

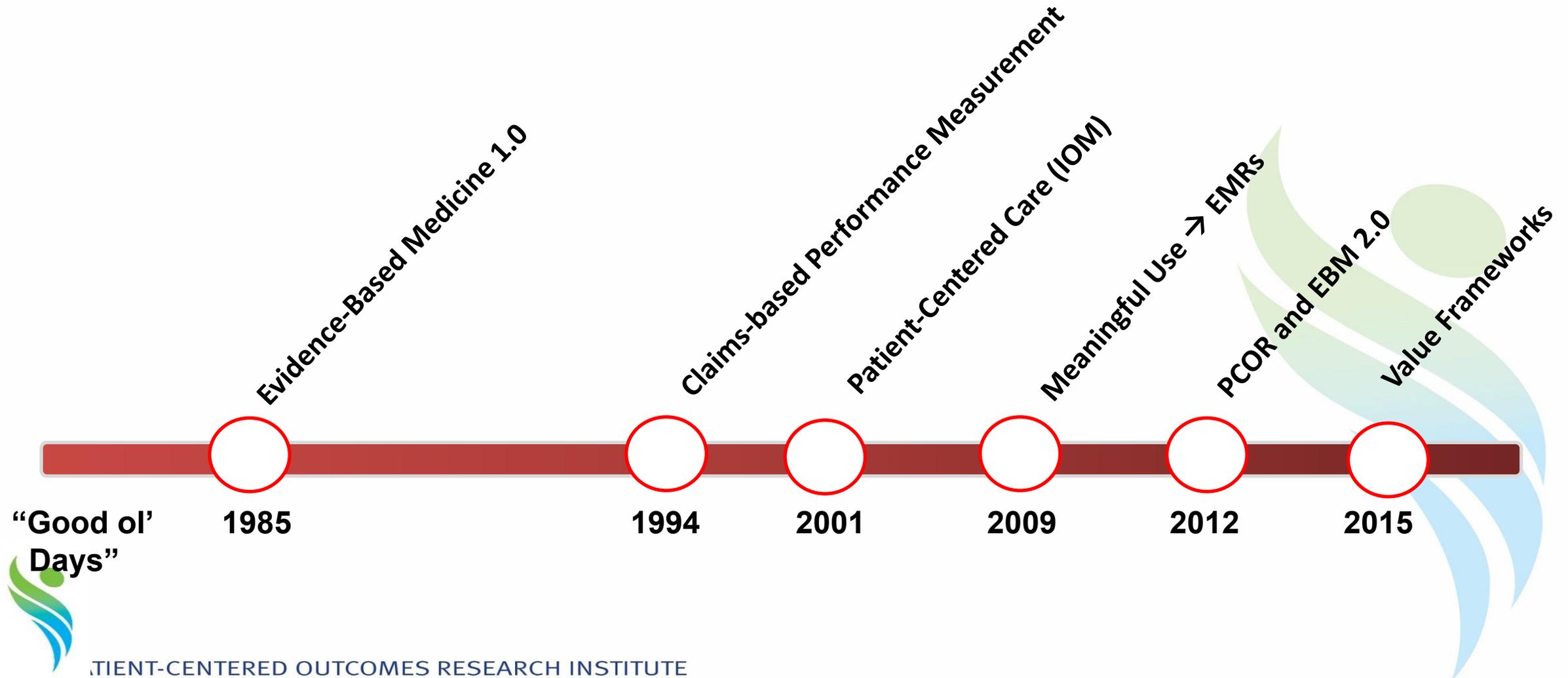
# After 20 Years: Population Management meets Patient-Centered Care

**ECRI Conference**

**Joe Selby, Executive Director,  
Patient-Centered Outcomes Research Institute, PCORI  
November 29, 2017**



# Progress: How We Got Here



“Good ol’ Days”

1985

1994

2001

2009

2012

2015



# Treatment Heterogeneity → Personalized Medicine - One size does not fit all!

*(From the Patient-Protection and Affordable Care Act, 2010)*

Research shall be designed, as appropriate, to take into account **the potential for differences in the effectiveness of health care treatments, services, and items** as used with various **subpopulations, such as racial and ethnic minorities, women, age, and groups of individuals with different comorbidities, genetic and molecular sub-types, or quality of life preferences** and include members of such subpopulations as subjects in the research as feasible and appropriate.



