Evidence-based health policy: PET and AD (and) The saga of explicit national coverage decision making

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Clinical practice/policy issues

- PET for diagnosis of AD
- AICD for prevention of sudden death
- Health and behavior assessment and management
- PET for cancer diagnosis, followup

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"Does having a PET scan significantly improve the likelihood of better cognitive function?
Because if it does -- as one who values my cognitive function -- I want one.
And health insurance should pay for it."

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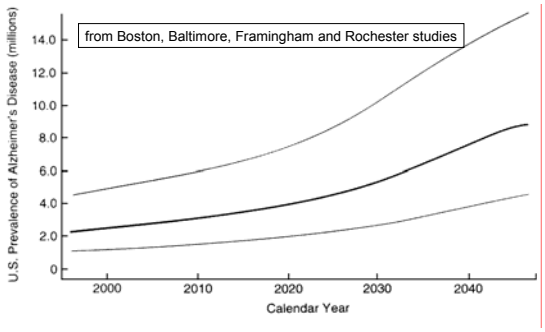
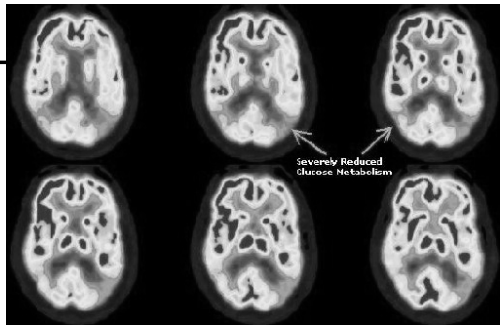
Prevalence of AD in the general population



Brookmeyer R, et al. Am J Public Health, 1998; 88:1337



www.petnetpharmaceutical.com/

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The old regime

- A world guided by personal experience and tacit knowledge
  - Scientific principles generate plausible hypotheses
  - Failures: cognitive errors
- This applied to coverage decision making (marked by informality and variability)

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The modern era: the rise of the RCT

- Archie Cochrane: The power of RCTs
- Failures: practical and ethical
  - Not all trials are feasible, generalizable and desirable

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Parachute therapy

"Parachutes reduce the risk of injury after gravitational challenge, but their effectiveness has not been proved with randomised controlled trials"

Smith GCS, Pell, JP. Parachute use to prevent death and major trauma related to gravitational challenge: systematic review of randomised controlled trials. BMJ 2003;327:1459-1461

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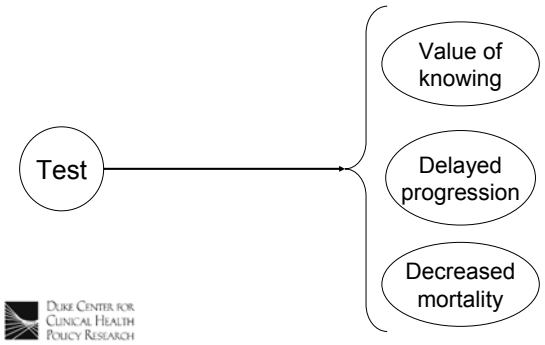
Illustration: Neuroimaging (NI) in cognitive impairment



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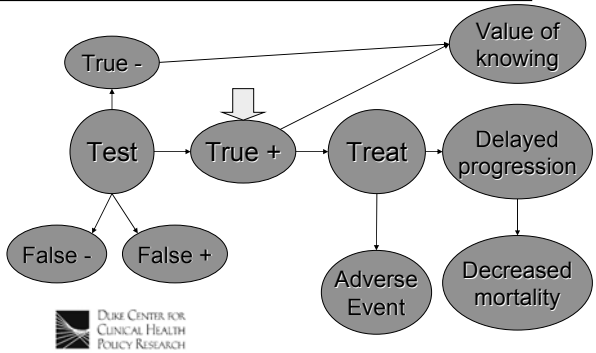
Indirect inference: causal pathway



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NI and AD diagnosis: mild dementia

	True + (%)	False + (%)	False – (%)	True – (%)	Correct (%)
No NI/ Rx	56	44	0	0	56
NI/ Rx+	49	6	7	38	87
No NI/ no Rx	0	0	56	44	44

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Many fewer false positives and a few more false negatives

Integrative Model

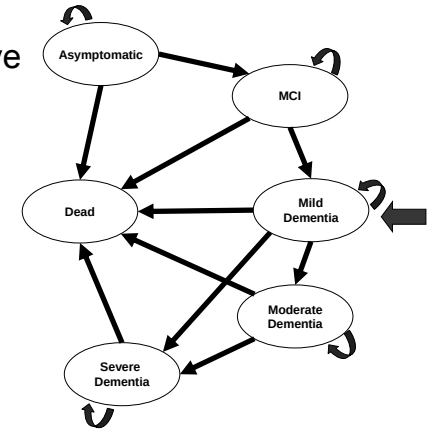
Data inputs

Epidemiological studies

Test performance studies

Drug trials

QOL/preference studies



Results: integrative model

	QALY	LE	SDFLE
No PET/ Rx	4.10	7.89	4.02
PET/ Rx+	4.09	7.88	4.00
No PET/ no Rx	4.02	7.82	3.86

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Primary conclusion

- NI could improve the overall accuracy compared to clinical exam

However,

- Treatment based on an exam leads to better health outcomes than treatment based on NI results.

