

What Health Outcomes Should Be of Interest to CMS In Studies for Heart Failure Treatment Technologies

John D. Carroll, MD
Professor of Medicine
University of Colorado
School of Medicine

Disclosures

- I, John Carroll, have no financial disclosures relevant to this topic

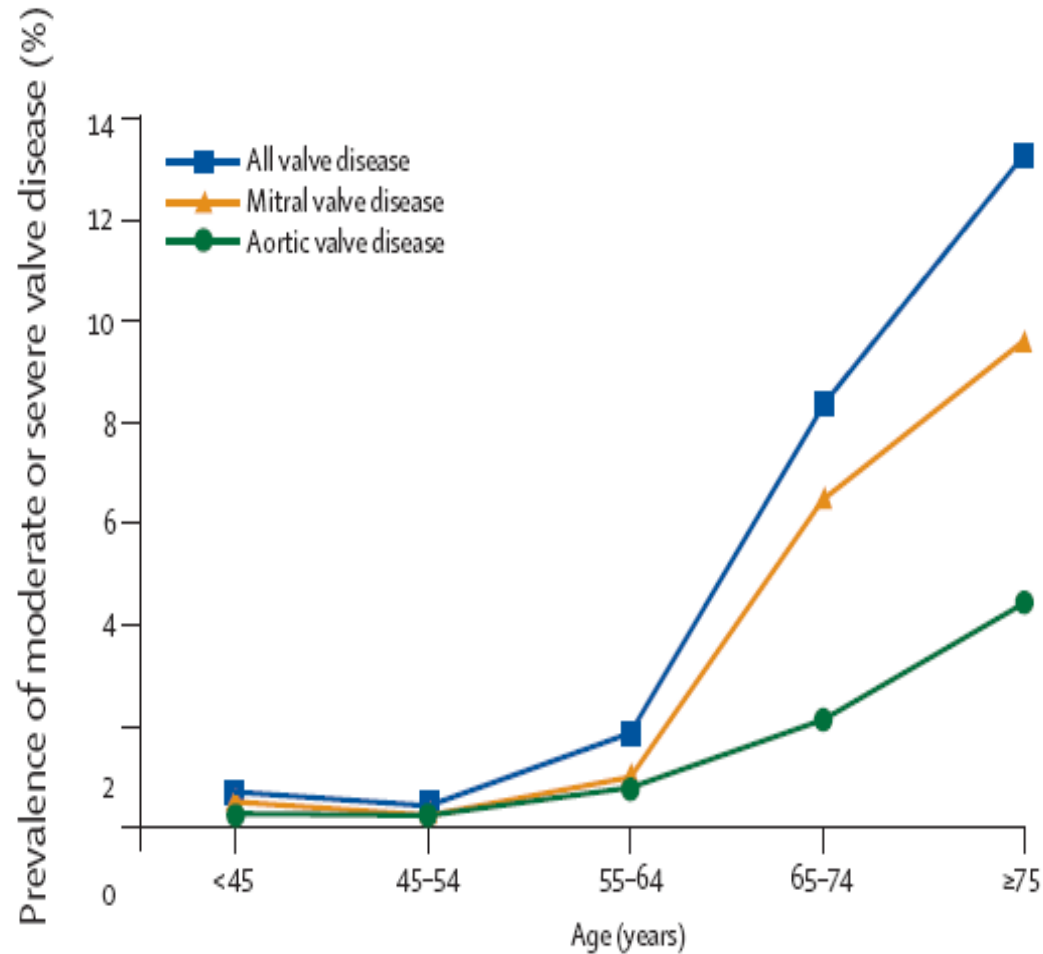
Goal

Advise CMS about the ideal health outcomes in research studies of heart failure treatment technologies and appropriate follow-up duration

Perspective

- Interventional cardiologist
- Areas of experience relevant to this topic
 - CHF related to valvular heart disease and treated with surgical and transcatheter approaches to valve replacement and repair.
 - CHF related to intracardiac shunts and treated with transcatheter closure and plugging technologies.

Clinically Significant Valvular Heart Disease is Becoming More Prevalent in the Aging US Population



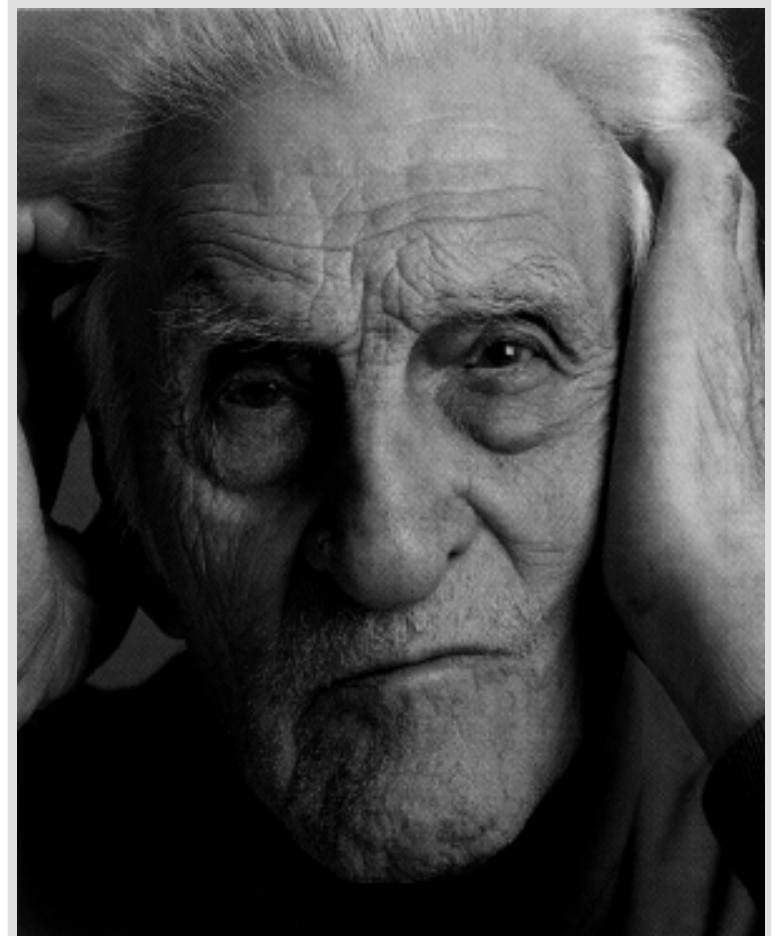
Lancet 2006; 368:1005-11

Major Issues Confounding Outcome Assessment

- Advanced age
- Socio-economic status

A Focus on The Elderly

- “The elderly are the fastest-growing segment of the population, and CVD is a leading cause of morbidity and mortality in older people. The presence of significant comorbidities, cognitive dysfunction, poor social support, and diminished functional status influences both decision making and treatment outcomes..”



