

# **MEDCAC Meeting**

## Health Outcomes in Heart Failure Treatment Technology Studies

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# Disclosures

Dan Schaber, PharmD

- Medtronic employee (major)

# Heart Failure Care Has Improved Survival; Morbidity and Mortality Remain Significant

- Aims of innovation include:
  - Improve outcomes, reduce healthcare expenditures
  - Measure safety and effectiveness accurately and in an optimal timeframe
  - Efficiently detect treatment success or failure
  - Enable timely access to treatment

# The Use of Surrogate/Intermediate Endpoints Should Be...

- Meaningful to patients, clinicians, providers, and payers
- Biologically plausible
- Widely available
- Least burdensome
- Minimally influenced by bias
- Cost efficient for trial design and execution

Most importantly

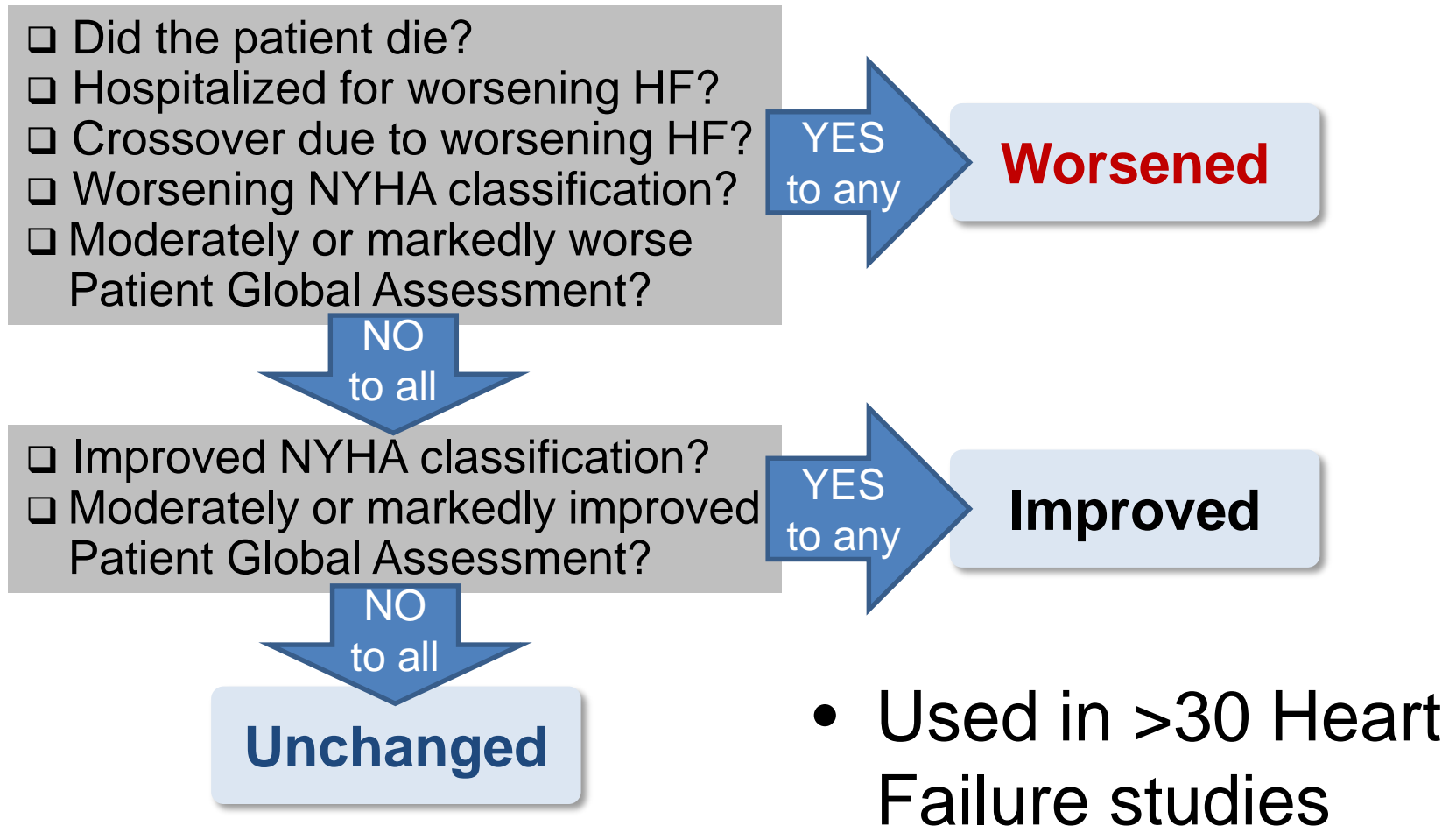
- Predictive of longer-term outcomes
- Corroborated by real-world findings