# Risk-Based Treatment Heterogeneity

## Step One: Predicting Risk

**Predicting Risk** – an epidemiologic analysis using patient characteristics to determine risk of adverse outcomes – what docs try to do with every patient.

**General premise:** patients at higher risk are more likely to benefit from preventive measures

- a. Framingham Risk Score – 10-yr risk of coronary heart disease
- b. Oncotype DX – – a panel of genetic markers in breast cancer tissue that predicts risk for recurrence after initial surgical treatment. Women with high-risk scores are prescribed adjuvant chemotherapy after surgery.



# Step Two: Examining Benefits and Harms of Treatment By Level of Risk



BMJ 2015;350:h454 doi: 10.1136/bmj.h454 (Published 19 February 2015)

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RESEARCH



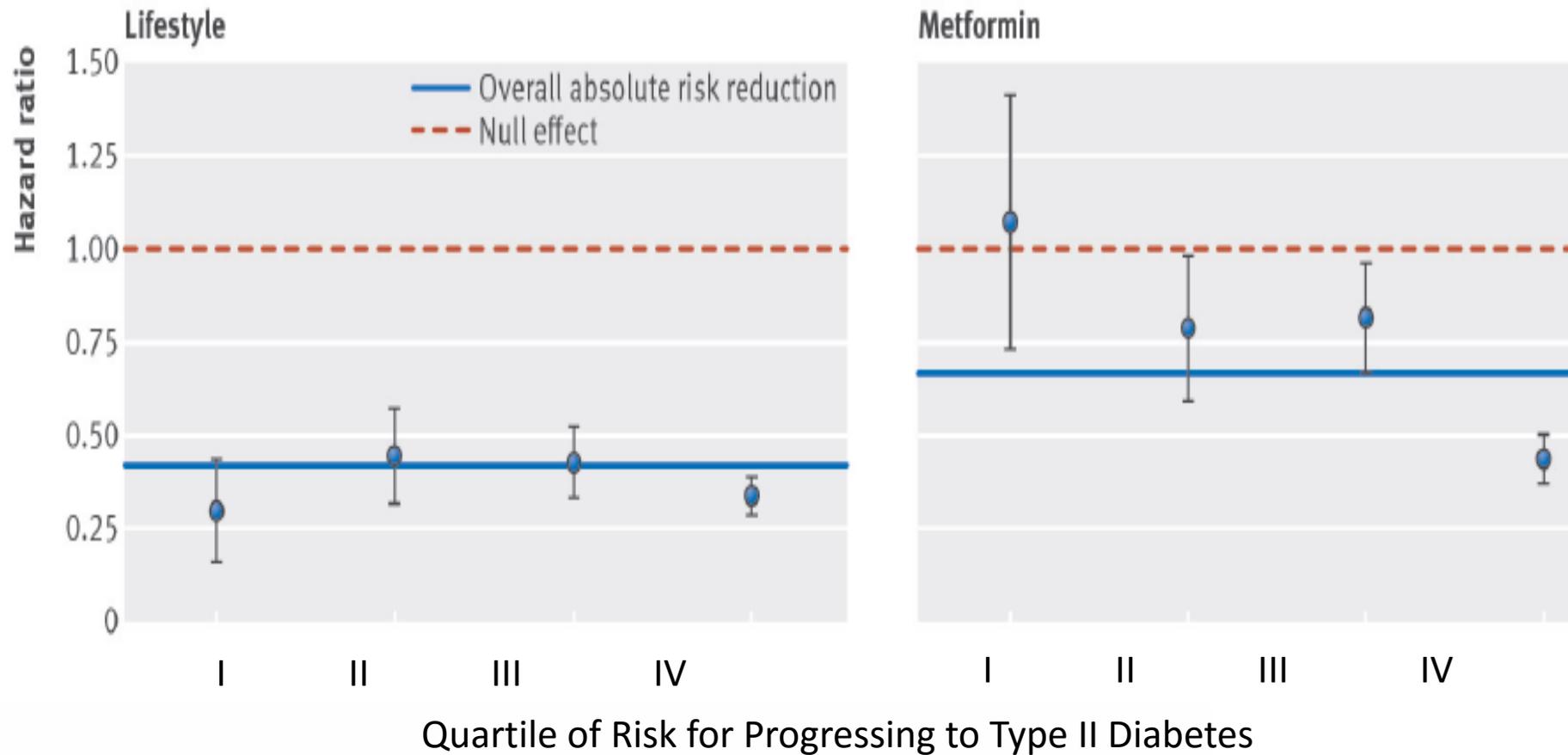
## Improving diabetes prevention with benefit based tailored treatment: risk based reanalysis of Diabetes Prevention Program