Transcatheter Therapies and the Realities of Life Expectancy

- Recent U.S. census and Medicare data on average life expectancy
 - 50-year-old: 31.5 years
 - 70-year-old: 14.9 years
 - 80-year-old: 8.7 years
 - 90-year-old: 4.6 years
- Survival benefits are important; however, other benefits are extremely important for judging the value of transcatheter therapies
 - improved or maintained functional status
 - quality of life
 - freedom from hospitalization

Outcomes Assessment Must be Put in a Broader Context of The Patients Daily Existence



Social Determinants of Risk and Outcomes for Cardiovascular Disease

- Socioeconomic position
- Race, ethnicity
- Social support
- Culture and language
- Access to care
- Residential environment

Markers of Socioeconomic Position

- Material conditions (based on income and wealth)
- Health
- Education
- Access to valued personal activities (eg, work)
- Political voice
- Social connections
- Environment
- Physical insecurity (crime, violence)

Havraek EP et al. A Scientific Statement
From the American Heart Association.
Circulation. 2015;132:873-898.

Outcomes to be Considered

1. Survival
2. Objective assessment of the disease-specific anatomical-physiologic variable that the treatment addresses
3. Presence/absence of treatment complications
4. Improved patient-reported health status
5. Objective functional assessment
6. Freedom from hospitalization and loss of independent living

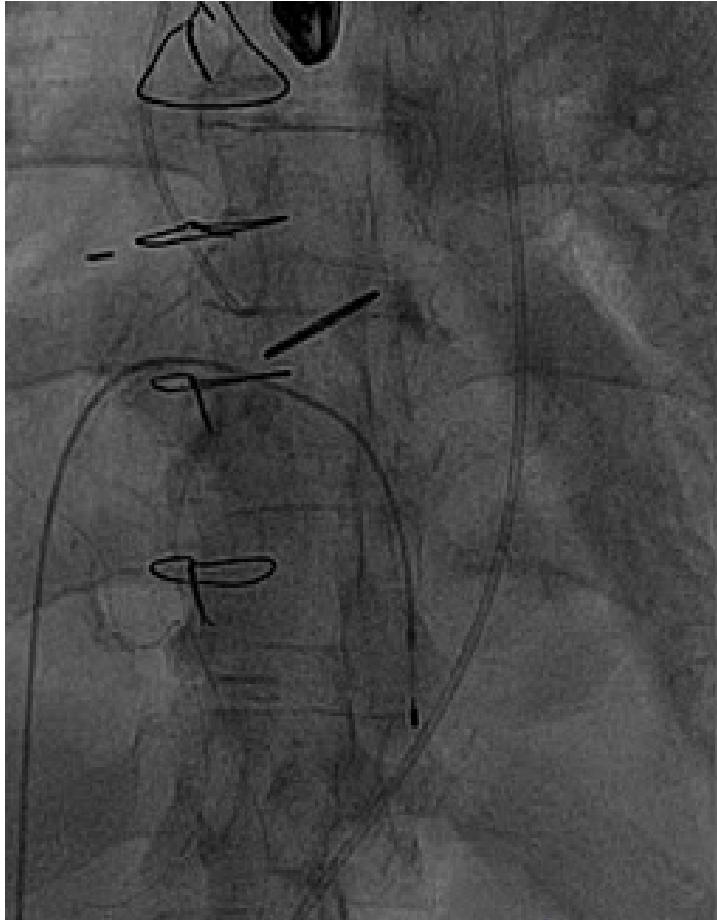
Objective assessment of the
disease-specific anatomical-
physiologic variable that the
treatment addresses