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How can this make sense?

- Recall, net accuracy with NI is better because there are many fewer false positives and a few more false negatives
- However, false positives ≠ false negatives
  - Incorrectly treating (false positive) is not as bad as incorrectly not treating (false negative)
    - » Incorrectly treating: Rx is relatively benign and may be beneficial even if patient doesn't have AD
    - » Incorrectly not treating: patient loses benefit of Rx

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When testing is preferred

1. If a new treatment becomes available that is not only more effective than current therapies but is also associated with a risk of severe adverse effects.

   Increases the benefit of avoiding false positives

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When testing is preferred

2. If the results have demonstrable benefits beyond informing choice of therapies - the "value of knowing."

Improves ability to adjust and to plan

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When testing is preferred

3. If testing could be demonstrated to be a better reference standard than clinical examination.

Predicting response to therapy: compared to a standard examination, testing better distinguishes patients who respond to therapy or who will have adverse effects

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Now…a bit about Medicare coverage

Medicare is ¼ th of health care expenditures; influences the other ¾ ths

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Medicare coverage policy

- Medical services and items that are reasonable and necessary for Medicare beneficiaries under Title XVIII of the Social Security Act
  - National Coverage Decisions
  - Local Coverage Decisions
  - Denial resolved on a case-by-case basis in the absence of a decision.

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Foote. J. Health Politics,Policy and Law, 2002

Why explicit criteria?

- New technologies present a policy challenge that demands a response
- The current situation is undesirable
  - Patchwork of local coverage mandates
  - Consumer lawsuits for non-coverage
  - Dissatisfaction and distrust of providers and payors

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Medicare, 1965

- No specific coverage list in the original Social Security Amendments
- Congress delegated to HEW (now HHS)
  - May not reimburse for items and services which are not reasonable and necessary…"

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Administrative rule making

- Procedures can be defined in the enabling statute
- Otherwise, rules can be developed
  - Not requiring formal notice-and-comment
  - "Legislative" or "substantive"
- Agencies prefer legislative rule making

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Stakeholder preferences

Physicians and patients:

- Impacted but MD and patient groups are not major participants in the debate about how evidence should relate to coverage (why not?)

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Industry individually and via assns.

- Major player
- Wants
  - Clarity: sufficient certainty for planning
  - Low barrier to the marketplace

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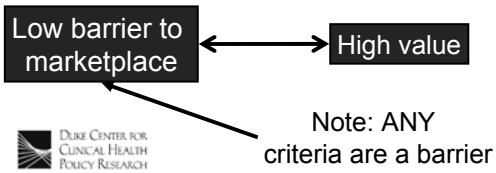
Stakeholder preferences

- CMS (was HCFA)
  - Societal representative
    - » Protector of the Medicare Trust Fund
  - Wants clarity and high value



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From 1965

- R&N judged via implicit, local review, rarely national informal review
- 1974: highly visible technologies (e.g., CT, dialysis), increased stakes
  - OTA (advisory to Congress)
  - NCHCT (advisory to federal health insurance programs)
  - OCP (within HCFA)

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National coverage policy: Take 1

- 1970s, heart transplant development catalyzed national coverage policy effort
- 1979: request for coverage accepted (limited to Stanford; others objected)
- 1980: HHS reversed, commissioned special study
- 1987: Transplant accepted, with limits

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National coverage policy: Take 1 (cont'd)

- Draft policy in 1980
- Goal: provide beneficiaries with best technology while protecting the Trust Fund
- Criteria: safety, economics, ethical, social factors
- Process: NCHCT/HCFA collaboration

Died, NCHCT folded in 1981

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1980s interlude

- Focus moved to controlling costs via reimbursement methodology
- Congress expanded NCHSR to include OHTA and authorizing National Advisory Council on Health Care Technology Assessment
  - Review and make recommendations

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National coverage policy: Take 2

- 1986, new legal challenges to coverage process
- 1989, HHS settled by agreeing to be "explicit", initiated "Notice of Proposed Rulemaking (NPRM)"
- Same as 1979 plus "cost-effectiveness"

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National coverage policy: Take 2 (cont'd)