Two such notable endpoints:

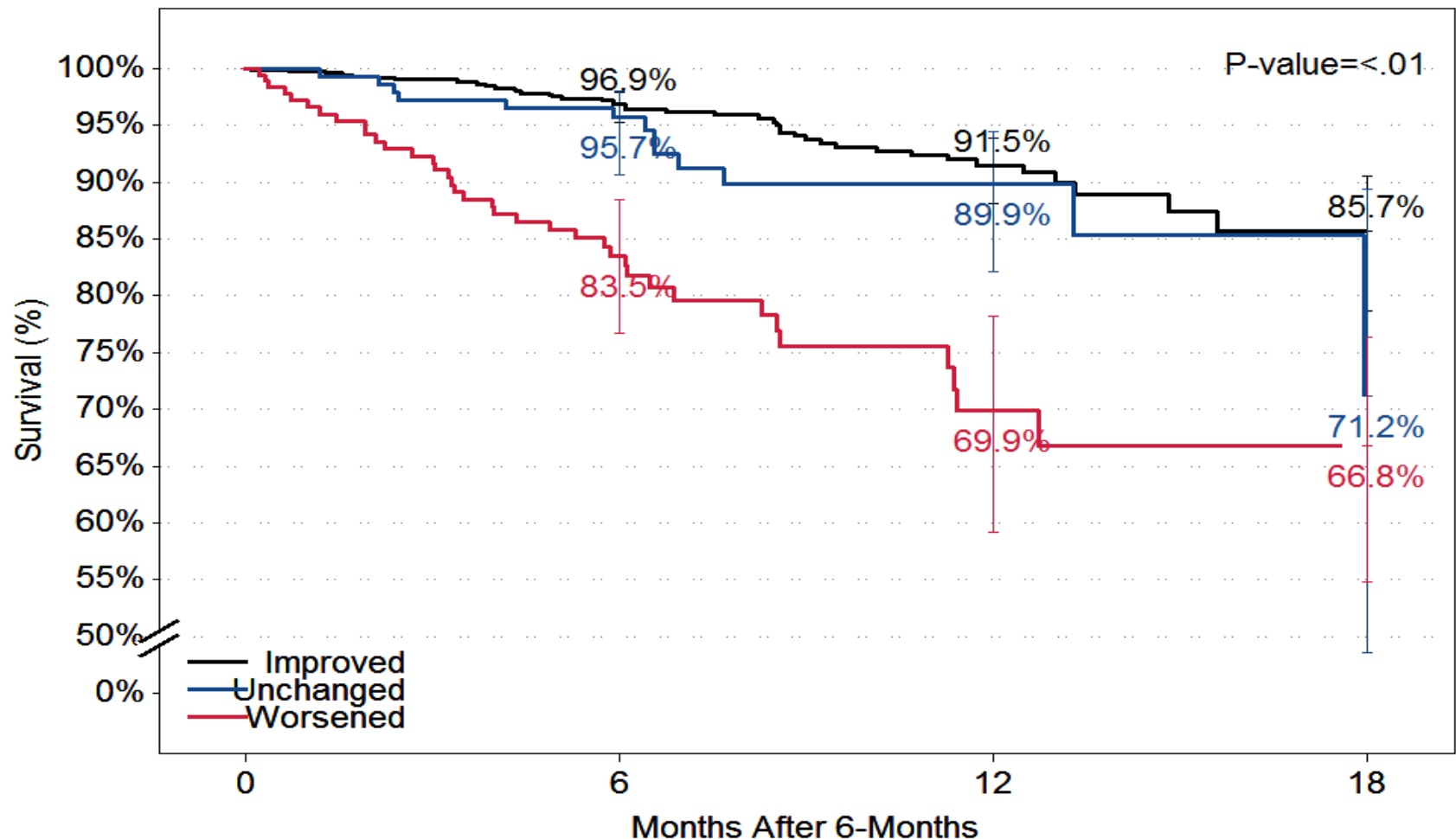
1. Clinical: Clinical Composite Score\*
2. Structural: Left Ventricular End Systolic Volume (LVESV)

\*Packer M. J Card Fail. 2001.