Time Frame of Assessment

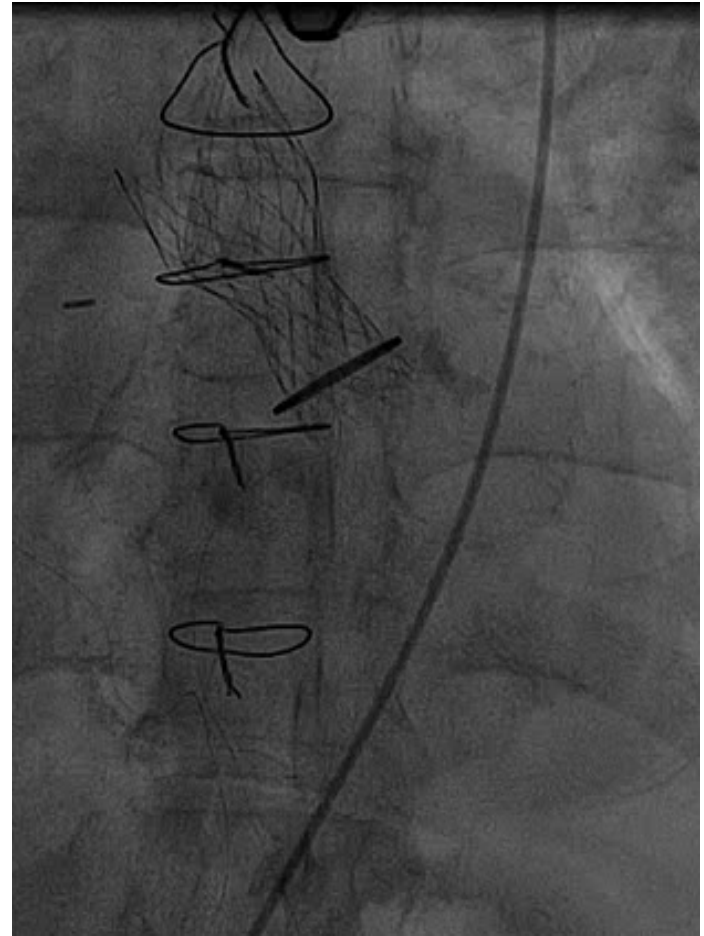
Immediate to 30 Days

One-Year: If durability is potential issue

Outcome Assessment by Angiography



**4+ Aortic Regurgitation Thru Aortic
Bioprosthesis: 23 mm Hancock MO valve**

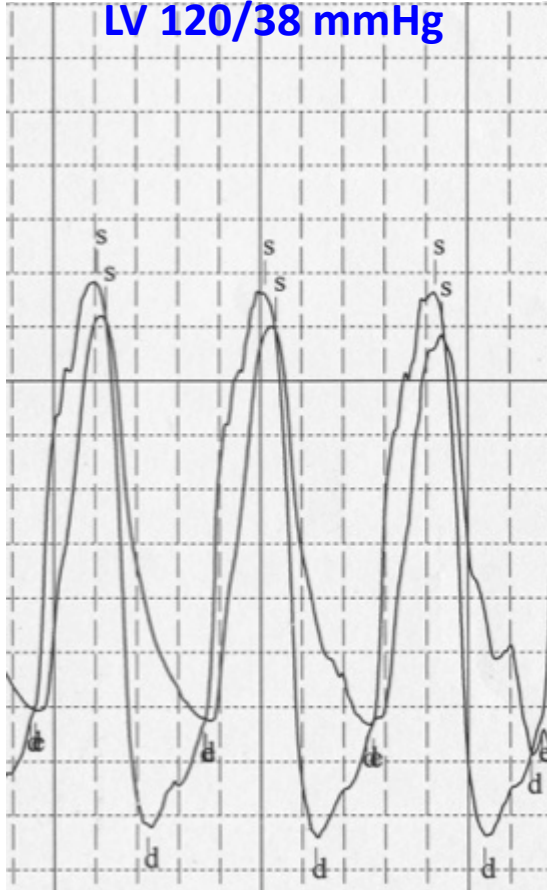


**No AR Post Valve-in-Valve and
Enhanced Contrast Flow in
Descending Thoracic Aorta**

Outcome Assessment by Hemodynamics

**Aorta 114/33 (67)
mmHg**

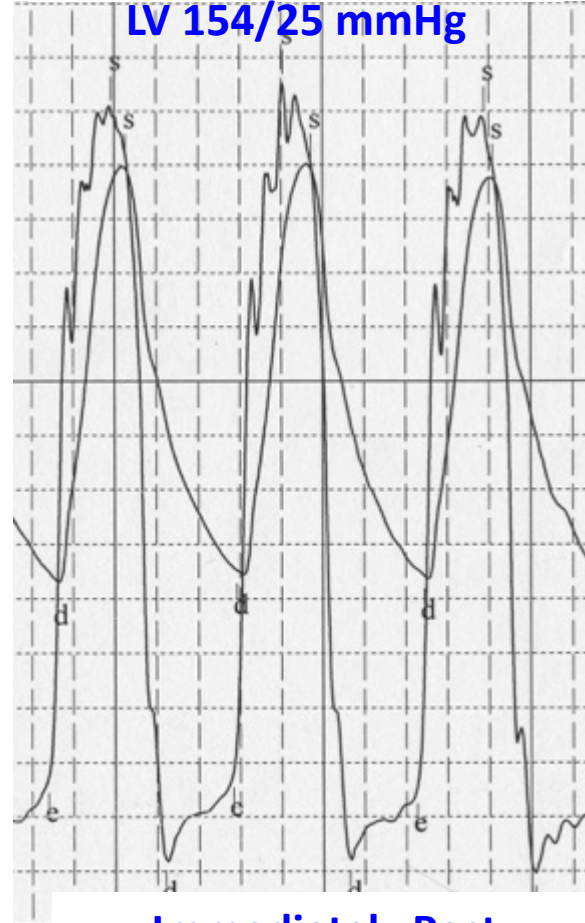
LV 120/38 mmHg



**Baseline:
Severe Aortic Regurgitation**

**Aorta 142/63 (94)
mmHg**

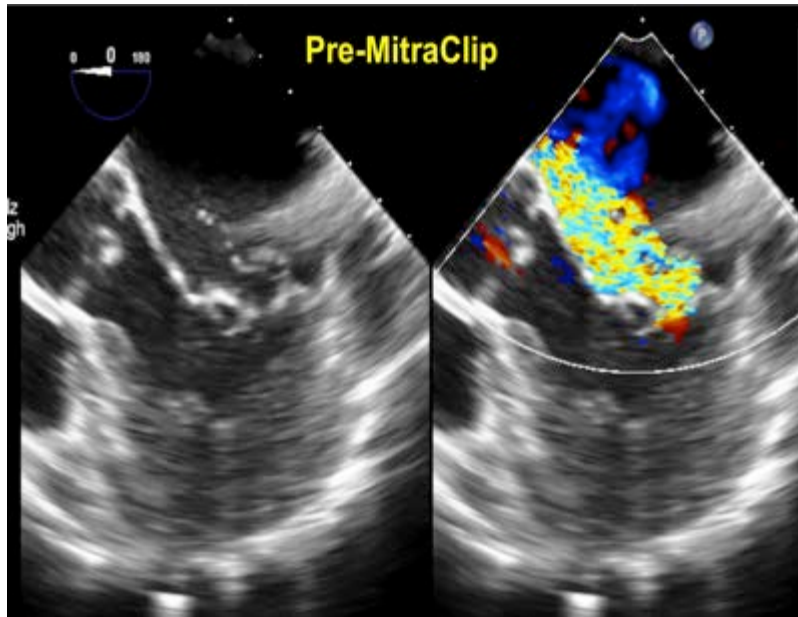
LV 154/25 mmHg



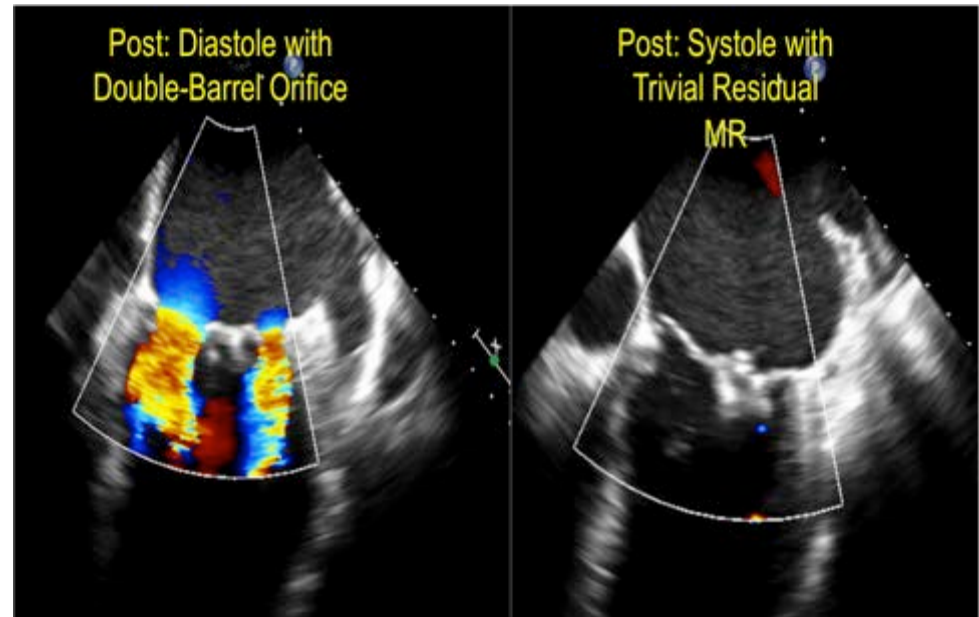
**Immediately Post
Valve-in-Valve**

Outcome Assessment by Cardiac Ultrasound

Pre-Procedure Documentation
of Severe Mitral Regurgitation



Post-Procedure Documentation of
Mitral Regurgitation Reduction



