (HRQoL). HRQoL is calculated on a scale where 0 = ‘death’ and 1 = ‘perfect’ health (the scale also allows for negative scores).

EUPATI

SF-6D

EQ-5D

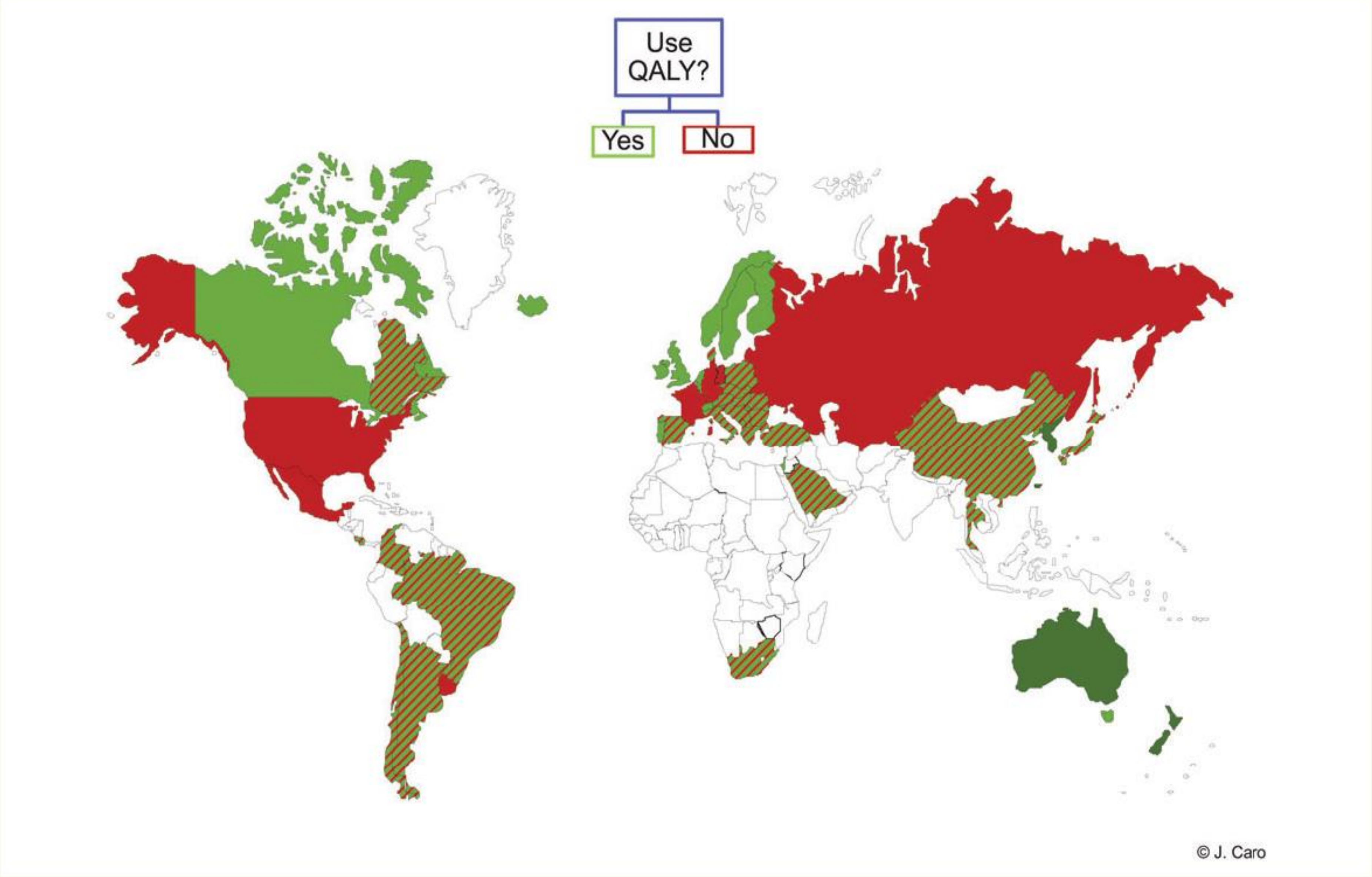
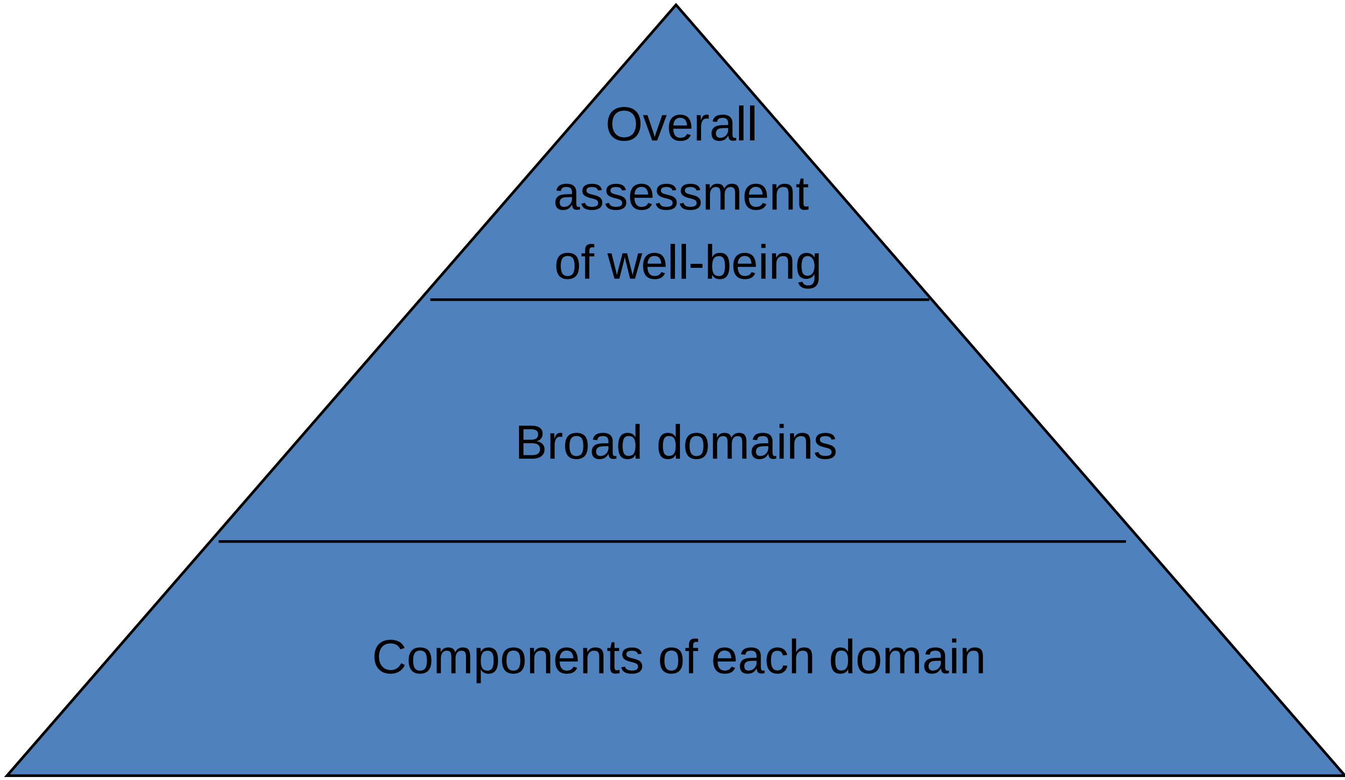


Figure 3 Global use of quality-adjusted life years to inform reimbursement decisions. Courtesy: Jaime Caro.

