Comparative Effectiveness Research: Importance and Utility for CMS Reflections from NIA

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“Begin with the End in Mind”

CMS Mission

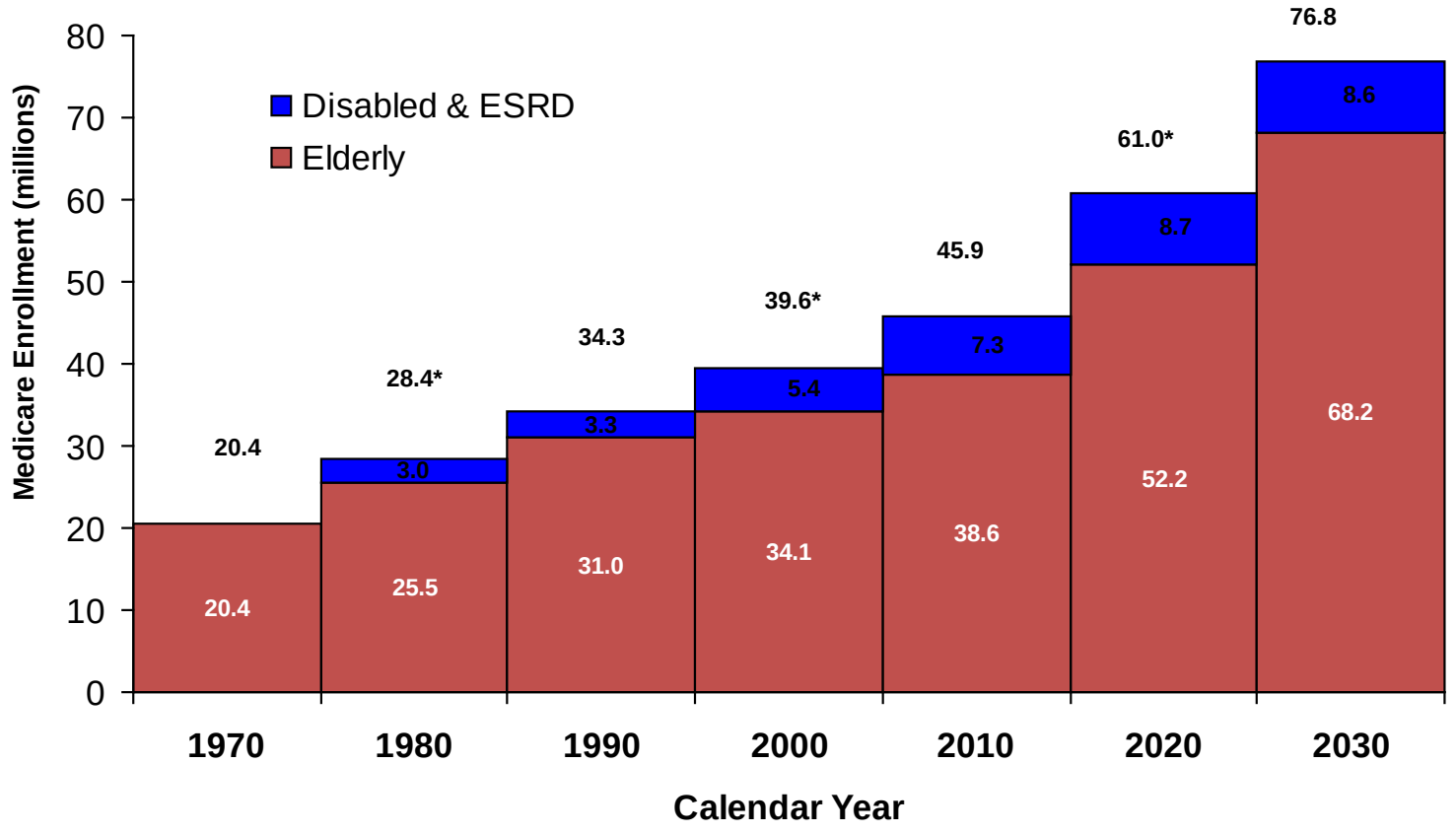
- To ensure effective, up-to-date health care coverage and to promote quality care for beneficiaries

CMS Vision

- To achieve a transformed and modernized health care system.
- CMS will accomplish our mission by continuing to transform and modernize America's health care system.

Steven Covey; CMS website

Number of Medicare Beneficiaries 1970-2030



* Numbers may not sum due to rounding.

Source: CMS, Office of the Actuary.