Presence/absence of treatment complications

Time Frame of Assessment

Immediate to 30 Days

One-Year: If a late complication is potential issue

Lessons from Assessment of Physician-Hospital Performance

Isolated surgical aortic valve replacement with a composite score based solely on outcomes

1. Risk-standardized mortality
2. Any-or-none risk-standardized morbidity occurrence
 1. Sternal infection
 2. Reoperation
 3. Stroke
 4. Renal failure
 5. Prolonged ventilation

Shahian DM et al. The Society of Thoracic Surgeons Isolated Aortic Valve Replacement (AVR) Composite Score: A Report of the STS Quality Measurement Task Force. Ann Thorac Surg 2012;94:2166 –71)

Improved patient-reported health status

Time Frame of Assessment

Baseline

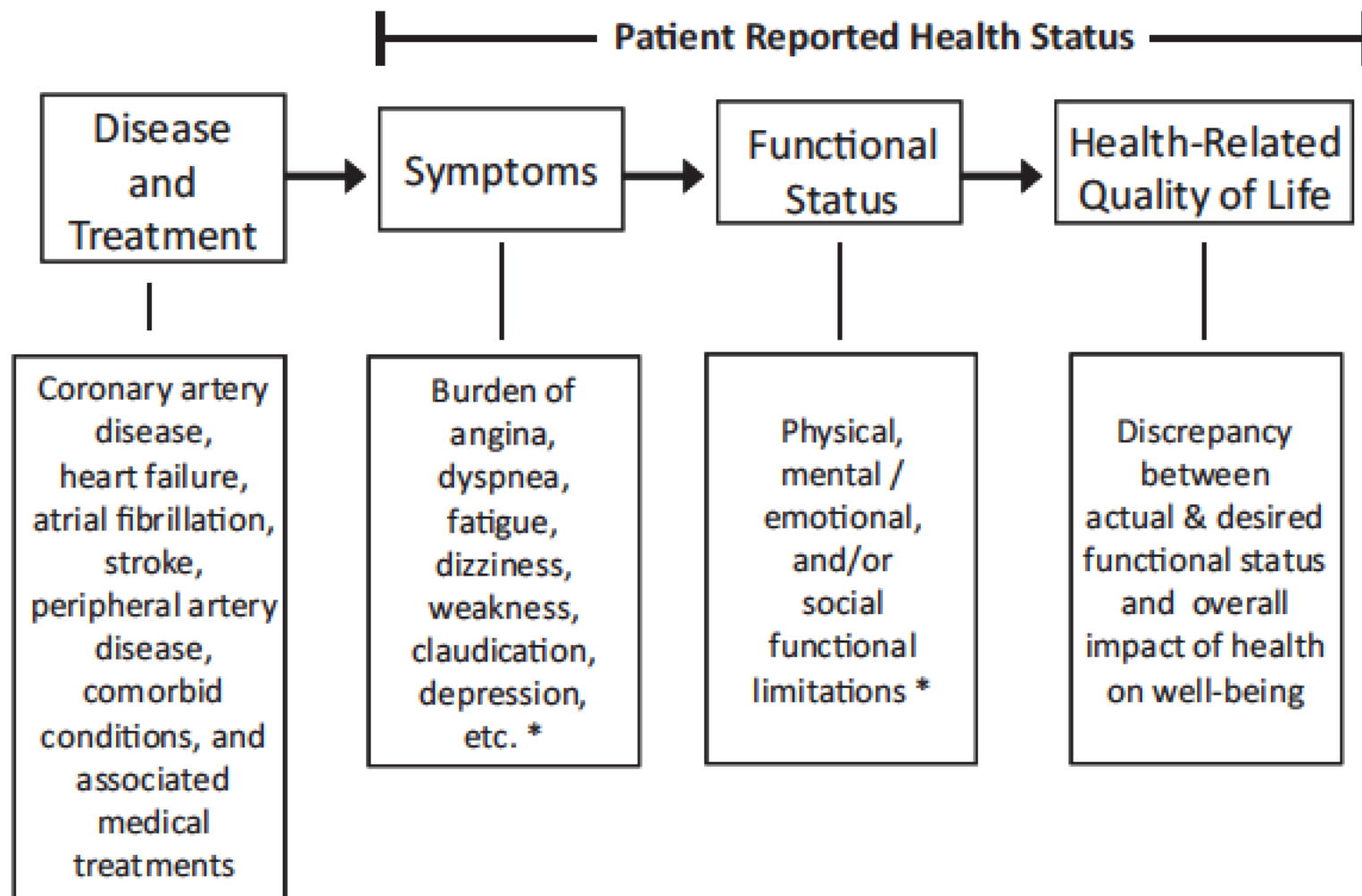
One Year

If patient population is elderly or has other comorbid condition reducing one-year survival then add 30 Days

