

**Day one-presentation-session III**  
**Jean Slutsky**

**Patient Registries and their Role in Understanding Health Outcomes**

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Patient registries have long been used in quality improvement and monitoring patient safety. More recently, there is renewed interest in the role of patient registries in better understanding health outcomes. For patients who are not well represented in traditional clinical studies because they have rare diseases, are at extremes of the age spectrum, have multiple co-morbidities, and have conditions for which meaningful health outcomes require longitudinal study, patient registries can help identify important treatment and prognostic outcomes. As with all quasi-experimental studies, there are methodological issues that must be explored that include meaningful clinical data, incentives to participate, privacy, and lack of an intrinsic control group. This session will discuss:

- The role of registries in understanding health outcomes in different populations
- Current ARRA and Comparative Effectiveness Research activities involving patient registries
- Methods, incentives, and privacy issues
- Collaborative activities



*Uniting Rare Diseases*

*Advancing Rare Disease Research:  
The Intersection of Patient Registries, Biospecimen Repositories  
and Clinical Data*

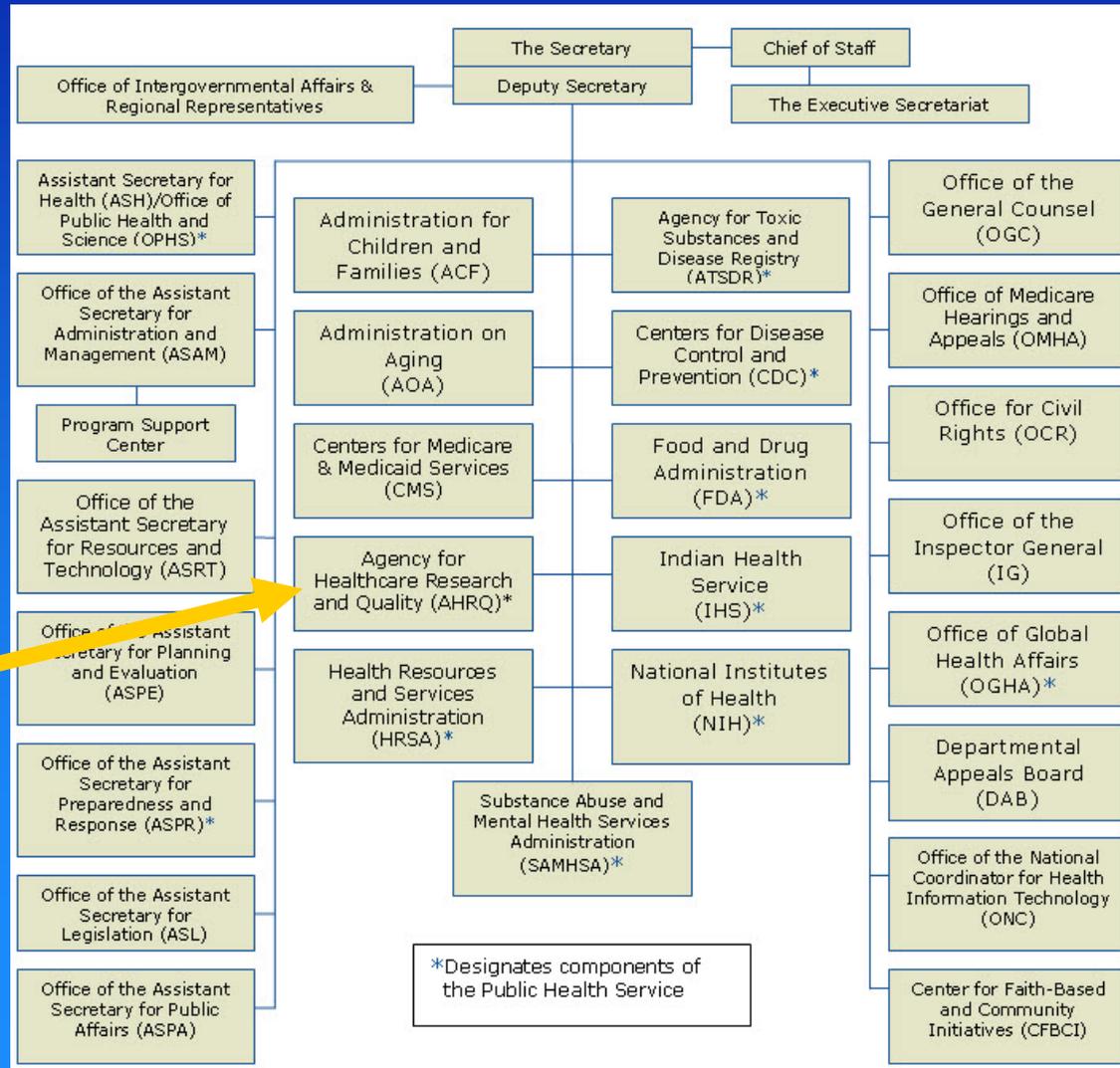
*Session III  
Clinical Research, Patient Care and Disease Management*