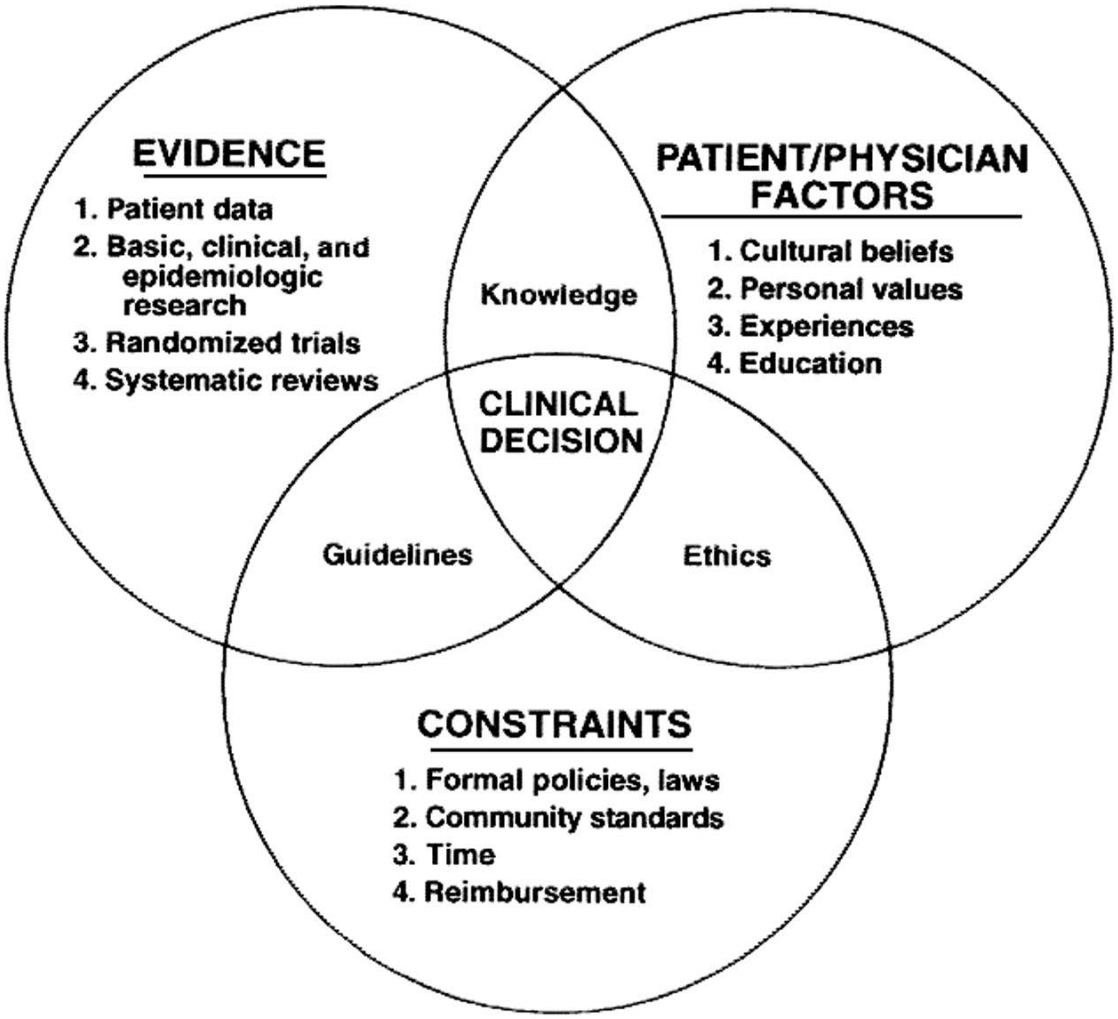
ACA Expands Coverage: 2014--

- Expand Medicaid to all individuals under age 65 with incomes up to 133% of the poverty level ($14,400/individual or $29,300/family of 4).
  - Impact 19.4 million additional covered lives by 2019.
- Create new Health Insurance Exchanges where individuals and small employers can purchase coverage (subsidized for eligible individuals and families with incomes up to 400% of the poverty level)
  - Impact 15.9 additional covered lives by 2019.
- Medicare covered lives unchanged, growing

Donald Berwick, MD

- Improve the health of the population;
- Enhance the patient experience of care (including quality, access, and reliability); and
- Reduce, or at least control, the per capita cost of care.

Clinical decision-making



Mulrow C D et al. Ann Intern Med 1997;126:389-391

CMS Needs Information

- Should we pay for this service? = Coverage
- Did we pay for the correct service correctly? = Payment
- Was the service we paid for performed optimally? = Quality
- How should we transform health care? = Innovation

- CER: Comparative Effectiveness Research
- HTA: Health Technology Assessment
- EBM: Evidence-Based Medicine

CER, HTA and EBM

- *Comparative effectiveness research (CER)*
  - Research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in "real world" settings
  - Evidence generation and synthesis
- *Evidence based medicine (EBM)*
  - Evidence synthesis to assist patients' and/or physicians' decisions. Individual clinical decision making (also clinical guidelines and quality measures)
- *Health technology assessment (HTA)*
  - Evidence synthesis used to inform reimbursement coverage decisions Considers clinical effectiveness, safety, cost-effectiveness (benefits vs. harms and economic evaluation)

International Working Group for HTA Advancement. Luce BR, Drummond MF, Jonsson B, Neumann PJ, Schwartz JS, Siebert U, Sullivan SD. EBM, HTA, and CER: Clearing the Confusion. *Milbank Memorial Fund Quarterly. 2010;88:256-276.*

Coverage

Should we pay for this service?

Carotid Stenting Coverage Decision

- Patients who are at high risk for carotid endarterectomy (CEA) and symptomatic carotid artery stenosis ≥ 70%, covered for FDA-licensed CAS systems
- Otherwise (symptomatic carotid artery stenosis between 50% and 70%, and asymptomatic carotid artery stenosis ≥ 80%), in accordance with
  - NCD: post approval studies
  - Category B IDE clinical trials regulation
  - clinical trials policy

http://www.cms.gov/mcd/viewdecisionmemo.asp?id=157

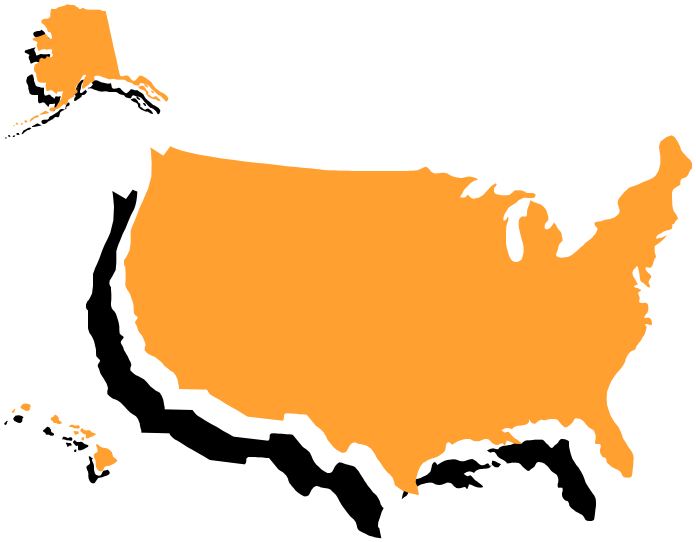
CT Colonography Coverage Decision

- "The evidence is inadequate to conclude that CT colonography is an appropriate colorectal cancer screening test …. CT colonography for colorectal cancer screening remains noncovered."
- Findings not necessarily generalizable
  - (age 58 vs ~75 years)
  - Potential risks incompletely evaluated
- USPSTF: Insufficient evidence

http://www.cms.gov/mcd/viewdecisionmemo.asp?id=220; N Engl J Med 2009; 360:2699-2701

CMS National Coverage Decisions

- National Coverage
- National Noncoverage
- National Coverage with restrictions
  - Specific subpopulations
  - Specific providers/facilities
  - Evidence development (CED)



Steps to Medicare Coverage Determination and Payment

Outside of CMS:

- Congress determines benefit categories
  - This section of talk focuses on Medicare Part A/B
- FDA approves drugs/devices for market

Within CMS:

- Coverage
- Coding
- Payment

Social Security Act 1862(a)(1)(A-B)

Reasonable & Necessary

"…no payment may be made…for items or services - which, except for items and services described in a succeeding subparagraph, are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member ,… which are not reasonable and necessary for the prevention of illness"

What is a Covered Service?

An Item or Service:

- for which there is a Medicare Benefit Category (& service meets benefit category requirements)

- which is not Statutorily Excluded based on 1862 (a)(2)-(15)

- which is Reasonable and Necessary based on 1862 (a)(1)(A or B)

How Does CMS Apply R&N Today?