 OPEN ACCESS

Jeremy B Sussman *research scientist*<sup>1</sup> *assistant professor*<sup>2</sup>, David M Kent *professor of medicine and director*<sup>3</sup>, Jason P Nelson *statistician*<sup>3</sup>, Rodney A Hayward *professor of medicine*<sup>1 2 4</sup>



# Metformin and Lifestyle for Preventing Type 2 Diabetes – by Level of Risk at Baseline



Research

JAMA Neurology | **Original Investigation**

# Targeting Pioglitazone Hydrochloride Therapy After Stroke or Transient Ischemic Attack According to Pretreatment Risk for Stroke or Myocardial Infarction

Walter N. Kernan, MD; Catherine M. Viscoli, PhD; Jennifer L. Dearborn, MD; David M. Kent, MD; Robin Conwit, MD; Pierre Fayad, MD; Karen L. Furie, MD, MPH; Mark Gorman, MD; Peter D. Guarino, PhD; Silvio E. Inzucchi, MD; Amber Stuart, BA; Lawrence H. Young, MD; for the Insulin Resistance Intervention After Stroke (IRIS) Trial Investigators

**IMPORTANCE** There is growing recognition that patients may respond differently to therapy and that the average treatment effect from a clinical trial may not apply equally to all

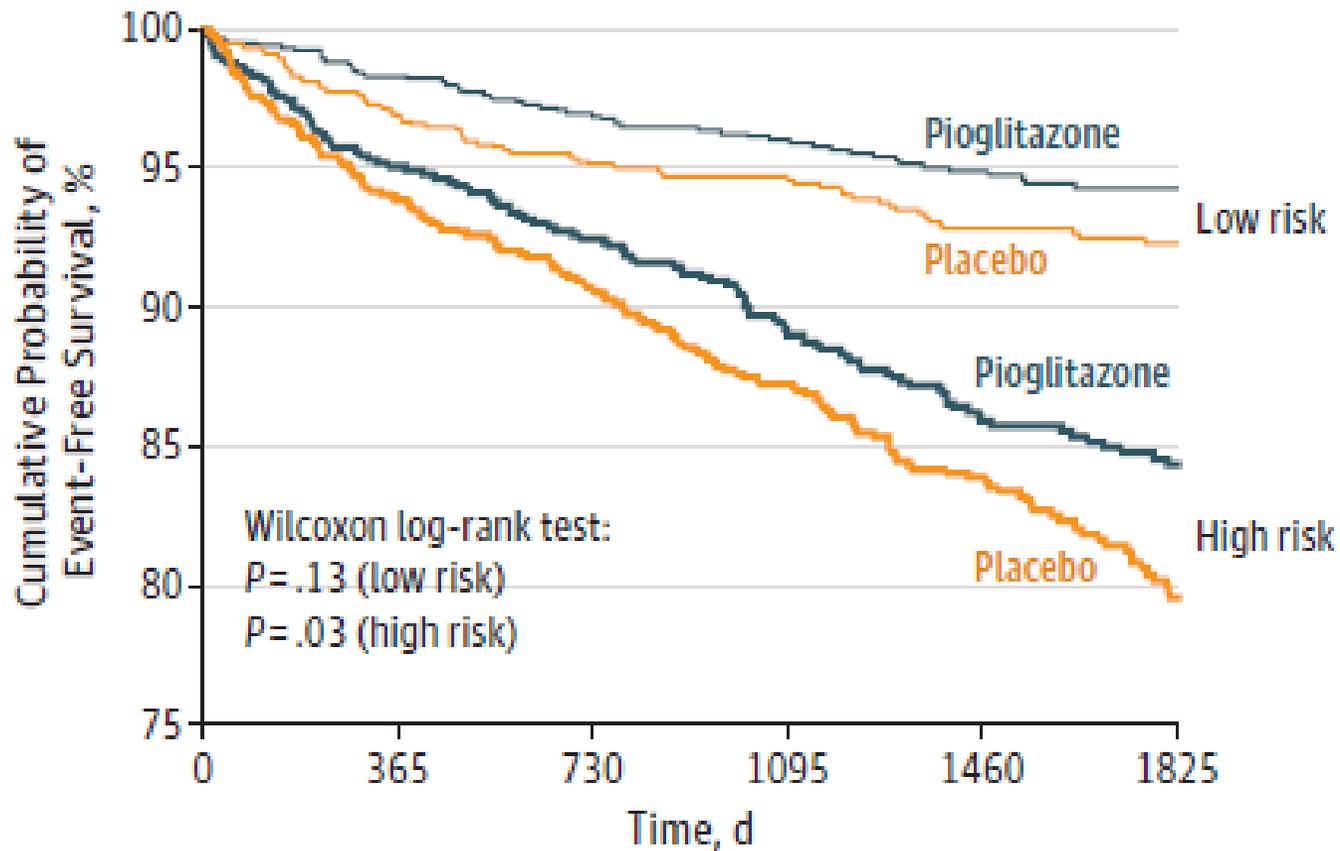
[← Editorial](#)

[+ Supplemental content](#)