Levels of PROMS



Global items

Overall assessment of well-being

Generic instruments

Broad domains

Disease specific

Components of each domain

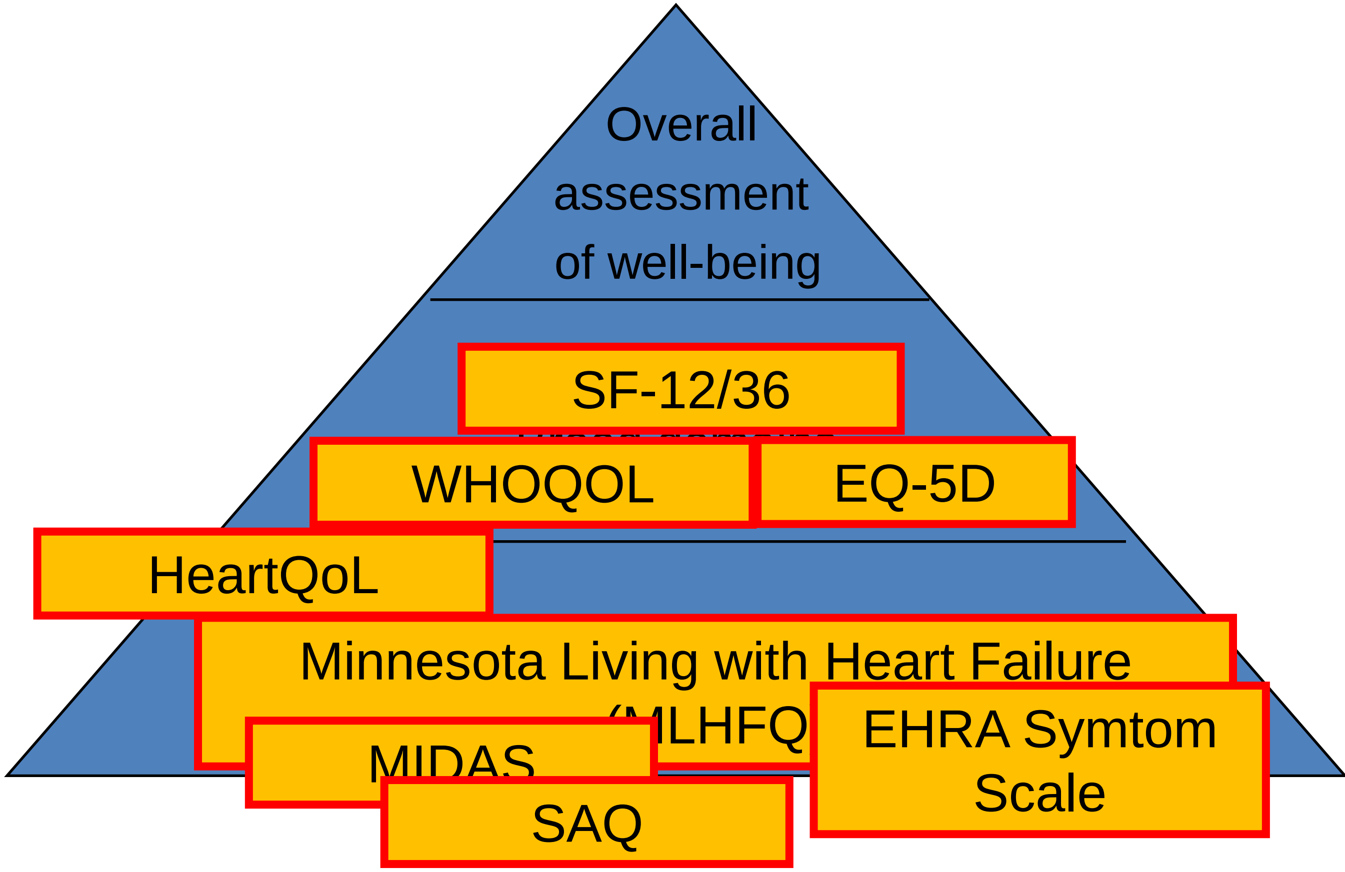
(Adapted from Spilker B 1996)