- Sufficient level of confidence that evidence is adequate to conclude that the item or service:
  - improves health outcomes
  - generalizable to the Medicare population
- Similar to health technology assessment conducted by other payors/systems.

CMS Assessment of Evidence

- Synthesis
  - Staff assessment
  - Commissioned assessment
- Evidence-based guidelines
- Clinical trials
- Observational studies
- Comparative effectiveness?

Quality of Evidence

- Awards or deducts points for a study's description of
  - Randomization,
  - Double blinding,
  - Withdrawals and
  - Dropouts.
- Used in CMS decision memo for acupuncture
- Typically no explicit standard described

Jadad et al. Controlled Clinical Trials 1996;17:1-12.

Health Outcomes for Coverage

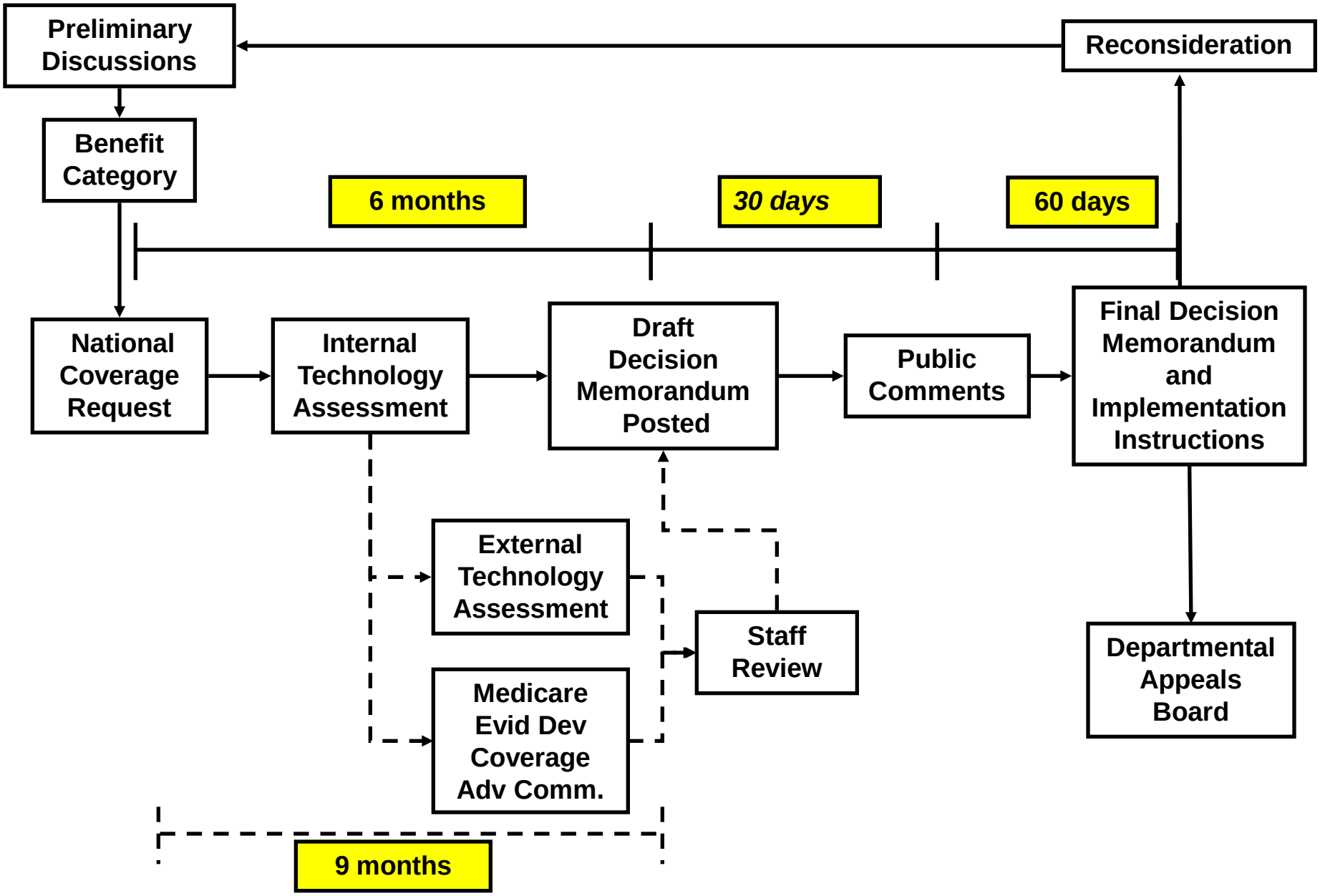
More Impressive

- Longer life and improved function/participation
- Longer life with arrested decline
- Significant symptom improvement allowing better function/participation
- Reduced need for burdensome tests and treatments

Less Impressive

- Longer life with declining function/participation
- Improved disease-specific survival without improved overall survival
- Surrogate test result better
- Image looks better
- Doctor feels confident