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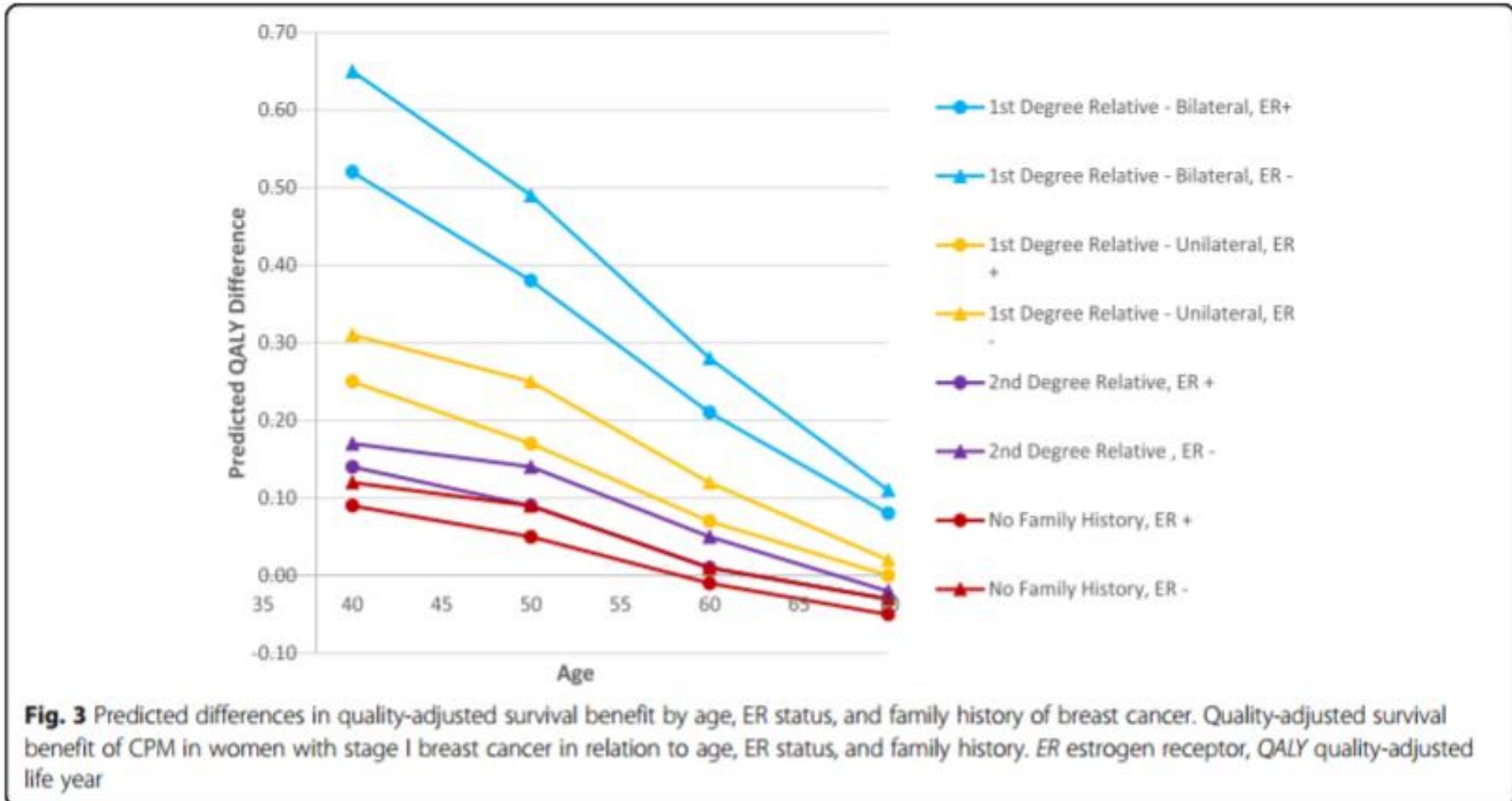
Figure. Time to First Stroke or Myocardial Infarction (MI) by Treatment Group and Baseline Risk Strata