Levels of PROMS

Global items

Generic instruments

Disease specific



Overall assessment of well-being

SF-12/36

WHOQOL

EQ-5D

HeartQoL

Minnesota Living with Heart Failure (MLHFQ

MIDAS

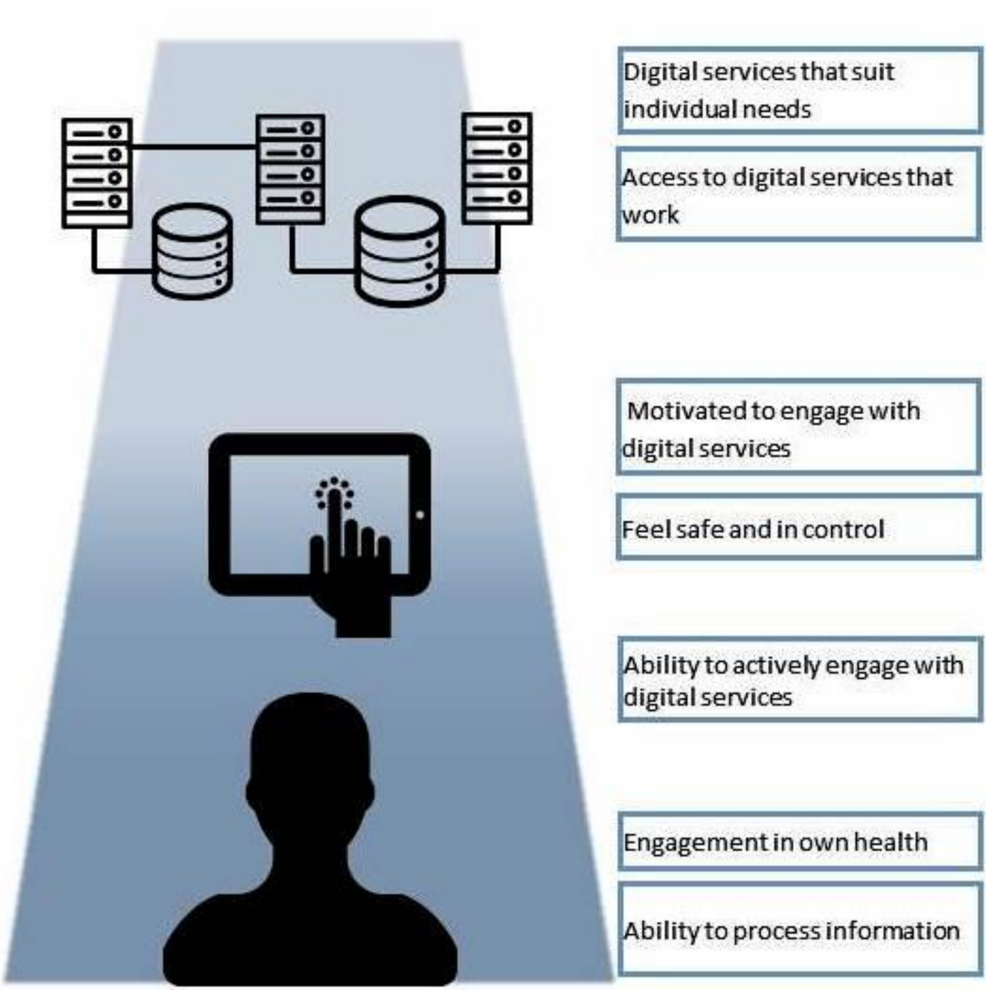
SAQ

EHRA Symtom Scale

(Adapted from Spilker B 1996)

Electronic health (eHealth) literacy

- How to describe and evaluate users’ digital capabilities and experiences?
- PROMS for eHealth literacy:
  - **eHEALS** (Normann and Skinner 2006)
    - 8 items; combined knowledge, comfort, and perceived skills at finding, evaluating, and applying electronic health information to health problems
  - **eHealth Literacy Questionnaire** (Kayser et al 2018)
    - 35 items; attributes of users, users ad technologies, and users experience of systems



Digital services that suit individual needs

Access to digital services that work

Motivated to engage with digital services

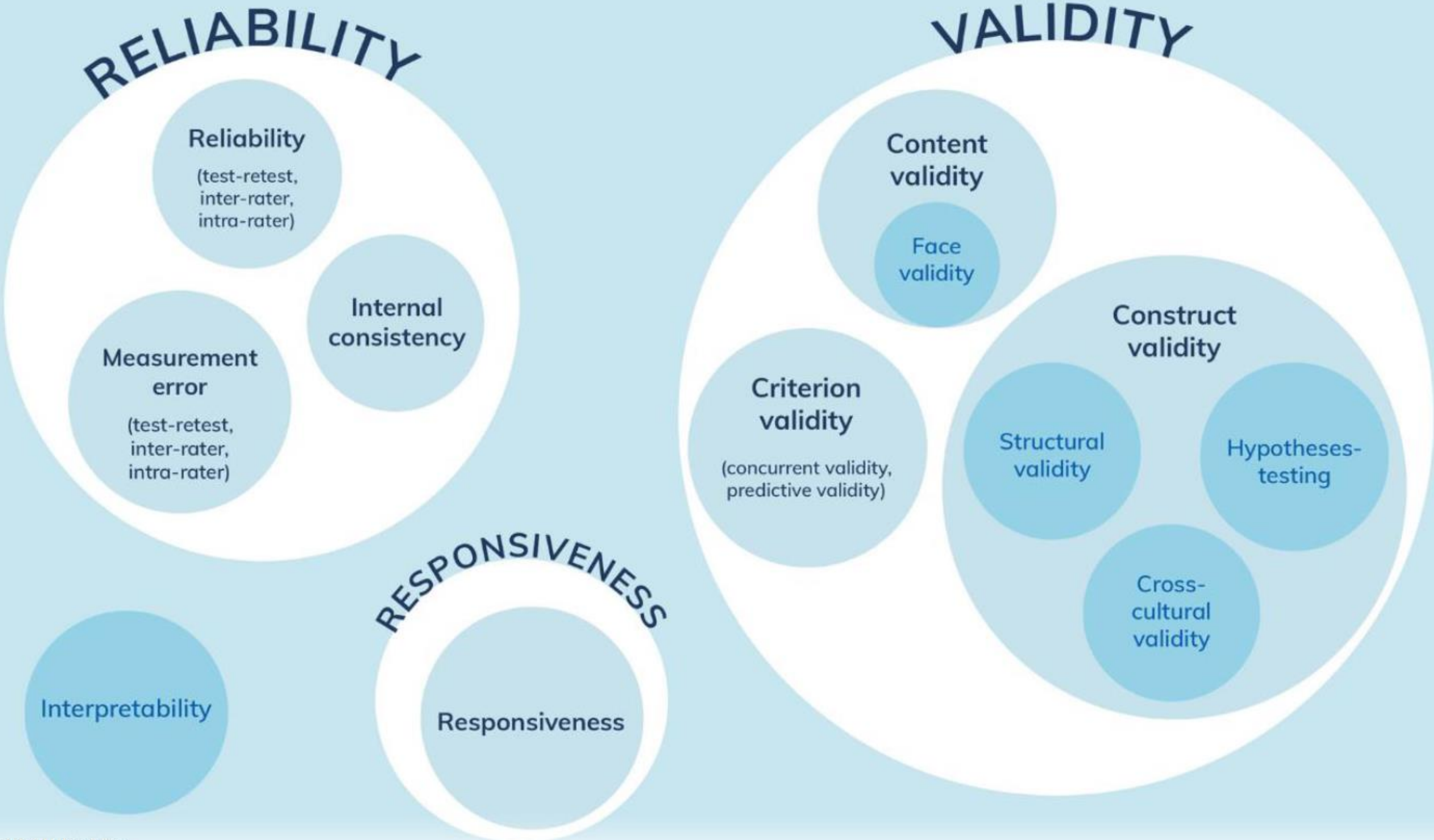
Feel safe and in control

Ability to actively engage with digital services

Engagement in own health

Ability to process information

Measurement Properties of Outcome Measurement Instruments



RELIABILITY

Reliability

(test-retest, inter-rater, intra-rater)

Internal consistency

Measurement error

(test-retest, inter-rater, intra-rater)

VALIDITY

Content validity

Face validity

Construct validity

Structural validity

Hypotheses-testing

Cross-cultural validity

Criterion validity

(concurrent validity, predictive validity)

RESPONSIVENESS

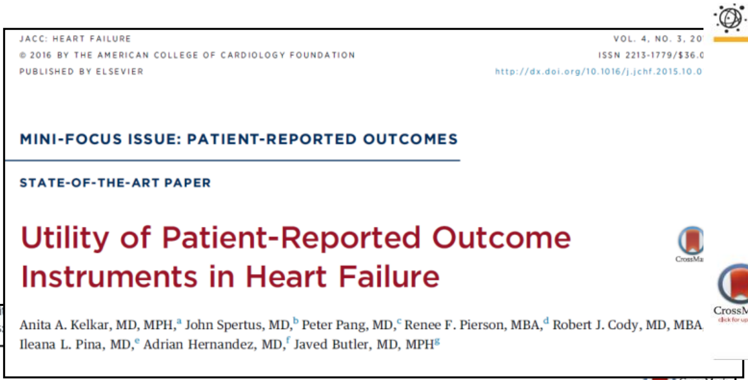
Responsiveness

Interpretability

COSMIN Taxonomy

Systematic reviews of PROMs

- The number of systematic reviews of PROMs is increasing
- COSMIN guidelines for systematic reviews of PROMS *(Prinsen CAC et al. Qual Life Res 2018;27:11447-1157)*