- Hospital, doctors, industry liked clarity, hated cost-effectiveness
  - "A foundation for rationing"
- FDA hated use of "safe and effective" criterion
- NIH hated failure to pay for experimental procedures (especially cancer therapies)

1992, #2 died: HHS called for more discussion

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1990s: interlude

- Focus on reimbursement for devices classified as IDEs (HHS, IG)
- Would have just been a step in poo, if not for a 1996 court decision (*Cedars-Sinai v. Donna Shalala*): strong signal from courts that rule making was "substantive"

National coverage policy: Take 3

- Under 1989 NPRM, HCFA moved in 1996 to issue a final rule
- Change from 1989:
  - "safety and effectiveness" ➔ "demonstrated medical effectiveness"
  - "cost-effectiveness" ➔ "comparability"
- Medical device industry rejected this, on procedural grounds (i.e., new NPRM needed)

Late 1990s: interlude

- Medicare changed course:
  - "Criteria evolve" ➔ implement criteria without rule-making; move to national coverage decisions, greater uniformity
  - Used the semisecret TAC
- Industry lobbied, particularly against potential of national non-coverage
- TAC disbanded
- Industry lobbied for legislative rule making

Late 1990s: interlude (cont'd)

- HCFA resists Congressional pressure, creates the Medicare Coverage Advisory Committee (MCAC) to replace TAC
  - Maximum of 120 HHS-appointed members, an executive committee and special panels
  - An open process
- Internal recommendations for evaluating effectiveness, an NPRM was promised

National coverage policy: Take 4

- May 2000, HCFA issued an NOI to Publish a Rule
  - Based on “medical benefit” and “added value”
- Industry expressed concern about “added benefit” implies economic evaluation in disguise and requested withdrawal of the NOI

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Current status: common law for criteria

- An open process, meticulous documentation, easy access via the CMS web site and public meetings of the MCAC
- The accumulated decisions constitute a common law

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The post-modern era

Maximize our public investment in research

- Start with an integrative model
  - Conceptual and calculational
  - Use the best data for its best purpose
    - » Epi studies → natural hx; RCTs → efficacy, etc.
- Determine if the current evidence is sufficient for decision making (closure)
- Use the model to guide research design
  - RCTs, cohort studies, registries, etc.
- Link the process to clinical practice
  - Guidelines, decision support, etc.

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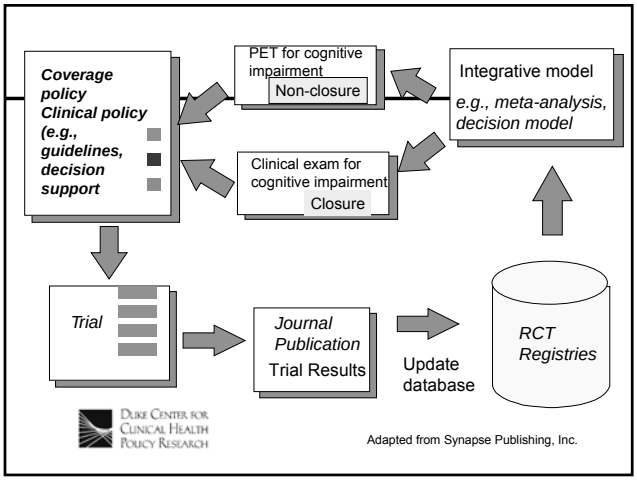

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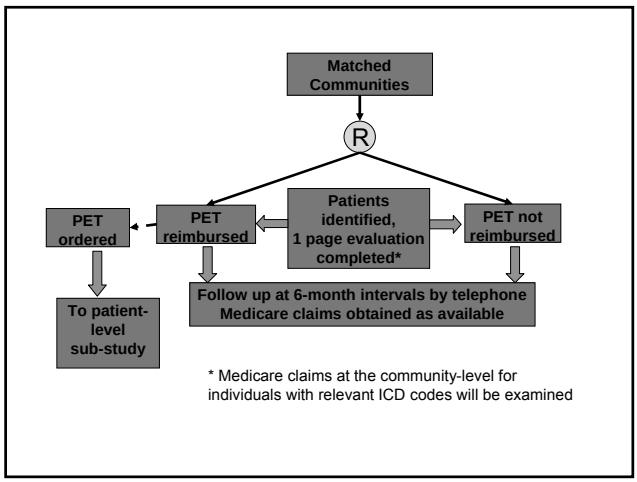
Adapted from Synapse Publishing, Inc.


Matched Communities
R
PET ordered
PET reimbursed
Patients identified, 1 page evaluation completed*
PET not reimbursed
To patient-level sub-study
Follow up at 6-month intervals by telephone
Medicare claims obtained as available
* Medicare claims at the community-level for individuals with relevant ICD codes will be examined