No. at risk



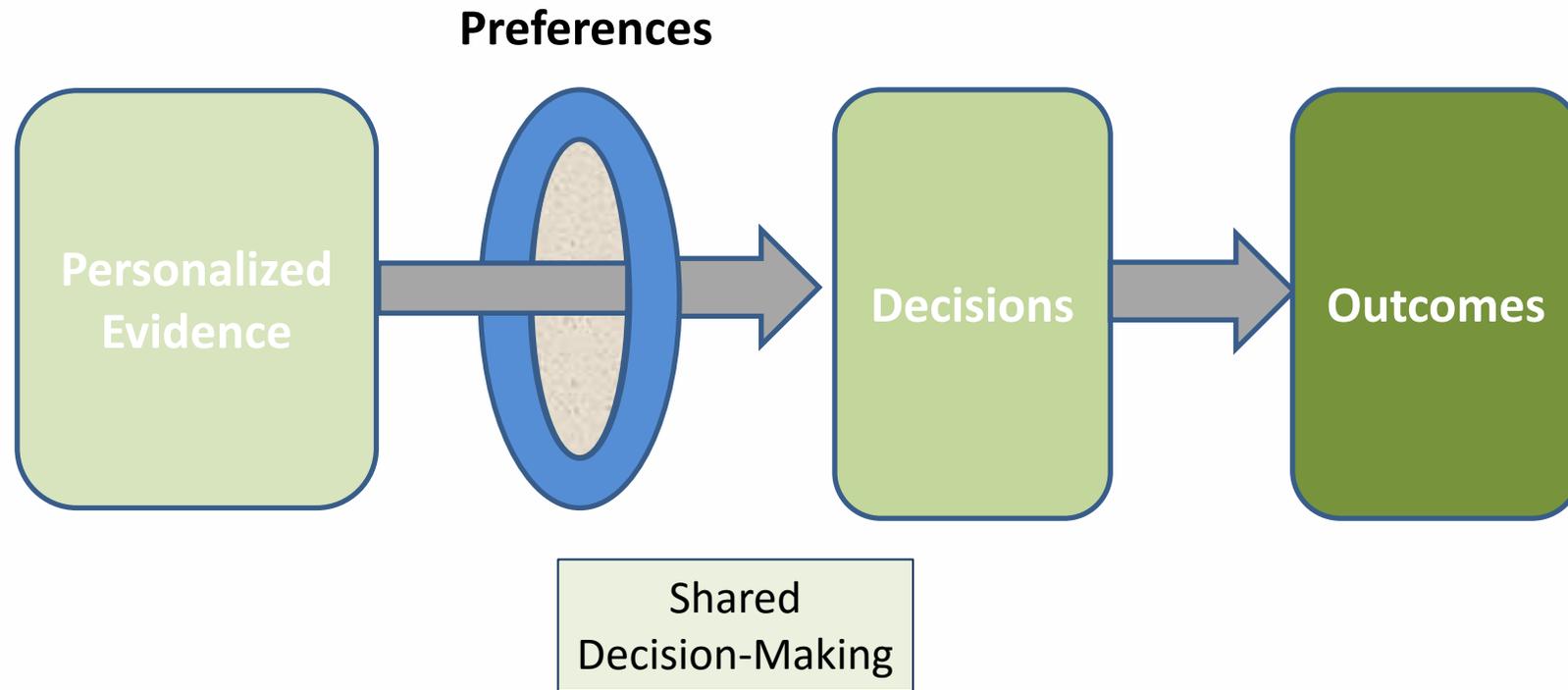
# Should women who have had breast cancer and are BRCA1/2 negative ever consider contralateral prophylactic mastectomy?



Davies et al. Breast Cancer Research (2016) 18:93



# Step 3: Apply Patient Preferences to the Complex Personalized Evidence



# WISDOM: A Preference-Tolerant RCT of Personalized vs. Annual Screening for Breast Cancer

**Study Population:** Women aged 40 years and above with no history of breast cancer.

**Comparators:** Risk-based screening based on multiple genetic tests and personal characteristics vs. recommendation for annual screening

**Design:** preference tolerant randomized trial; non-inferiority for effectiveness; superiority for false positives, radiation exposure