JACC: HEART FAILURE
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PUBLISHED BY ELSEVIER

VOL. 4, NO. 3, 20
ISSN 2213-1779/$36.0
http://dx.doi.org/10.1016/j.jchf.2015.10.0

MINI-FOCUS ISSUE: PATIENT-REPORTED OUTCOMES

STATE-OF-THE-ART PAPER

Utility of Patient-Reported Outcome Instruments in Heart Failure

Anita A. Kelkar, MD, MPH,[a] John Spertus, MD,[b] Peter Pang, MD,[c] Renee F. Pierson, MBA,[d] Robert J. Cody, MD, MBA, Ileana L. Pina, MD,[e] Adrian Hernandez, MD,[f] Javed Butler, MD, MPH[g]

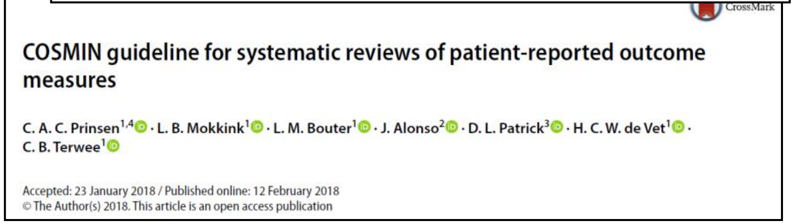
PLOS | ONE

RESEARCH ARTICLE

Patient-Reported Outcomes for Quality of Life Assessment in Atrial Fibrillation: A Systematic Review of Measurement Properties

Dipak Kotecha[1,2,4]*, Amar Ahmed[1], Melanie Calvert[3], Mauro Lencioni[4], Caroline B. Terwee[5], Deirdre A. Lane[1,2]

1 Institute of Cardiovascular Sciences, University of Birmingham, Birmingham, United Kingdom, **2** City Hospital, Sandwell and West Birmingham Hospitals NHS Trust, Birmingham, United Kingdom, **3** Institute of Applied Health Research, University of Birmingham, Birmingham, United Kingdom, **4** University Hospitals Birmingham NHS Trust, Birmingham, United Kingdom, **5** Department of Epidemiology and Biostatistics and the EMGO Institute of Health and Care Research, VU University Medical Center, Amsterdam, The Netherlands



COSMIN guideline for systematic reviews of patient-reported outcome measures

C. A. C. Prinsen[1,4] · L. B. Mokkink[1] · L. M. Bouter[1] · J. Alonso[2] · D. L. Patrick[3] · H. C. W. de Vet[1] · C. B. Terwee[1]

Accepted: 23 January 2018 / Published online: 12 February 2018
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Europace (2014) **16**, 787–796
doi:10.1093/europace/eut369

REVIEW

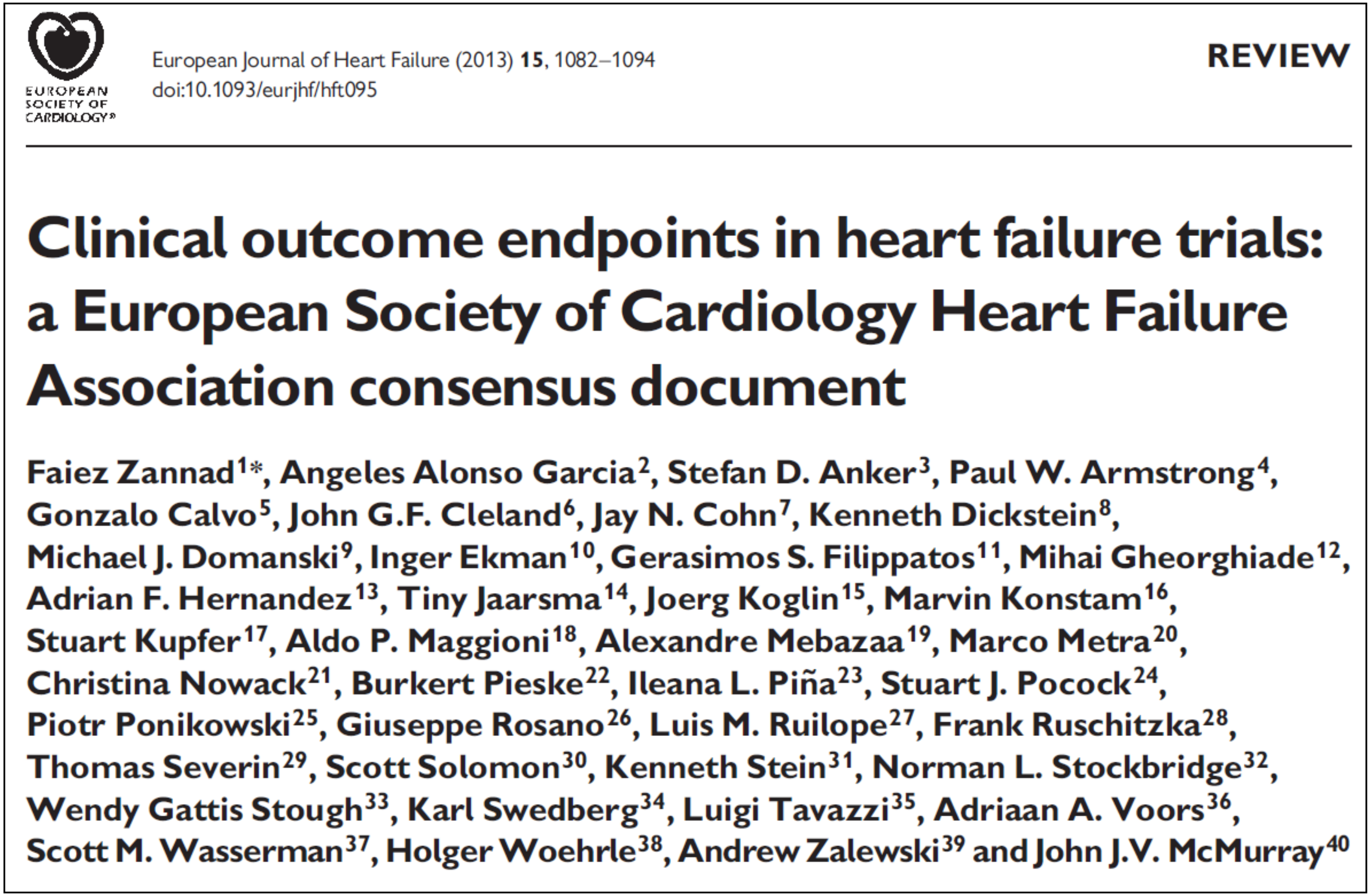
Quality of life in patients with atrial fibrillation: how to assess it and how to improve it

Etienne Aliot[1]*, Giovanni L. Botto[2], Harry J. Crijns[3], and Paulus Kirchhof[4,5]