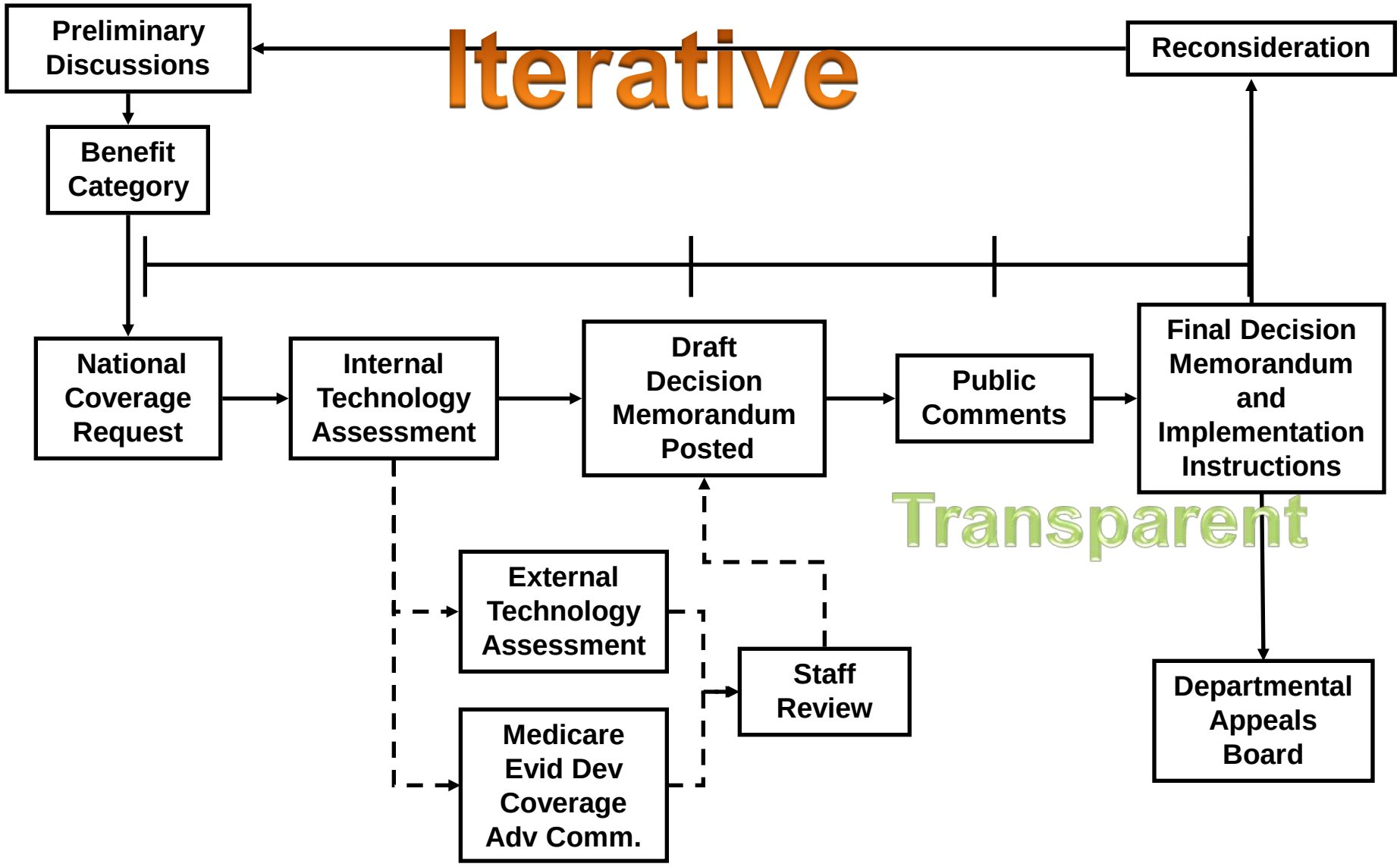
CMS National Coverage Decision (NCD) Process



CMS Assessment of Evidence

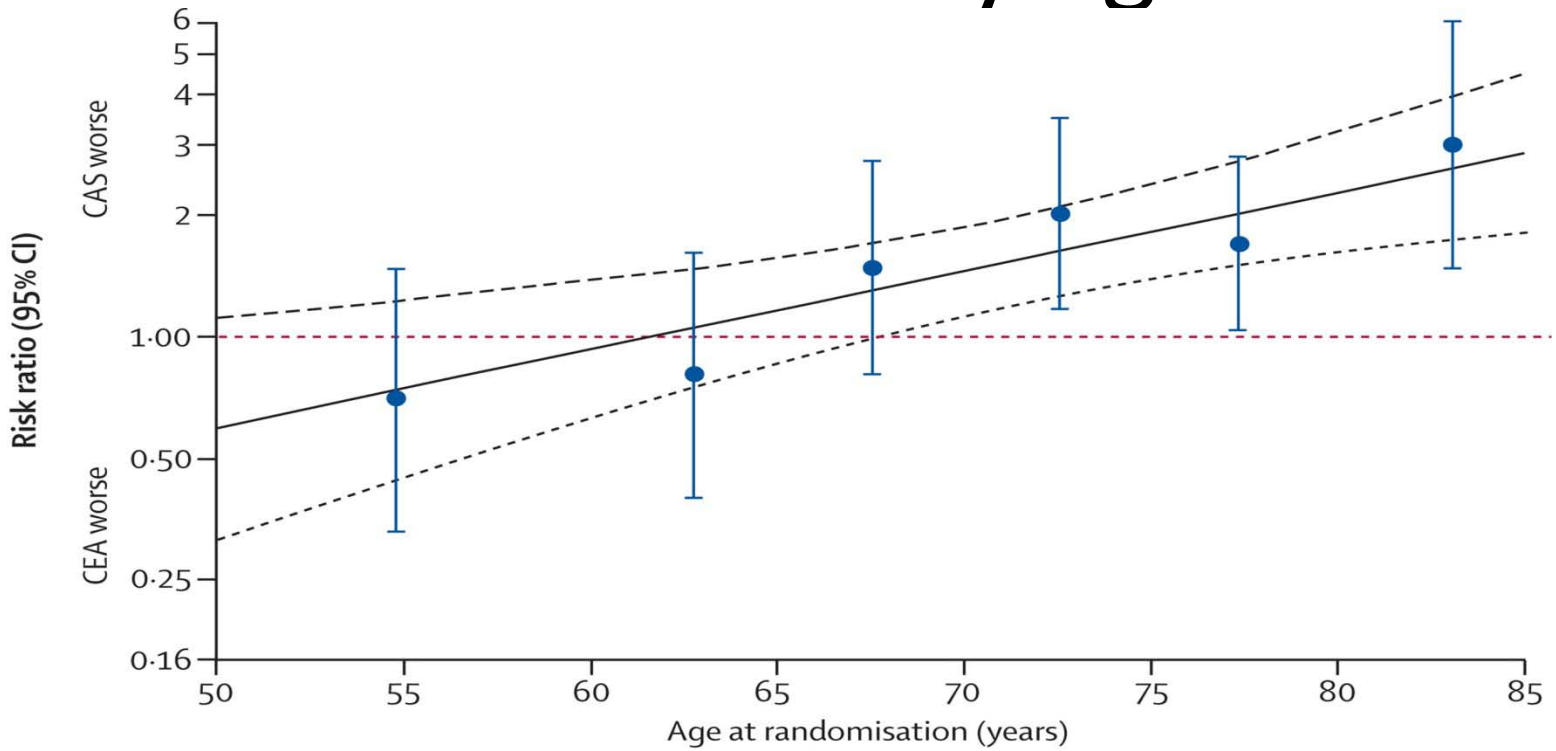
- Synthesis (or commissioned assessment)
- Evidence-based guidelines
- Clinical trials
- Observational studies
- May include CER results (ACA 1182)
  - *Iterative and transparent (public) process*
  - *Considers subpopulations*
  - *Not sole basis for non-coverage*