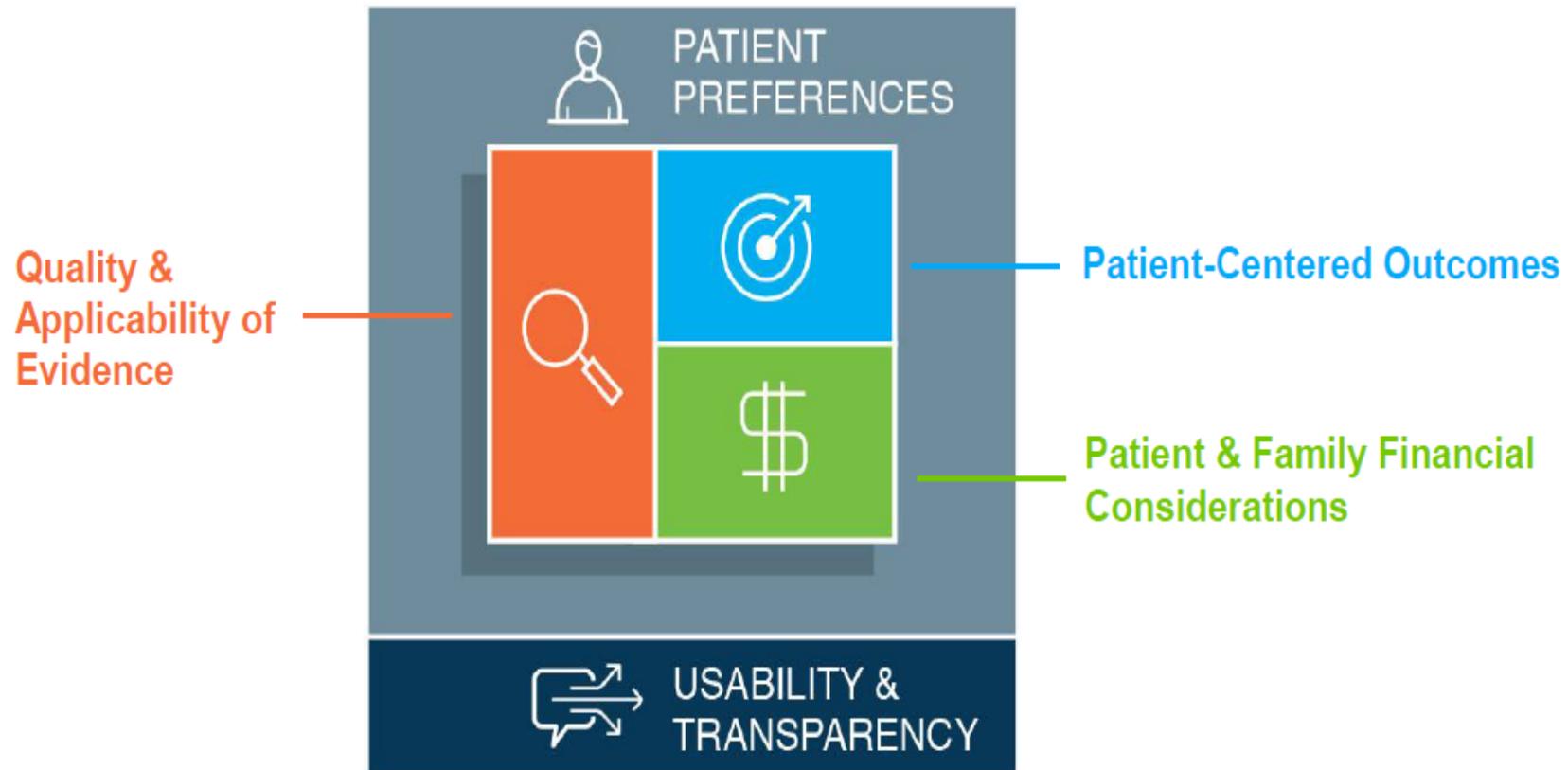
**Outcomes:** incidence of lymph node positive cancer, interval cancers, false positive mammography recalls, negative biopsies, overall diagnostic procedures, patient anxiety, incidence of estrogen receptor (ER) positive disease, of ER negative and Her2 positive disease, adherence to screening recommendations, satisfaction and confidence in breast health decisions