The Importance of Measuring Patient-Reported Health Status

1. The principal goals of health care are to help people “live longer and live better,” that is, to optimize both survival and health.
2. Patient-reported health status, which includes symptom burden, functional status, and HRQL (health-related quality of life), is an important measure of health.
3. Validated patient health status surveys, including disease-specific instruments for patients with cardiovascular disease, allow for the quantification of this critical, patient-centered outcome.
4. Cardiovascular patient health status surveys have been used successfully in clinical trials and other research studies to quantify treatment benefits with regard to symptoms, functional status, and HRQL; however, they remain underutilized.
5. Patient health status is a risk marker for adverse outcome (mortality and morbidity) and healthcare costs

John S. Rumsfeld, Karen P. Alexander, David C. Goff, Jr, Michelle M. Graham, P. Michael Ho, Frederick A. Masoudi, Debra K. Moser, Véronique L. Roger, Mark S. Slaughter, Kim G. John S. Rumsfeld, Karen P. Alexander, David C. Goff, Jr, Michelle M. Graham, P. Michael Ho, Scientific Statement From the American Heart Association Cardiovascular Health. Circulation. 2013;127:2233–2249

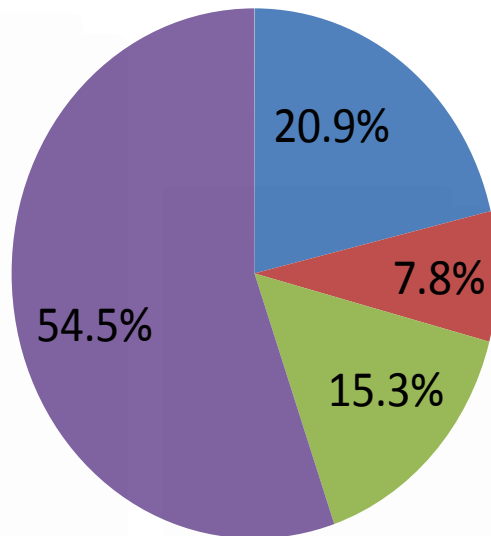


Patient-Reported Health Status

- The Kansas City Cardiomyopathy Questionnaire (KCCQ) is a health status measure that integrates patients' symptoms, functional status, and quality of life into a single measure.
- The KCCQ is reliable, patient-centered, and easily collected in routine clinical practice.

Improved Patient-Reported Outcomes

Change in KCCQ score from baseline to 30 days



- No change or decreased
- Minimum improvement (>=5-9 points)
- Moderate improvement (>=10-19 points)
- Large improvement (>=20 points)

Source: STS/ACC TVT Registry Data Mart 3,362 pt records from 2014-15, as of 4-24-16