# 1. Clinical Composite Score



# Clinical Composite Score - Predictive of Survival



Subjects at Risk

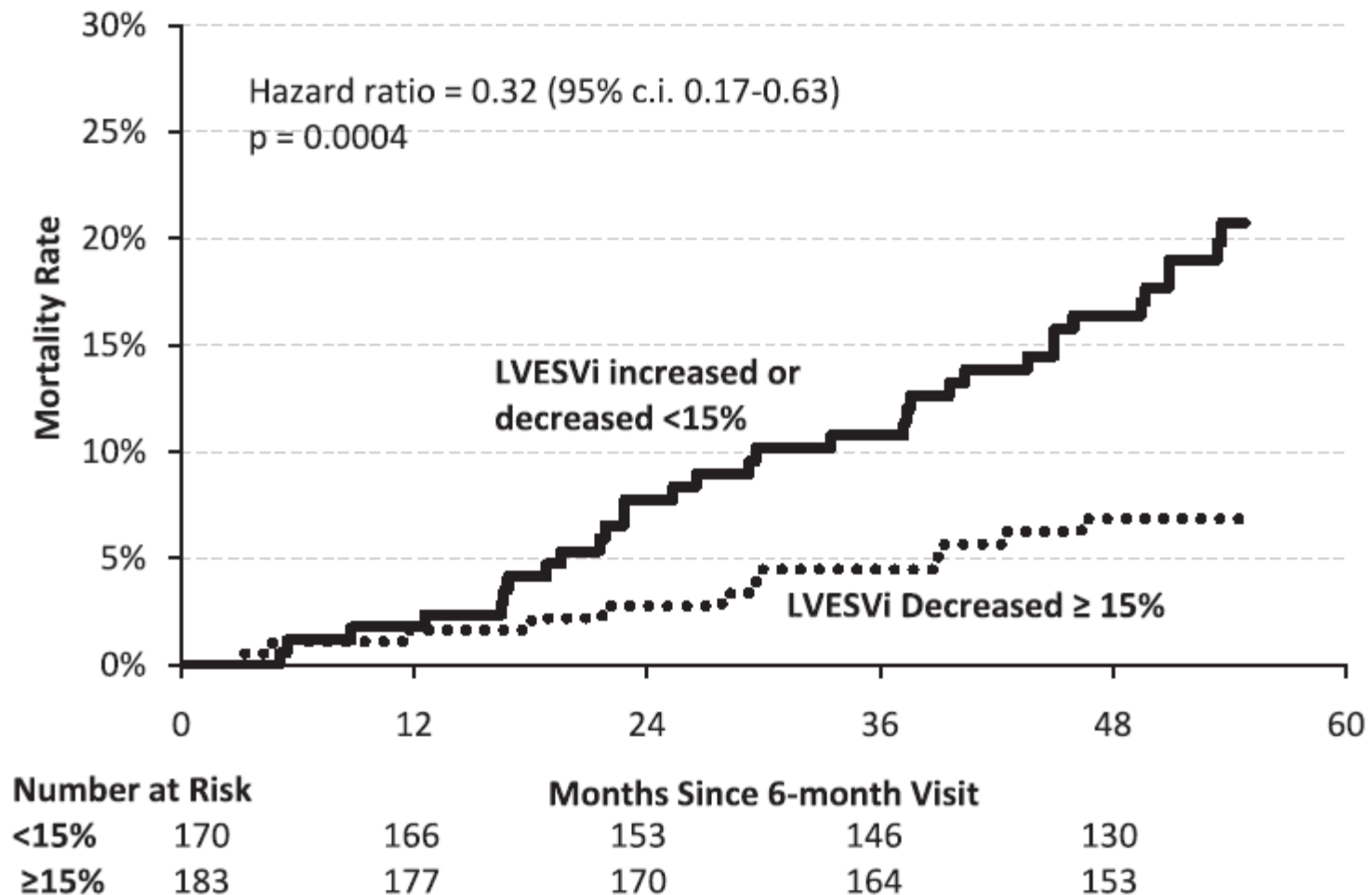
Chung ES, et al. Circulation.

2014;130(S2).