CMS National Coverage Decision (NCD) Process



Responsiveness to new evidence

RCT Data: Comparative effectiveness by age

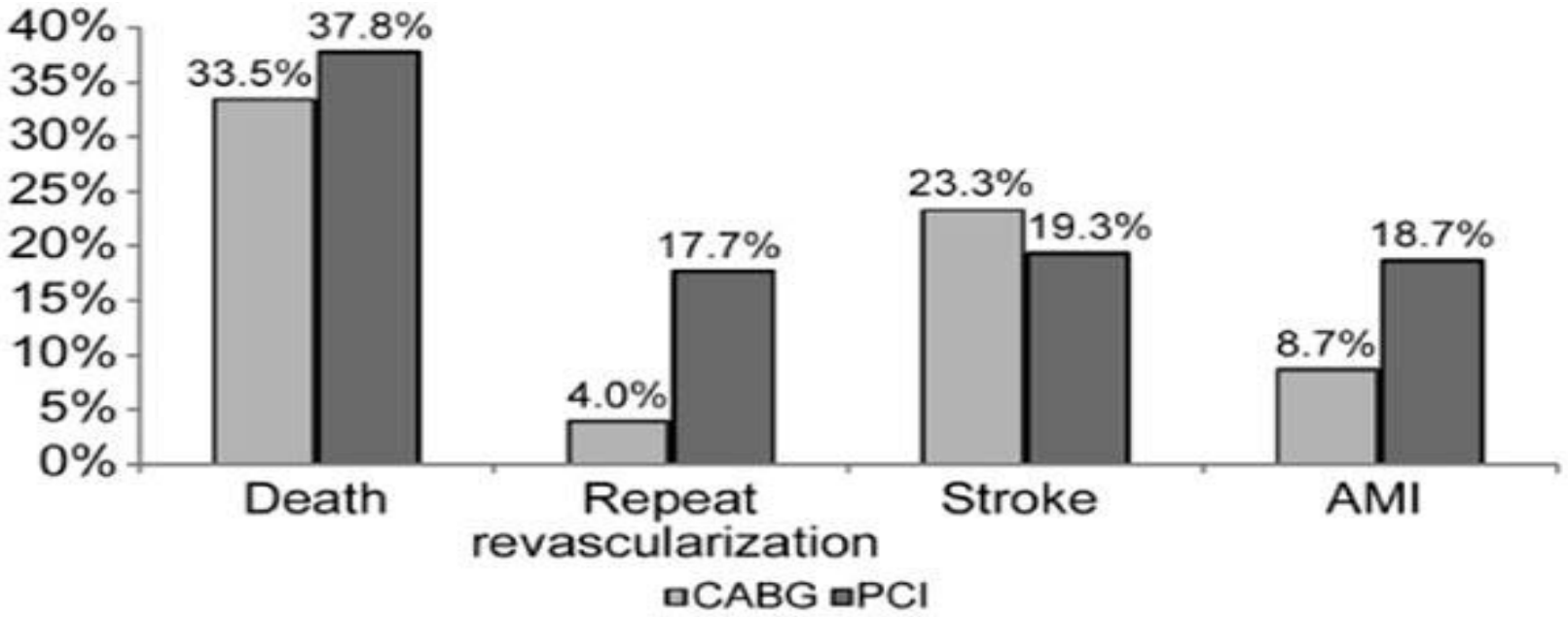


Age group (years)	<60	60–64	65–69	70–74	75–79	≥80
CAS events	11	14	25	41	35	27
CEA events	16	16	16	17	24	10

Carotid Stenting Trialists' Collaboration. Lancet 376; 2010:1062-1073.

Funding: Stroke Association

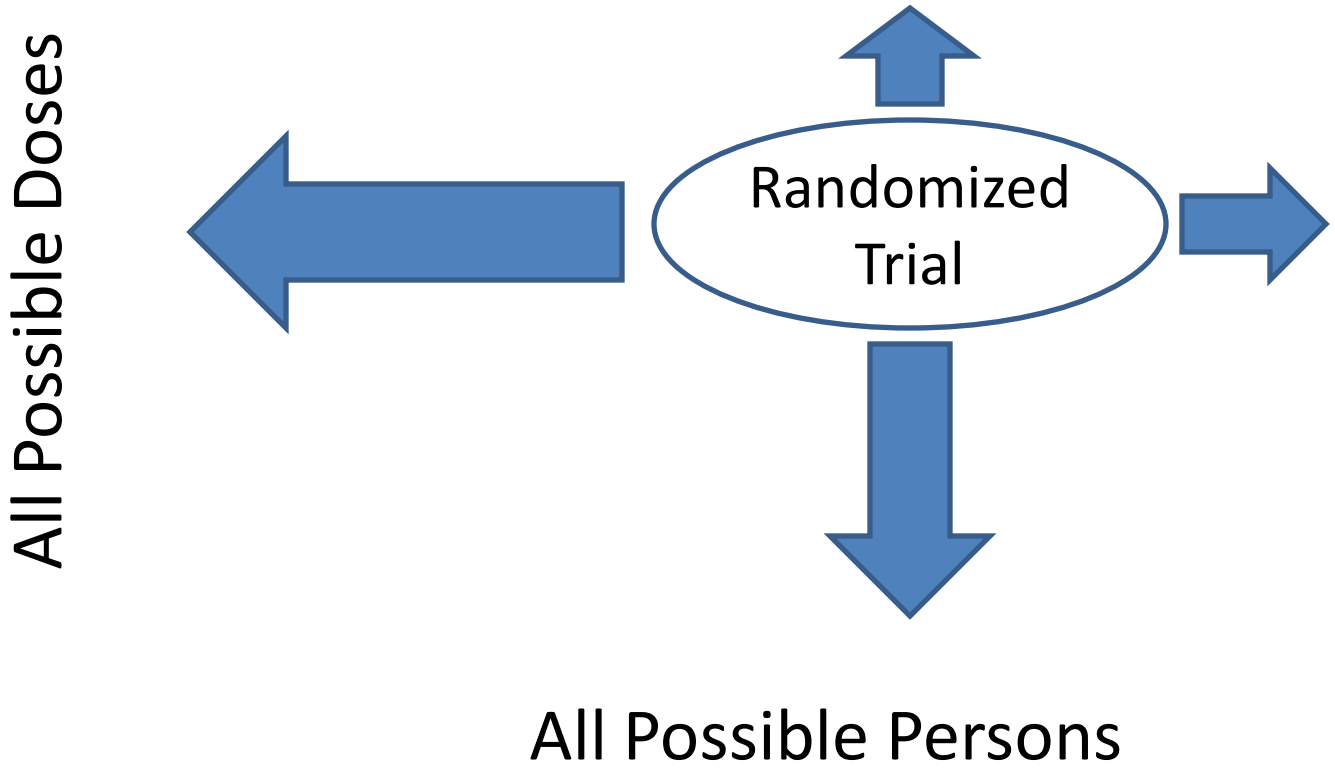
Coronary revascularization age ≥85 3-year outcomes (observational)



Sheridan et al. Ann Thorac Surg. 2010; 89: 1889–1895

Funding: NIA, NIGMS

Longitudinal studies extend and generalize RCT findings



Guralnik J, et al. JAGS 58;2010:S337-342

Economic data for coverage?