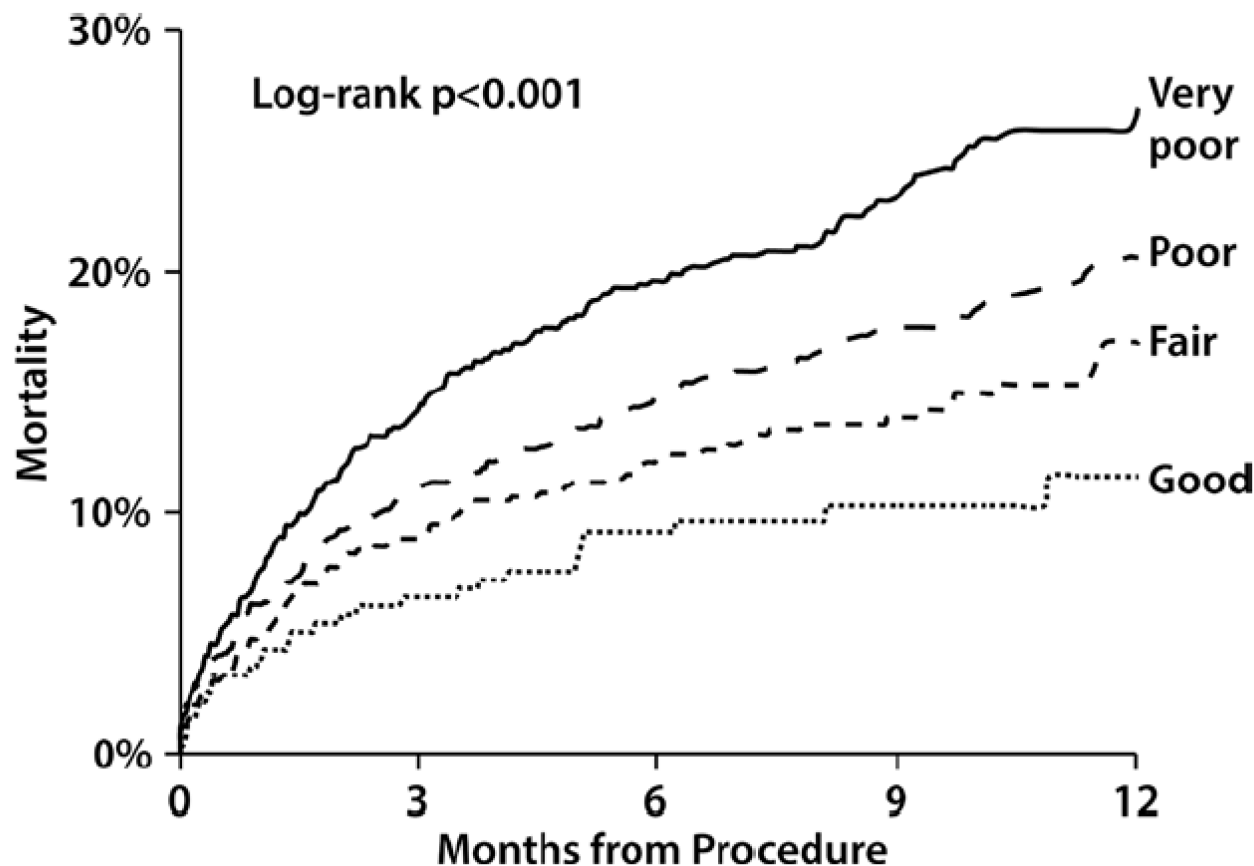
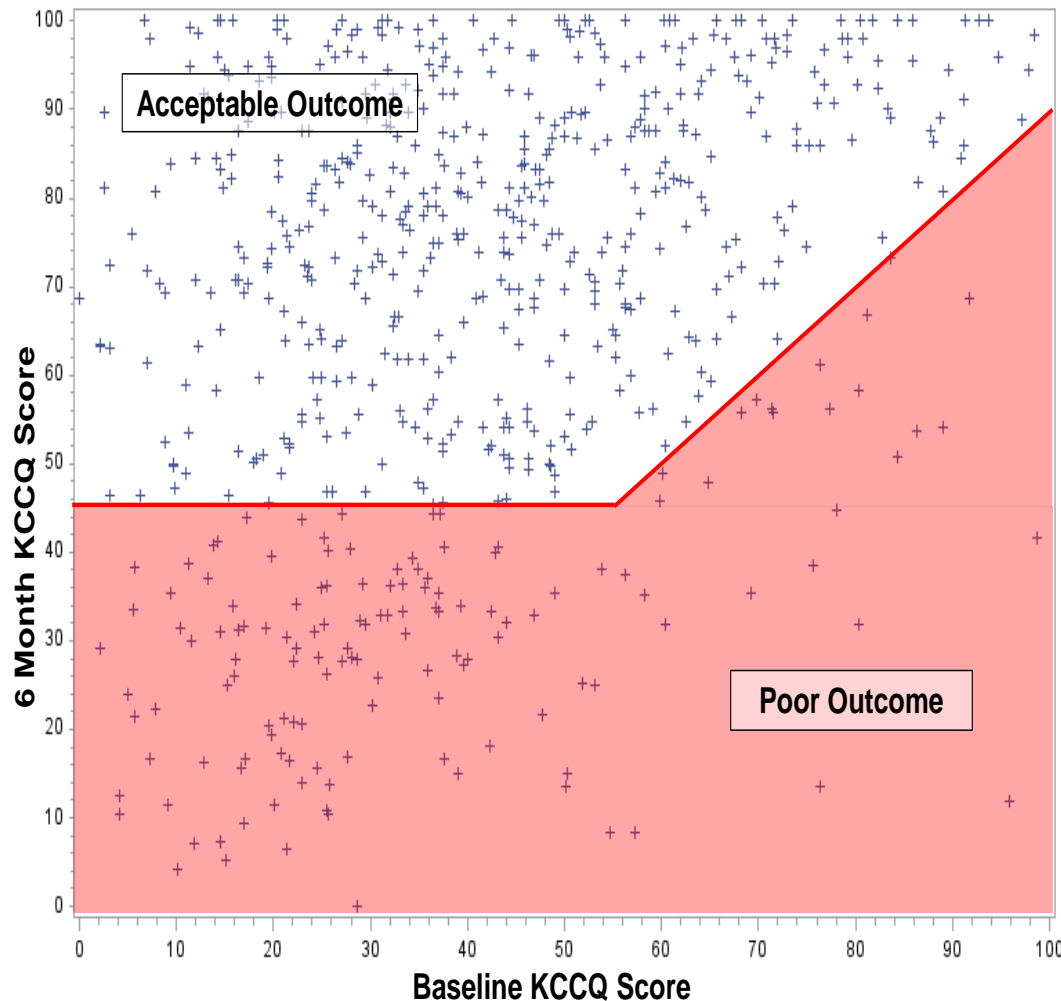


Figure 2. Kaplan–Meier mortality curves for patient after transcatheter aortic valve replacement (TAVR) according to baseline health status. Blue line indicates those patients with very poor health status before TAVR (Kansas City Cardiomyopathy Questionnaire [KCCQ] <25); red line: poor health status (KCCQ 25–49); green line: fair health status (KCCQ 50–74); and brown line: good health status (KCCQ ≥75).

Arnold et al. Circ Cardiovasc Interv. 2015;8:e002875.

Good versus Poor Outcome: Conceptual Framework

Arnold SV, et al. Circ CV Qual Outcome 2013



- For patients at high risk of surgical AVR, a poor outcome should include both a mortality and a QOL component
- Reasonable definition of a poor outcome might be:
 - *Death within 6 months*
 - *Persistent KCCQ <45*
 - *KCCQ decrease of > 10 points vs. baseline*

Predicting Outcomes and Then Assessing What Happens



- Risk model algorithms to predict mortality – immediate treatment related.
- Predicting who will respond to treatment and who will not.