## 2. Left Ventricular End Systolic Volume (LVESV)

- Standard, routine echocardiography measure of cardiac function
- Dilation (remodeling) is associated with poor prognosis
- Decreased size (reverse remodeling) is associated with improved prognosis
- Powered objective used in pivotal HF therapy trials

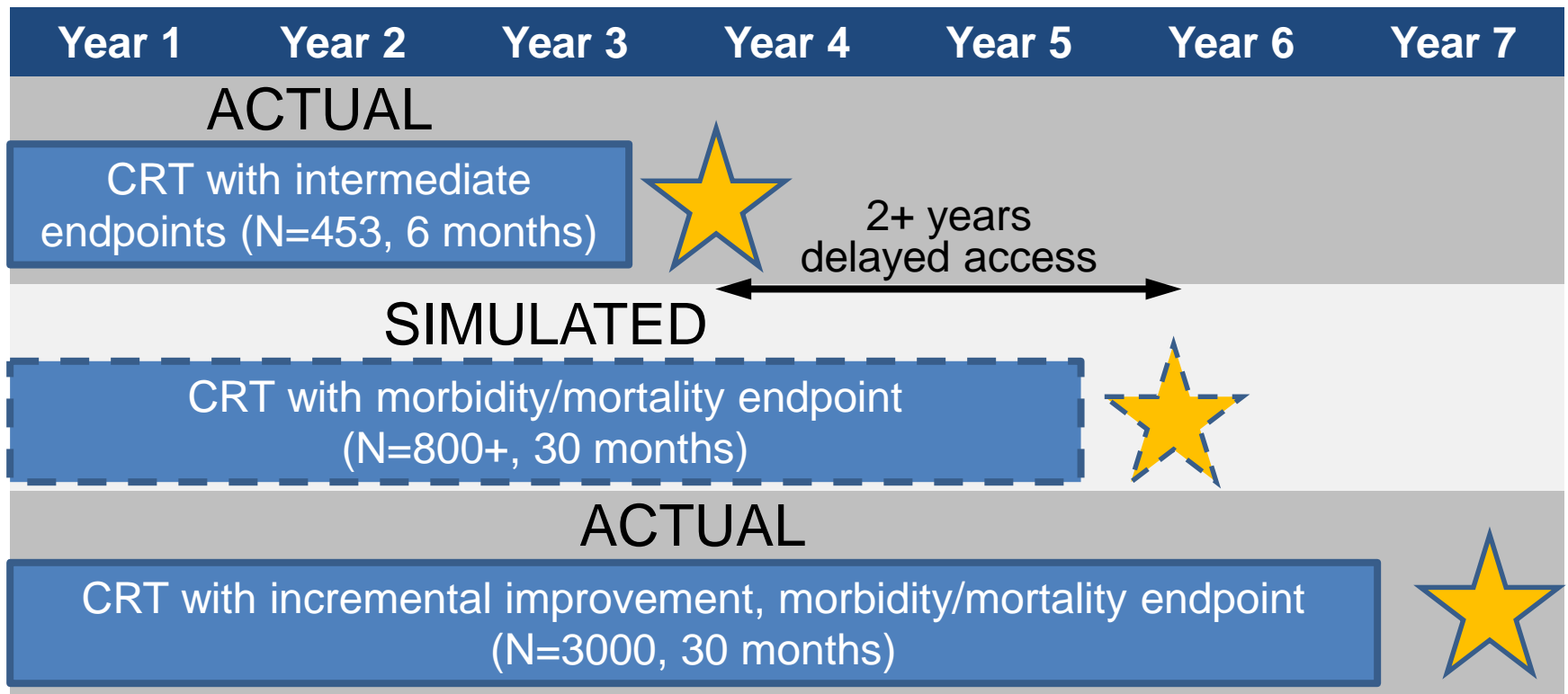
# LVESV Predictive of Mortality



Gold MR, et al. Heart Rhythm. 2015.



# Intermediate Endpoints Provide Earlier Access; Corroborated by Long Term Results



- Morbidity/mortality endpoints increase costs significantly both directly and in lives lost or worsened while therapies are under evaluation

# Conclusions

- Use of Surrogate/Intermediate endpoints such as CCS, LVESV for Heart Failure interventions:
  - Are meaningful
  - Allow for accurate measure of treatment effect in an optimal timeframe
  - Can be corroborated by post-market measures of long-term outcomes
  - Can be easily implemented and tracked
  - Strategic implementation may expedite access to life-extending innovations improving lives while saving healthcare expenditures