[1]Cardiology Department, Institut Lorrain du Coeur et des Vaisseaux, CHU de Nancy, 54500 Vandoeuvre-lès-Nancy Cedex, France; [2]Cardiology Department, S. Anna Hospital, Como, Italy; [3]Department of Cardiology and CARIM, Maastricht University Medical Centre, Maastricht, The Netherlands; [4]University of Birmingham Centre for Cardiovascular Sciences and SWBH NHS Trust, Institute for Biomedical Research, Birmingham B15 2TT, UK; and [5]Department of Cardiology and Angiology, University Hospital Münster, Münster, Germany

Received 24 June 2013; accepted after revision 27 October 2013; online publish-ahead-of-print 26 January 2014

(ICHOM Standards: J Am Heart Assoc 2015;4:e001767)



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European Journal of Heart Failure (2013) **15**, 1082–1094
doi:10.1093/eurjhf/hft095

REVIEW

Clinical outcome endpoints in heart failure trials: a European Society of Cardiology Heart Failure Association consensus document

Faiez Zannad[1]*, Angeles Alonso Garcia[2], Stefan D. Anker[3], Paul W. Armstrong[4], Gonzalo Calvo[5], John G.F. Cleland[6], Jay N. Cohn[7], Kenneth Dickstein[8], Michael J. Domanski[9], Inger Ekman[10], Gerasimos S. Filippatos[11], Mihai Gheorghiade[12], Adrian F. Hernandez[13], Tiny Jaarsma[14], Joerg Koglin[15], Marvin Konstam[16], Stuart Kupfer[17], Aldo P. Maggioni[18], Alexandre Mebazaa[19], Marco Metra[20], Christina Nowack[21], Burkert Pieske[22], Ileana L. Piña[23], Stuart J. Pocock[24], Piotr Ponikowski[25], Giuseppe Rosano[26], Luis M. Ruilope[27], Frank Ruschitzka[28], Thomas Severin[29], Scott Solomon[30], Kenneth Stein[31], Norman L. Stockbridge[32], Wendy Gattis Stough[33], Karl Swedberg[34], Luigi Tavazzi[35], Adriaan A. Voors[36], Scott M. Wasserman[37], Holger Woehrle[38], Andrew Zalewski[39] and John J.V. McMurray[40]

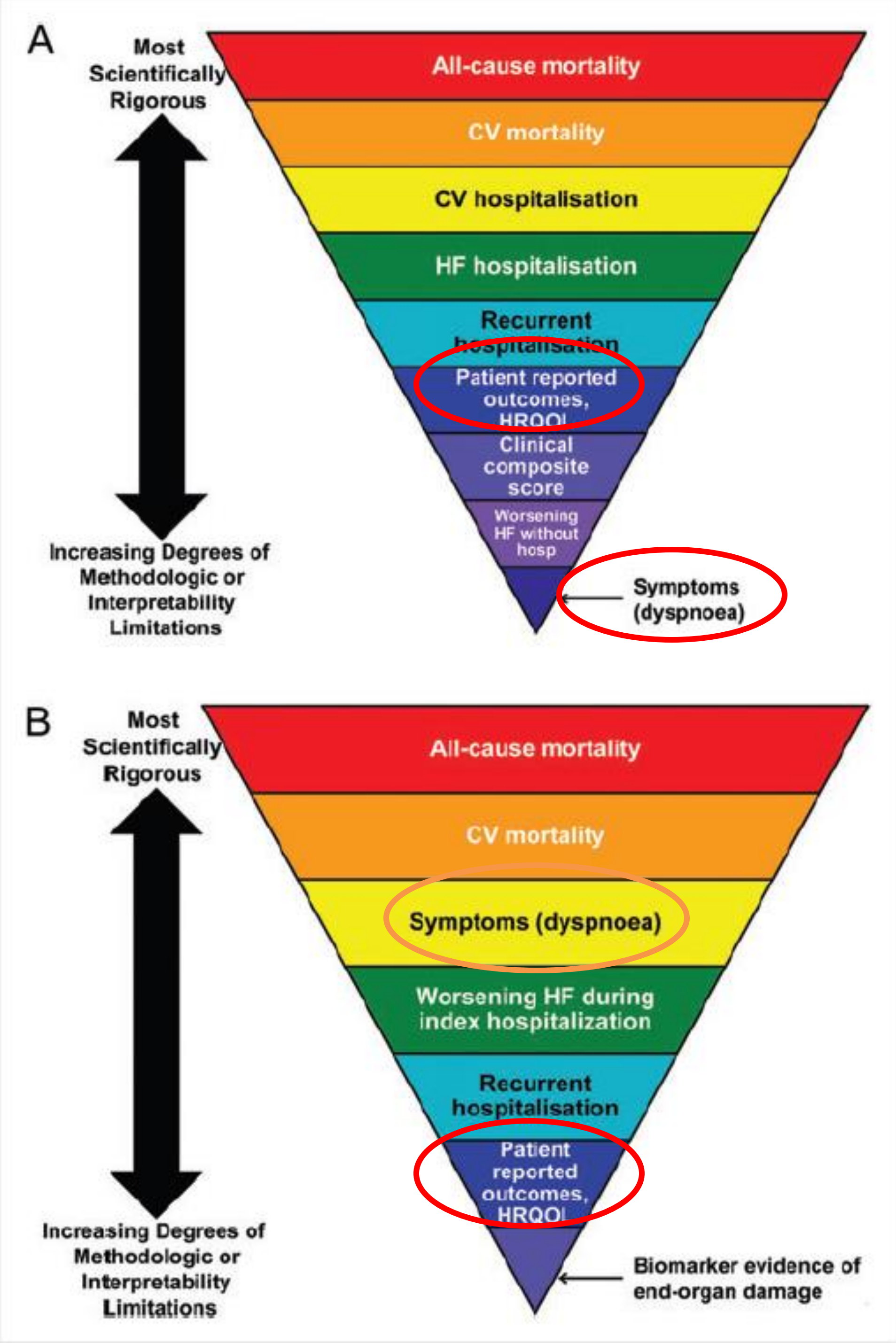
(Zannad F et al, Eur J Heart Failure. 2013 Oct;15(10):1082-94*)*

Chronic heart failure:

"…QoL & PROs… provide insight into treatment effects from the patient's perspective. The therapeutic goal in HF patients is not limited to prolonging survival; improving the QoL is equally important." (p. 1089)

Acute heart failure:

(Zannad F et al, Eur J Heart Failure. 2013 ct;15(10):1082-94*)*



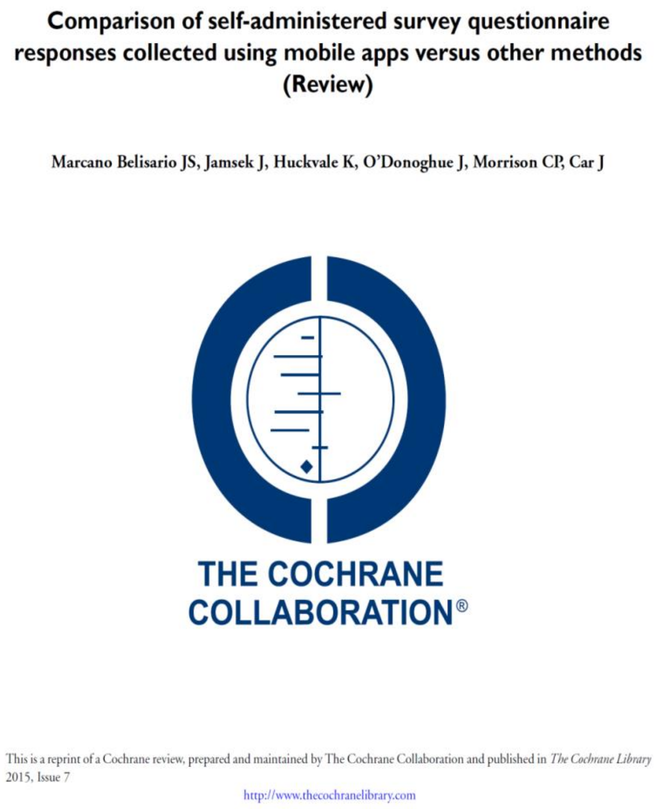
Consensus among HF experts

- *More research is needed to develop robust methods for capturing HF events other than hospitalization or death*
- *Patient-reported outcomes are independent endpoints, not surrogates for mortality*
- *Instruments should be self-adminstered when possible*

(Zannad F et al, Eur J Heart Failure. 2013 Oct;15(10):1082-94*)*

Data collection of PROMS

- Apps may be equivalent to other delivery modes such as paper, laptops and SMS
- May result in more complete datasets compared to paper
- Faster completion times?
- Response rates?
- Data accuracy?
- Not enough evidence to make clear recommendations about the impact of apps



Comparison of self-administered survey questionnaire responses collected using mobile apps versus other methods (Review)

Marcano Belisario JS, Jamsek J, Huckvale K, O'Donoghue J, Morrison CP, Car J

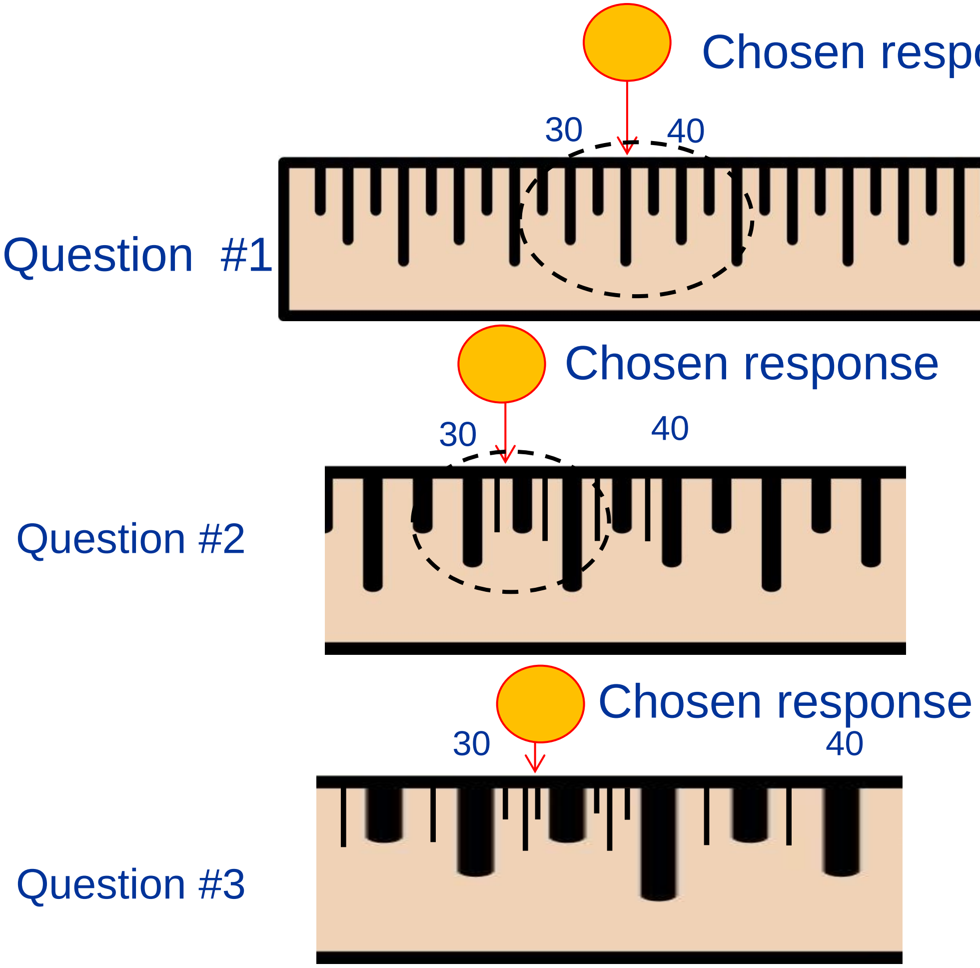
THE COCHRANE COLLABORATION®

This is a reprint of a Cochrane review, prepared and maintained by The Cochrane Collaboration and published in *The Cochrane Library* 2015, Issue 7

http://www.thecochranelibrary.com

(Marcano Belisario et al, Cochrane Library, 2015, Issue 7.)

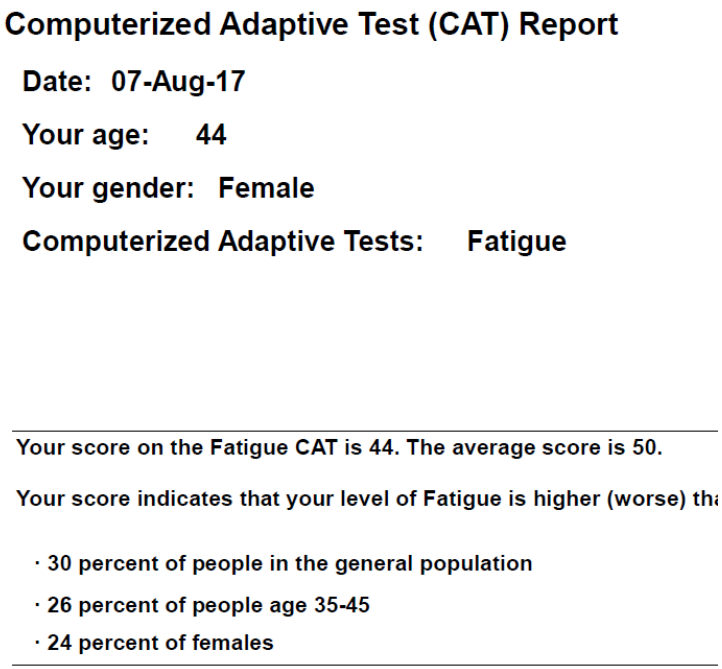
Computer Adaptive Testing



- The next question is generated from the response of the previous question
- A new final score is estimated at every step (new response)