- Impacts priorities
- Use permitted for certain preventive services (MIPPA 2008 and other authority)
  - Evidence from literature
  - AHRQ-commissioned assessments
- **By practice, CMS does not use for evaluation of diagnostic & therapeutic services**
- QALY threshold prohibited for coverage decisions (ACA 1182)

Economic analysis

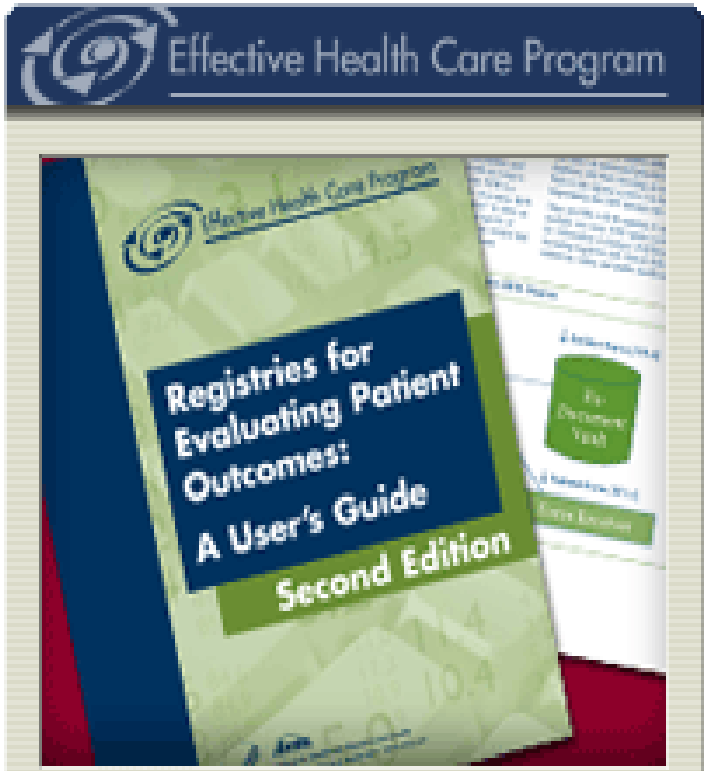
- CMS Decision memo: Screening Immunoassay Fecal Occult Blood Test (2003)
- “All FOBTs were cost-effective. Hemoccult II® at $4.50 had a cost-effectiveness ratio of $1,071 per life year gained and iFOBT at $28.00 had a cost effective ratio of $4,500 per life year saved assuming 100% compliance (lower levels of compliance would increase the cost per life year gained).”

CMS Promotes CER Data

- Coverage with evidence development
  - Registries
  - Practical clinical trials
- Linkage of claims data

User's Guide to Patient Registries

Registries for Evaluating Patient Outcomes: A User's Guide*



- The first government-supported handbook for establishing, managing and analyzing patient registries (now 2nd edition)
  - Designed so patient registry data can be used to evaluate the real-life impact of health care treatments
  - A milestone in growing CER efforts

* Co-funded by AHRQ & CMS

SEER-Medicare Linkage

- Created by linking 2 population-based sources
  - >1.5 million persons with cancer
  - Can be used to examine health care before, during and after cancer diagnosis
- SEER: detailed clinical, demographic and cause of death information for persons with cancer
- Medicare: longitudinal claims for all covered health services from time of eligibility to death

Quality Measurement

Was the service we paid for performed optimally?

Motivation to Measure Quality

- Need for accountability to oversight bodies and beneficiaries
- Desire to make evaluation of health care more objective
- Desire to improve value in government purchasing

Medicare program assessment

Outcome **Measure:** Improve the care of diabetic beneficiaries by increasing the rate of hemoglobin A1c and cholesterol (LDL) testing

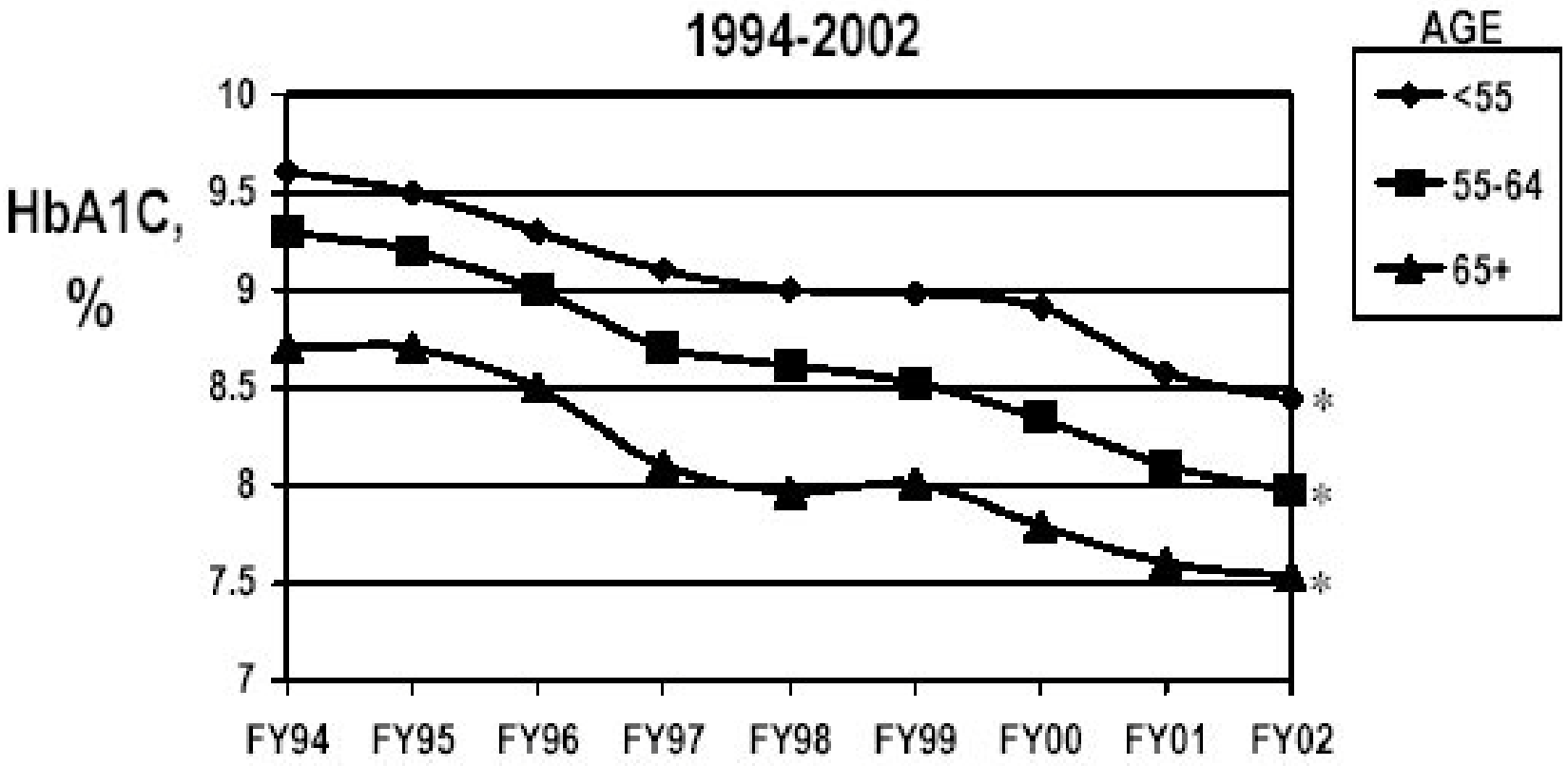
Year	Target	Actual
2005	Baselines - A1c; LDL	84.3%; 78.1%
2006	Dev. baselines/targets	Goal met
2007	85.0%; 80.0%	86.0%; 80.3%
2008	85.5%; 80.0%	Sep-09
2009	86.0%; 81.0%	Sep-10
2010	86.5%; 81.5%	Sep-11