Objective functional assessment

Time Frame of Assessment

Baseline

30 Days: Assessment of recovery

One-Year: Assessment of durability

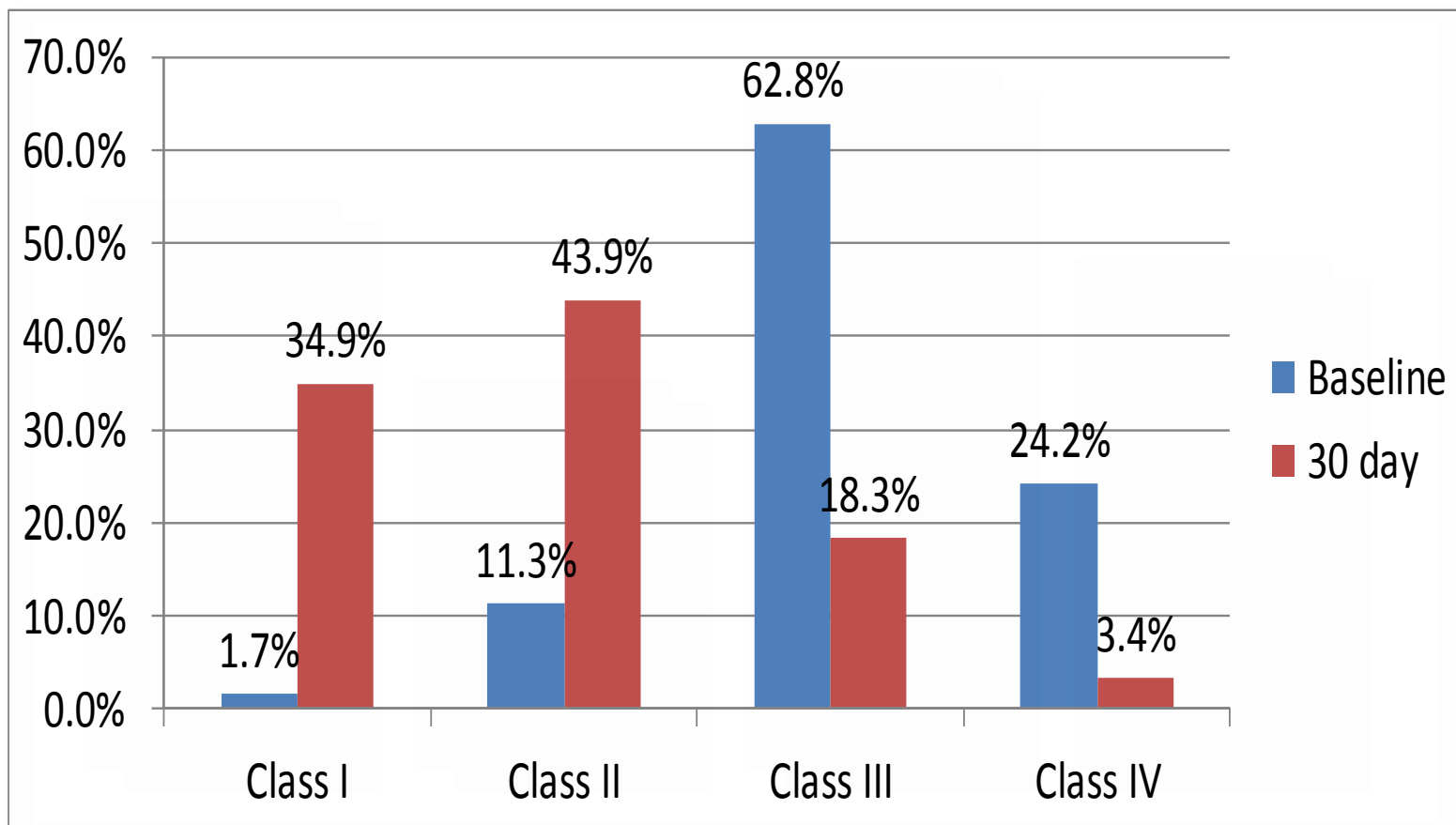
The New Metrics of Success



- Functional Assessment
 - Patient reported
 - NYHA class
 - 6 minutes walk test

ATS Statement: Guidelines for the Six-Minute Walk Test
Am J Respir Crit Care Med Vol 166. pp 111–117, 2002