http://www.healthmeasures.net/explore-measurement-systems/promis

Individual CAT report



Computerized Adaptive Test (CAT) Report

Date: 07-Aug-17

Your age: 44

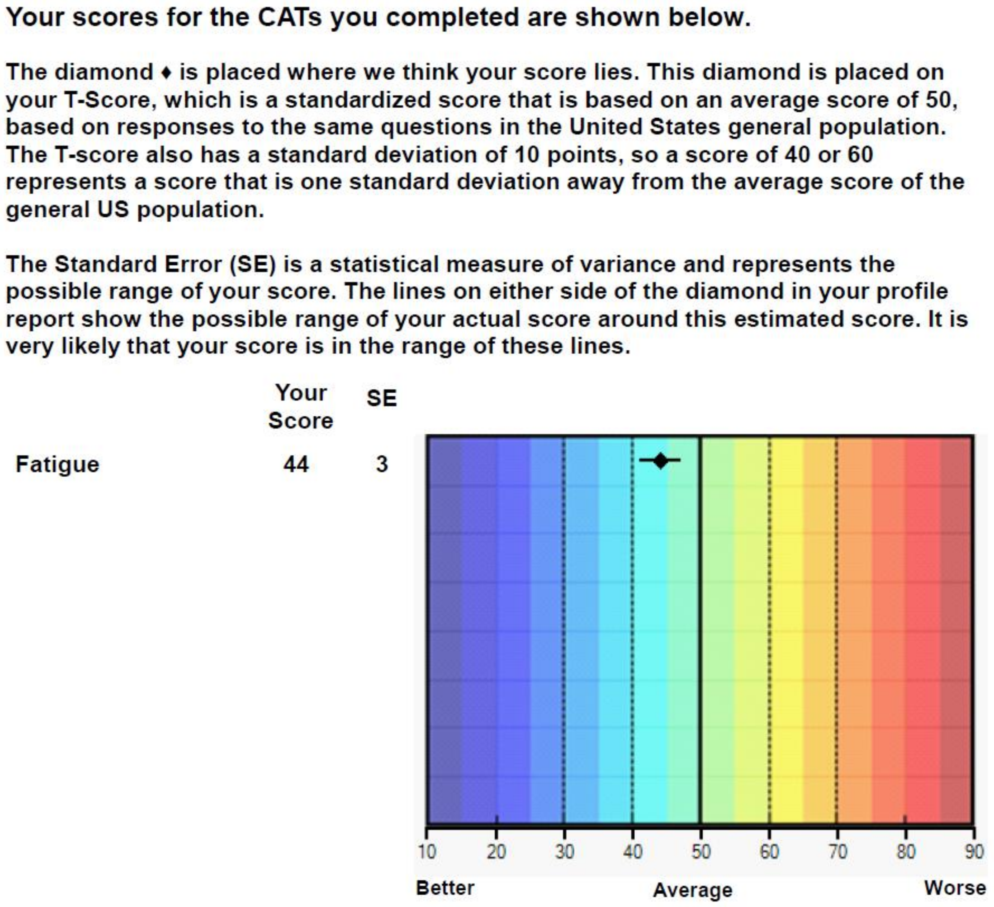
Your gender: Female

Computerized Adaptive Tests: Fatigue

Your score on the Fatigue CAT is 44. The average score is 50.

Your score indicates that your level of Fatigue is higher (worse) tha

- 30 percent of people in the general population
- 26 percent of people age 35-45
- 24 percent of females



Your scores for the CATs you completed are shown below.

The diamond ♦ is placed where we think your score lies. This diamond is placed on your T-Score, which is a standardized score that is based on an average score of 50, based on responses to the same questions in the United States general population. The T-score also has a standard deviation of 10 points, so a score of 40 or 60 represents a score that is one standard deviation away from the average score of the general US population.

The Standard Error (SE) is a statistical measure of variance and represents the possible range of your score. The lines on either side of the diamond in your profile report show the possible range of your actual score around this estimated score. It is very likely that your score is in the range of these lines.

	Your Score	SE
Fatigue	44	3

SRH predict survival in Norwegian HF clinics

- N=3632 (n=1778 for SRH)
- 24 hospital outpatient clinics
- MLHFQ in tertiles was an independent predictor of mortality

MLHFQ

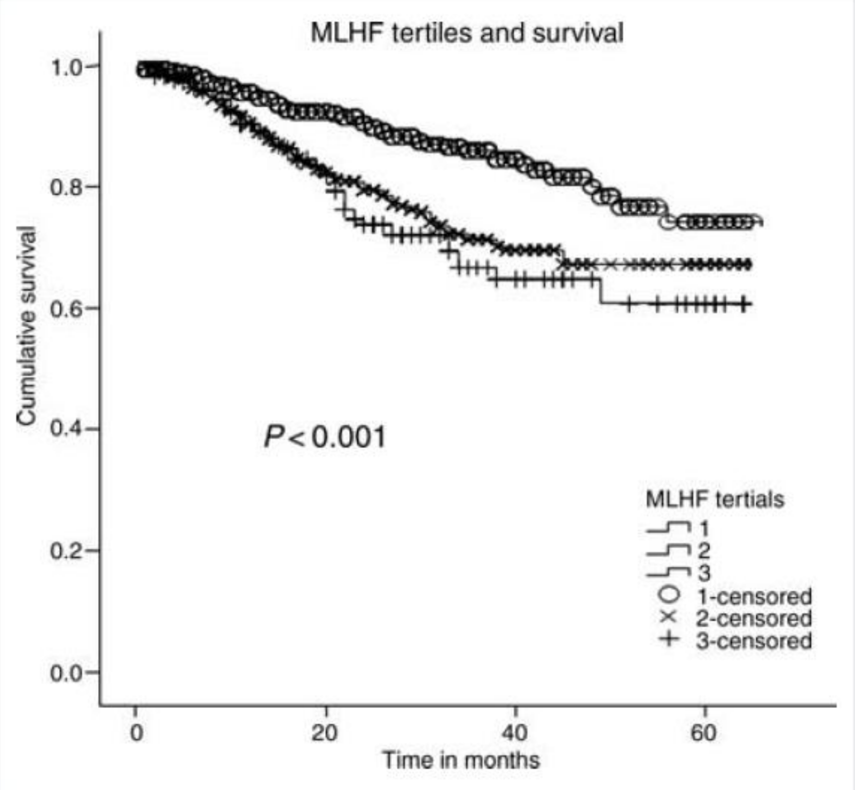


Figure 2 Survival in patients attending specialized outpatient heart failure clinics divided into tertiles of Minnesota Living with Heart Failure (MLHF) score. Tertile 1 = lowest MLHF score.