http://www.whitehouse.gov/omb/expectmore/detail/10001060.2003.html

Diabetes Care & Outcomes Audit

Outcome: Glycemic control

1994-2002



Source: IHS National Diabetes Program Statistics 1994-2002

*p<0.0001 comparing mean HbA1C levels in FY94 and FY02

http://www.hhs.gov/of/reports/account/acct03/sect2/PARsection2.html

PQRI 01 NQF 0059

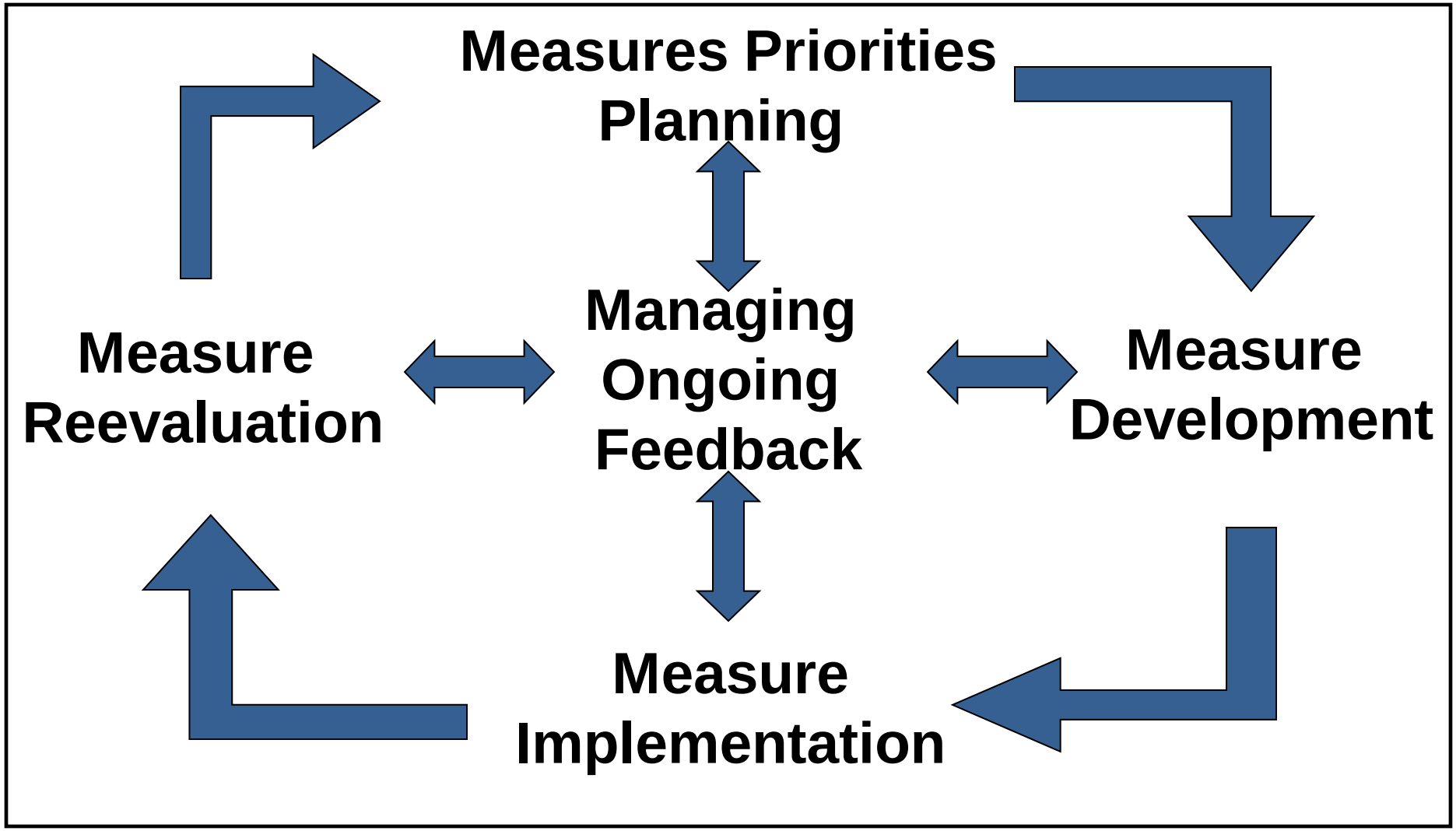
- Hemoglobin A1c Poor Control in Type 1 or 2 Diabetes Mellitus (2008)
- Percentage of patients aged 18 through 75 years with diabetes mellitus who had most recent hemoglobin A1c greater than 9.0%
- Developed by National Committee for Quality Assurance (NCQA)