Leaflet Clip Procedures - NYHA



Source: STS/ACC TVT Registry Data Mart 2,339 pt records from 2015, as of 4-24-16

Freedom from hospitalization and loss of independent living

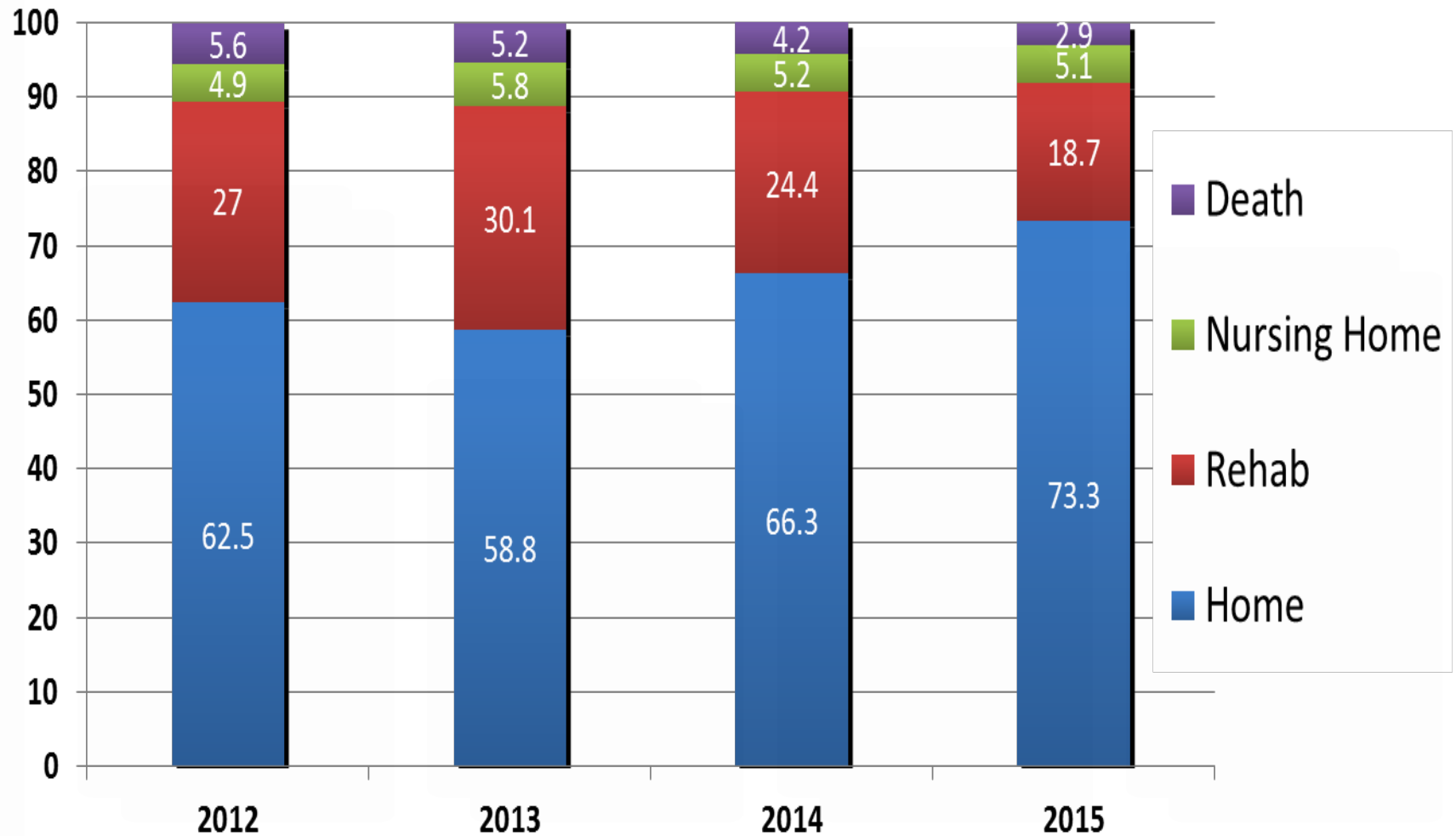
Time Frame of Assessment

Baseline

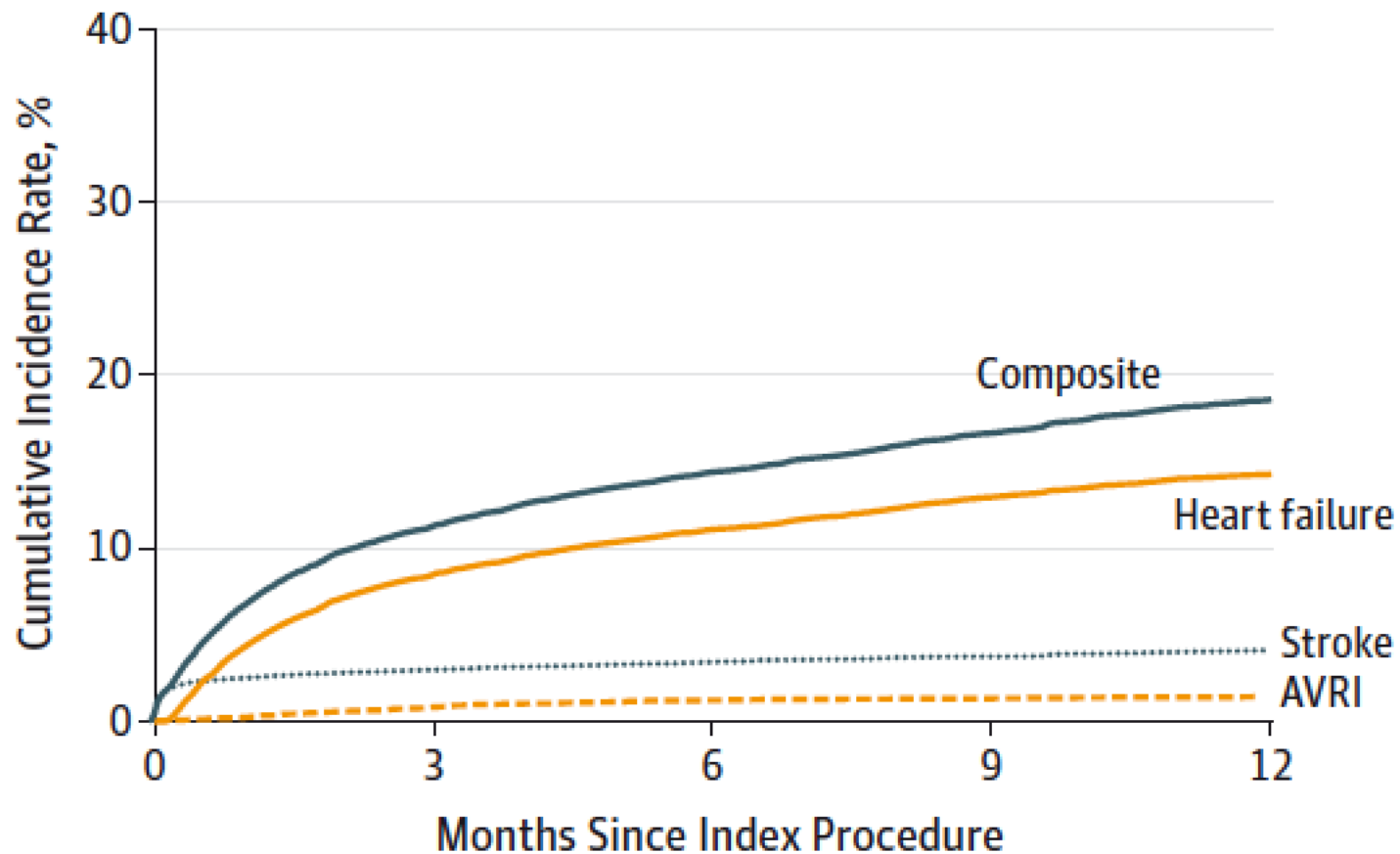
Discharge or 30 Day

One Year

After TAVR – The “Disposition”



Hospitalization Incidence After Transcatheter Aortic Valve replacement



Holmes D et al. JAMA. 2015;313(10):1019-1028.

Conclusions

- The assessment of outcomes must address the six major domains:
 1. Survival
 2. Objective assessment of the disease-specific anatomical-physiologic variable that the treatment addresses
 3. Presence/absence of treatment complications
 4. Improved patient-reported health status
 5. Objective functional assessment
 6. Freedom from hospitalization and loss of independent living

Conclusions

- The timing of the assessment of the different domains of outcomes should include:
 - Baseline assessment for comparison to post-treatment
 - Immediate to 30 day
 - Survival
 - Objective assessment of the disease-specific anatomical-physiologic variable that the treatment addresses
 - Presence/absence of treatment complications
 - One Year
 - Survival
 - Improved patient-reported health status
 - Objective functional assessment
 - Freedom from hospitalization and loss of independent living