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Director, Center for Outcomes and Evidence  
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*Patient Registries and their Role in Understanding  
Health Outcomes*



# AHRQ and HHS



**AHRQ**

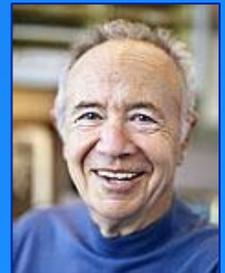


# Comparing Evidence: Medical vs. Semiconductor Research

“When I was doing semiconductor device research, it was expected that I would compare my results with other people's previously published results and that I would comment on any differences. But it seemed to be different in medicine.

“Medical practitioners primarily tended to publish their own data; *they often didn't compare their data with the data of other practitioners, even in their own field, let alone with the results of other types of treatments for the same condition.*”

*Intel co-founder and prostate cancer patient Andy Grove  
Forbes 5/13/96*





# What Healthcare Decision Makers Need To Know

- *Can it work?*
- *Will it work?*
  - For this patient?
  - In this setting?
- *Is it worth it?*
  - Do benefits outweigh harms?
  - Do benefits justify costs?
  - Does it offer important advantages over existing alternatives?

*adapted from Brian Haynes  
ACP Journal Club*



# Evaluating Effectiveness

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- Patient population: Who to give the intervention to
- Protocol of use: How to give the intervention
- Timing of use: When to give the intervention
- Provider characteristics: What are the qualifications necessary to use the intervention safely and effectively
- Setting characteristics: Where to give the intervention
- Trade-offs: Benefits and harms compared to alternatives



# Study Design Issues

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- Appropriate patient population
- Reference treatments
- Specific parameters of the intervention
- Appropriate outcome measures
- Statistical Issues
  - Power of studies
  - Dropouts/Intention-to-treat analysis
- Time scale of studies/follow-up
- Reporting of results



**CAUTION**

**THIS SIGN HAS  
SHARP EDGES**

**DO NOT TOUCH THE EDGES OF THIS SIGN**



**ALSO, THE BRIDGE IS OUT AHEAD**



**DO NOT TOUCH THE EDGES OF THIS SIGN**



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# Unadjusted and Adjusted 24-Month Outcomes Based on 6-Month Patient-Reported Clopidogrel Use

**Table 2.** Unadjusted and Adjusted 24-Month Outcomes Based on 6-Month Patient-Reported Clopidogrel Use

	No. at Risk at 6 Months	No. of Events in Interval			No. at Risk for Mortality at 24 Months	Unadjusted Outcomes, %			Adjusted Outcomes, %		
		Death	Nonfatal MI	Death or MI		Death	Nonfatal MI	Death or MI	Death	Nonfatal MI	Death or MI
DES with clopidogrel	637	7	5	11	290	1.6	0.8	2.1	2.0	1.3	3.1
DES without clopidogrel	579	21	13	31	245	5.8	3.3	8.4	5.3	2.6	7.2
BMS with clopidogrel	417	16	5	21	387	3.9	1.2	5.1	3.7	1.7	5.5
BMS without clopidogrel	1976	88	28	115	1852	4.5	1.4	5.9	4.5	1.6	6.0
<i>P</i> value (3 <i>df</i> )						.002	.09	<.001	.054	.63	.07
Interaction <i>P</i> value (1 <i>df</i> )*						.04	.054	.007	.18	.31	.12
Difference (95% CI)											
DES with clopidogrel and DES without clopidogrel						-4.2 (-7.1 to -1.4)	-2.5 (-4.5 to -0.5)	-6.3 (-9.6 to -3.1)	-3.3 (-6.3 to -0.3)	-1.3 (-3.4 to 0.8)	-4.1 (-7.6 to -0.6)
<i>P</i> value (1 <i>df</i> )						.004	.02	<.001	.03	.24	.02
BMS with clopidogrel and BMS without clopidogrel						-0.6 (-2.7 to 1.5)	-0.2 (-1.4 to 1.0)	-0.7 (-3.1 to 1.7)	-0.7 (-2.9 to 1.4)	0.2 (-1.6 to 1.9)	-0.5 (-3.2 to 2.2)
<i>P</i> value (1 <i>df</i> )						.60	.74	.55	.50	.86	.70
DES with clopidogrel and BMS with clopidogrel						-2.3 (-4.6 to -0.1)	-0.4 (-1.7 to 0.9)	-3.1 (-5.6 to -0.6)	-1.7 (-4.2 to 0.8)	-0.4 (-2.6 to 1.8)	-2.4 (-5.6 to 0.9)
<i>P</i> value (1 <i>df</i> )						.04	.52	.02	.18	.72	.16
DES without clopidogrel and BMS without clopidogrel						1.3 (-1.4 to 4.0)	1.9 (-0.1 to 3.8)	2.5 (-0.7 to 5.7)	0.8 (-1.8 to 3.5)	1.0 (-0.6 to 2.7)	1.2 (-1.8 to 4.2)
<i>P</i> value (1 <i>df</i> )						.34	.06	.12	.55	.22	.44
DES with clopidogrel and BMS without clopidogrel						-2.9 (-4.4 to -1.4)	-0.6 (-1.5 to 0.2)	-3.8 (-5.4 to -2.1)	-2.5 (-4.4 to -0.6)	-0.3 (-1.8 to 1.3)	-2.9 (-5.3 to -0.5)
<i>P</i> value (1 <i>df</i> )						<.001	.16	<.001	.01	.76	.02