(Hole T. et al, Eur J Heart Failure. 2010, 12; 1247-1252)

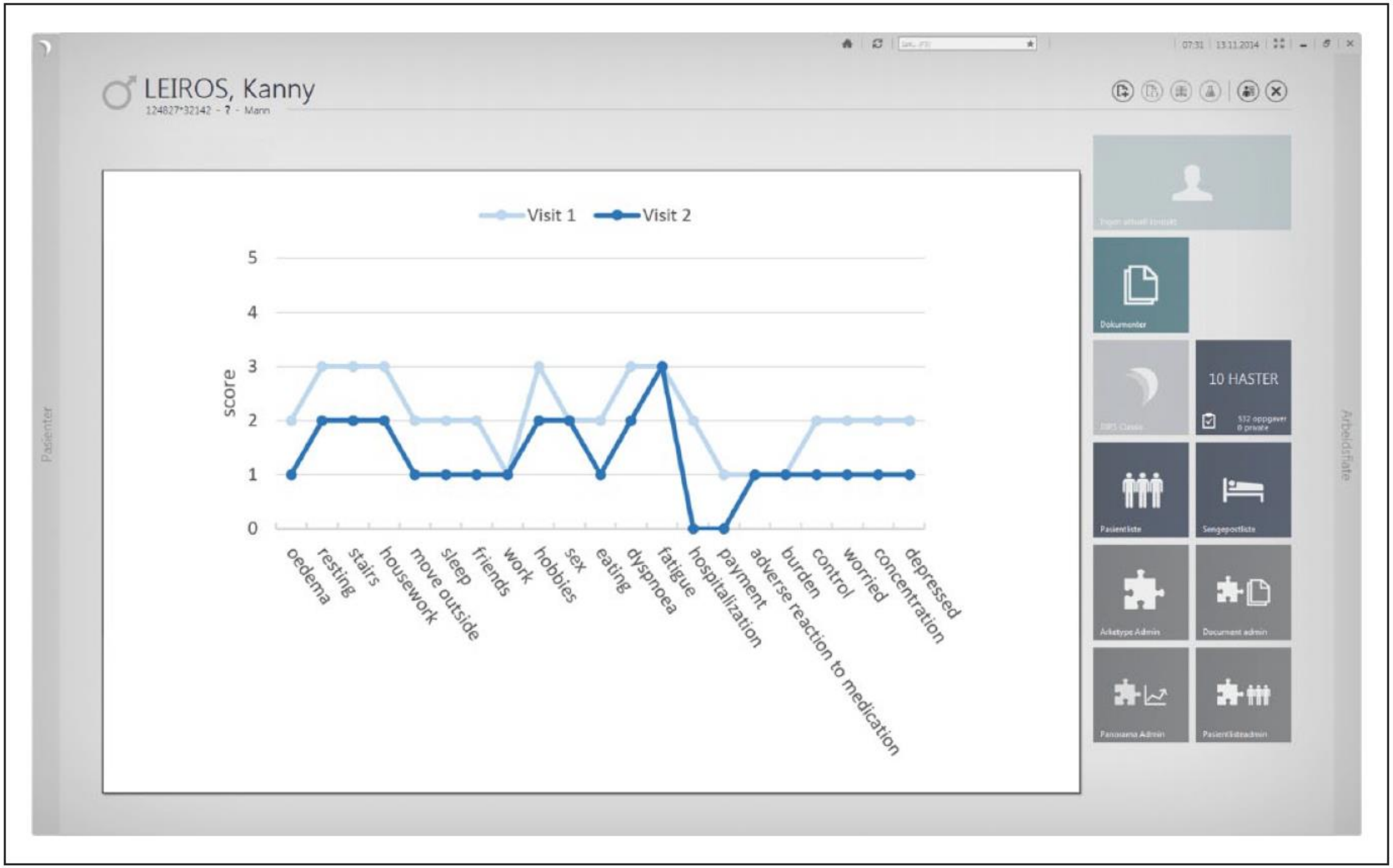
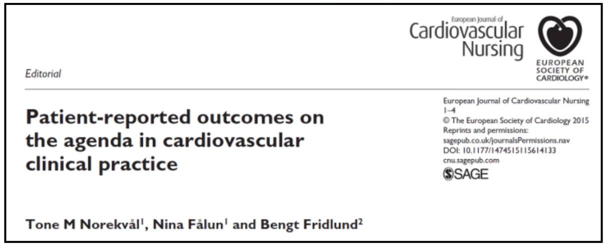


Figure 1. Clinical feedback system. Graphic score presentation of the Minnesota Living with Heart Failure Questionnaire (lower scores indicates better health) integrated into the patient medical records in order to be used by the patient and the cardiac team during the clinical encounter in a heart failure clinic.

(Norekvål TM et al, Eur J Cardiovasc Nurs, 2016, 15;2:108-11.)



European Journal of
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Editorial

Patient-reported outcomes on the agenda in cardiovascular clinical practice

European Journal of Cardiovascular Nursing
1–4
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DOI: 10.1177/1474515115614133
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Tone M Norekvål[1], Nina Fålun[1] and Bengt Fridlund[2]

European Heart Journal
doi:10.1093/eurheartj/ehv316

ESC GUIDELINES

2015 ESC Guidelines for the management of patients with ventricular arrhythmias and the prevention of sudden cardiac death

The Task Force for the Management of Patients with Ventricular Arrhythmias and the Prevention of Sudden Cardiac Death of the European Society of Cardiology (ESC)

Endorsed by: Association for European Paediatric and Congenital Cardiology (AEPC)

Authors/Task Force Members: Silvia G. Priori* (Chairperson) (Italy), Carina Blomström-Lundqvist* (Co-chairperson) (Sweden), Andrea Mazzanti† (Italy), Nico Blom[a] (The Netherlands), Martin Borggrefe (Germany), John Camm (UK), Perry Mark Elliott (UK), Donna Fitzsimons (UK), Robert Hatala (Slovakia), Gerhard Hindricks (Germany), Paulus Kirchhof (UK/Germany), Keld Kjeldsen (Denmark), Karl-Heinz Kuck (Germany), Antonio Hernandez-Madrid (Spain), Nikolaos Nikolaou (Greece), Tone M. Norekvål (Norway), Christian Spaulding (France), and Dirk J. Van Veldhuisen (The Netherlands)

Psychosocial impact of ICD treatment

Psychosocial management after implantable cardioverter defibrillator implantation

Recommendations	Class[a]	Level[b]
Assessment of psychological status and treatment of distress are recommended in patients with recurrent inappropriate shocks.	I	C
Discussion of quality-of-life issues is recommended before ICD implantation and during disease progression in all patients.	I	C

- Controlled defibrillator trials demonstrated preserved or improved QoL in recipients of a defibrillator compared with that in controls.
- Anxiety (8–63%) and depression (5–41%) common in defibrillator patients, most pronounced in patients experiencing inappropriate and/or frequent shocks (e.g. >5 shocks).

Psychosocial impact of ICD treatment

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European Journal of Cardiovascular Nursing
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Original Article

Patient-centred care of patients with ventricular arrhythmias and risk of sudden cardiac death: What do the 2015 European Society of Cardiology guidelines add?

European Journal of Cardiovascular Nursing
1–7

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Tone M Norekvål[1,2], Paulus Kirchhof[3,4] and Donna Fitzsimons[5]

Summary

- PROs are important and should be used in research, guidelines and clinical practice
- Choose valid and reliable measurement tools
- Choose relevant measurement tools; patient involvement
- Combination of generic and disease specific questions
- Take health literacy and ehealth literacy into account