Laura Esserman, MD



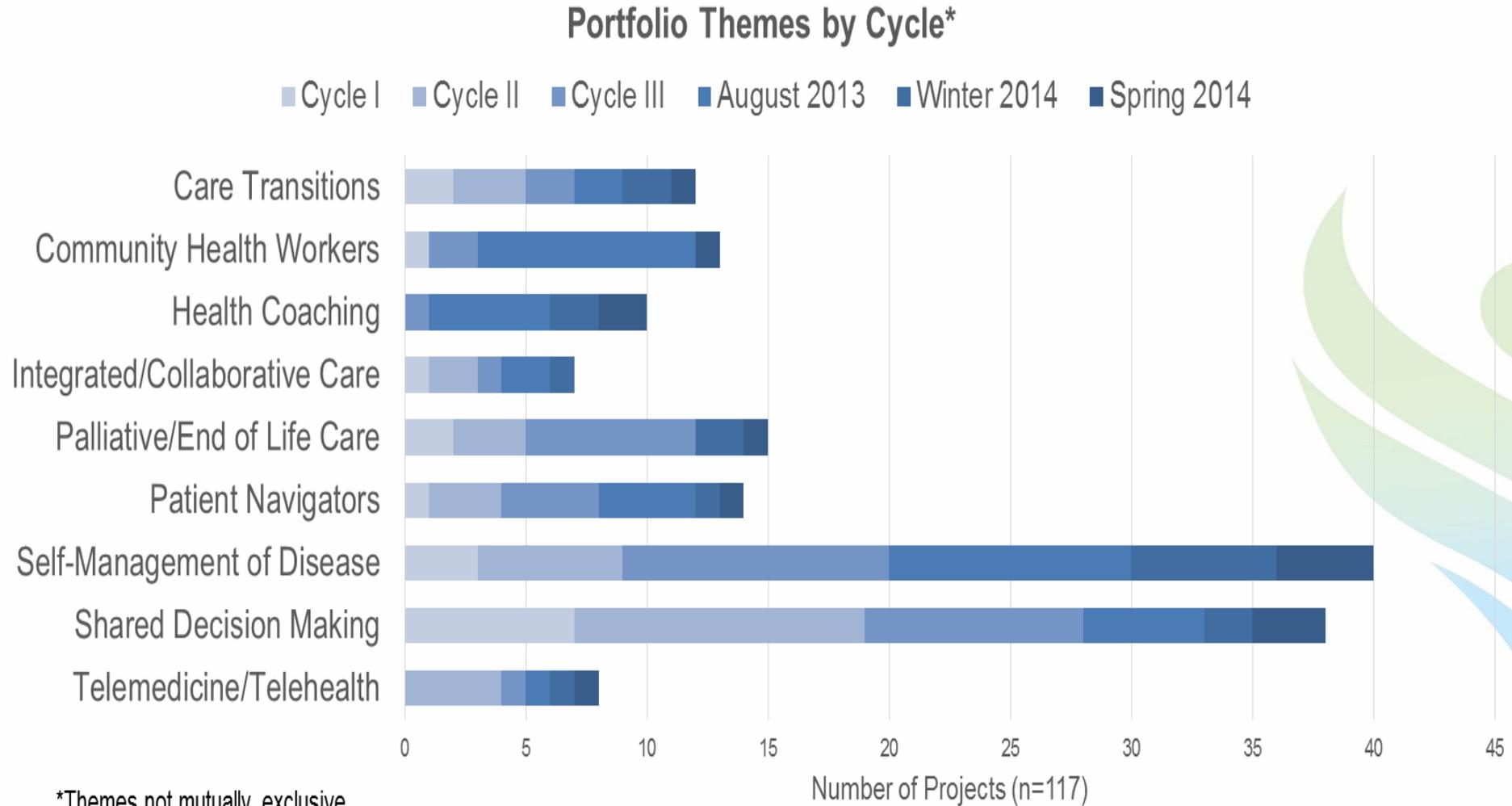
# A Proposed Patient-Perspective Value Framework



From: Patient-Perspective Value Framework: Draft Methodology. A Project of Avalere and FasterCures



# Research on Patient-Centered Care – 2012-14



# A Closing Suggestion and Some Predictions

- Patients need a prominent seat at the Quality Measurement Table
- They will likely be some resistance and their presence will change the discussion and ultimately the metrics selected
- Metrics may align much better with processes integral to good patient-centered care (e.g, linking processes of care with actual level of risk) AND with patient-centered outcomes of care and therefore with outcomes research (e.g., ICHOM disease-specific measures, 4 D's
- There will likely be a stronger emphasis on measures that cut across diseases; and on measures of adverse effects and over-treatment