Abbreviations: BMS, bare-metal stent; CI, confidence interval; DES, drug-eluting stent; MI, myocardial infarction.  
\*Interaction between stent type and clopidogrel use.



# Why Clinical Trials Often Don't Measure Effectiveness

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## ■ Patient Selection

- Exclusion of elderly patients, patients with comorbid conditions, rare conditions etc.

## ■ Intervention

- Careful adherence to protocol

## ■ Provider and Setting

- May have more experience with the procedure than in actual clinical practice

# AHRQ Registries Project



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

- Handbook for establishing, maintaining and evaluating registries.
- Collaborative effort with broad multi-stakeholder involvement.
  - Outcome Sciences DEcIDE center
  - Duke University EPC
  - CMS Coverage and Analysis Group
  - 39 contributors from industry, academia, health plans, physician societies and government
  - 35 invited peer reviewers and public comment
- Example driven: ~20 case studies illustrating specific challenges and solutions.



# Contents

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- Creating Registries
  - Planning a Registry
  - Registry Design
  - Data Elements for Registries
  - Data Sources for Registries
  - Principles of Registry Ethics, Data Ownership and Privacy



# Contents (cont)

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- Operating Registries
  - Patient and Provider Recruitment and Management
  - Data Collection and Quality Assurance
  - Adverse Event Detection, Processing and Reporting
  - Analysis and Interpretation of Registry Data to Evaluate Outcomes
- Evaluating Registries
  - Quality Domains



# Registries Handbook Part II

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- Update the existing Registries Handbook
- Analyze options to develop a registry of registries
- White papers on emerging issues
  - Use of registries in product safety assessment
  - When should a registry end?
  - Linking registry data: technical and legal considerations
  - Interfacing registries with electronic health records



# Challenges/Opportunities

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- Long term followup
  - Linking data sources
- Uniform definitions between registries
- Control groups
  - Need entry into registry at diagnosis rather than treatment
- Duplication of efforts in overlapping registries



# Core Infrastructure for CER at AHRQ

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- 14 Evidence-based Practice Centers (EPCs)
- 14 Developing Evidence to Inform Decisions about Effectiveness (DEcIDE) Centers – one Cancer Consortium
- 14 Centers for Education and Research on Therapeutics (CERTs)
- John M. Eisenberg Center for Clinical Decisions and Communications Science
- 1 CER Horizon Scanning Center - TBA
- 1 Citizen's Forum on CER - TBA



# Core Infrastructure for CER at AHRQ

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- On-going program announcement on CER
- Clinical and Health Outcomes Initiative in Comparative Effectiveness (CHOICE initiative – R01 - \$100 million)
- Prospective Outcome Systems using Patient-specific Electronic data to Compare Tests and therapies (PROSPECT Studies R01 - \$44 million)



# Conclusions

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- Lack of evidence may lead to adoption of ineffective and potentially harmful interventions
- Both randomized controlled trials and observational studies have strengths and weaknesses: careful study design and analysis is needed
- Well done registries can provide critical information on the benefits and harms of medical interventions



# AHRQ and CER

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- **Words of wisdom:** *“In theory, there is no difference between theory and practice. In practice, there is.” – Yogi Berra*
- Current information on AHRQ’s Effective Health Care Program and CER can be found at [www.effectivehealthcare.ahrq.gov](http://www.effectivehealthcare.ahrq.gov) and [www.ahrq.gov/fund](http://www.ahrq.gov/fund)

Context and perspective will be key....

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