https://www.cms.gov/apps/QMIS/measure_details.asp?id=790

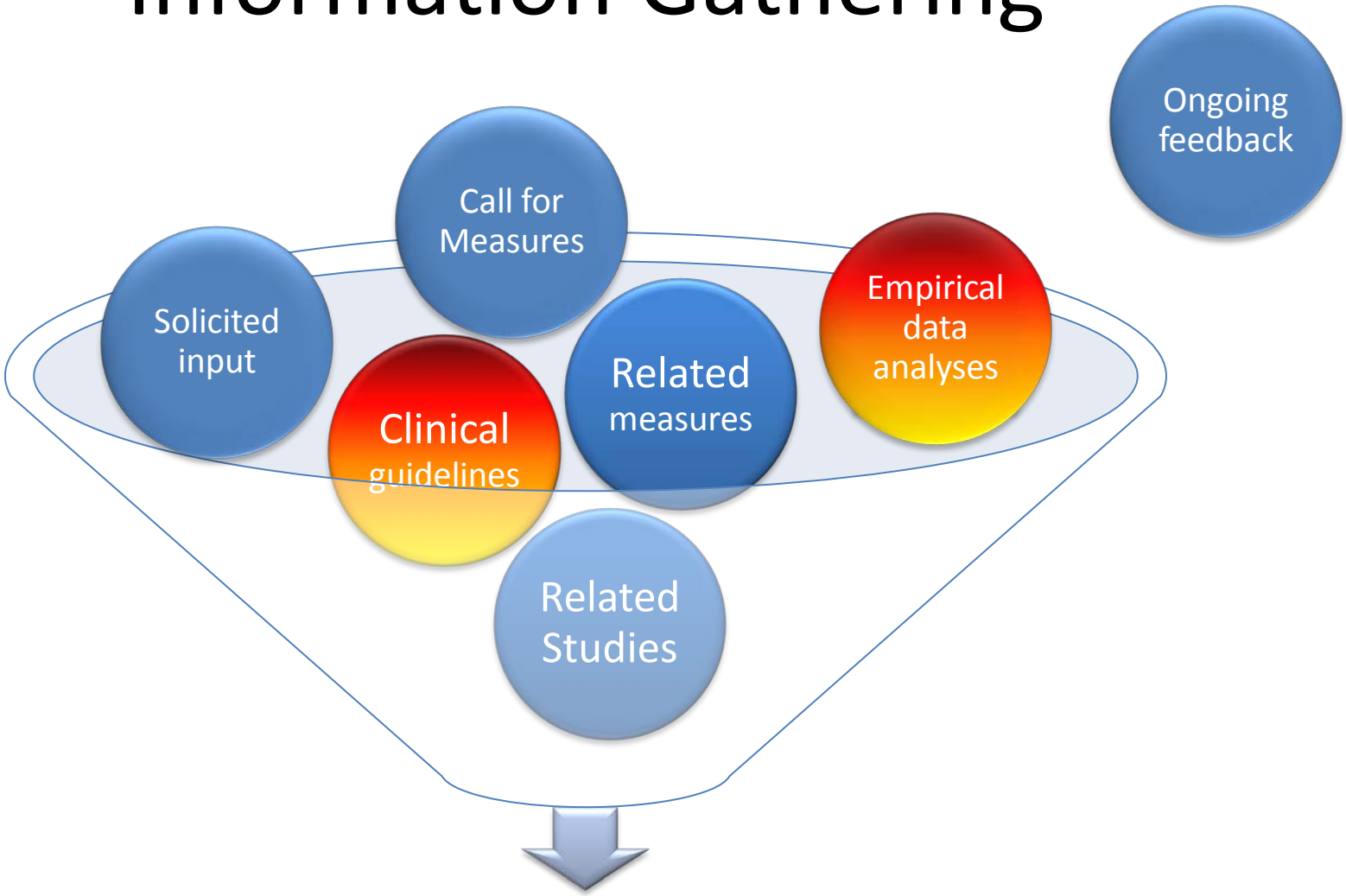
Current Quality and Performance Measure Sets

- HEDIS Healthcare Effectiveness Data and Information Set
- HOS Health Outcomes Survey
- CAHPS Consumer Assessment of Healthcare Providers and Systems
- Independent Review Entity data
- Part D Performance Measures

CMS Quality Measure Development



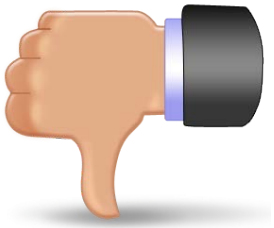
Information Gathering



Solid Foundation for Measures

Measure Evaluation

Importance?



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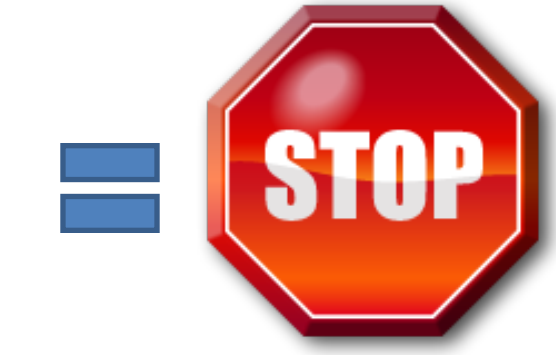

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- Scientific acceptability of measure properties
- Feasibility
- Usability

Quality measure development

- Technical Expert input
- Public comment periods
- Measure specification
  - Numerator, denominator
- Consider Risk adjustment
- Measure testing
  - Reliability, validity, feasibility
- Implementation

Many participants in process

The Joint Commission

AHRQ

NCQA 20 YEARS

CMS CENTERS for MEDICARE & MEDICAID SERVICES

NQF National Quality Forum

THE LEAPFROG GROUP Informing Choices. Rewarding Excellence. Getting Health Care Right.

RAND CORPORATION

Innovation

- How should we transform health care?
- This is fundamentally comparative effectiveness research

Center for Medicare and Medicaid Innovation

- Social Security Act 1115A, (ACA 3021) Creates CMI within CMS
- Purpose: test innovative payment and service delivery models to reduce program expenditures …while preserving or enhancing the quality of care
- give preference to models that also improve the coordination, quality, and efficiency of healthcare services

Center for Medicare and Medicaid Innovation (2)

- Model selection: there is evidence that the model addresses a defined population for which there are deficits in care leading to poor clinical outcomes or potentially avoidable expenditures.
- Model evaluation:
  - quality of care, patient-level outcomes
  - changes in spending (program level)

Further Information

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