



# **AHRQ'S Perspective On Clinical Utility**

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# Outline

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- Background
- AHRQ activities

# Background

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- Large gaps in knowledge of the impact of therapeutics and (especially) diagnostics on patient outcomes in real-world clinical practice
- Large number of interventions for common diseases: Added value of new?
- Valid information on benefits and harms is critical for decision-making: clinical; guidelines; coverage; regulatory



# Background

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- Natural history and disease pathogenesis often incompletely understood: Will improvement in surrogate markers improve patient outcomes?
- Limitations in existing infrastructure capabilities and in study methods affect validity and generalizability of conclusions
- Goals of biomedical researchers and clinical providers are typically not aligned



# Comparative Effectiveness Research (FCCER)

- Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in “real world” settings.
- The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances.

<http://www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf>



# Goals of AHRQ's Effective Health Care (EHC) Program

- ❖ Established in 2004 for conducting user-driven CER (mandated under MMA)
- Create new knowledge
- Review and synthesize existing knowledge
- Translate and disseminate findings
- Train and build capacity





# Genomics Projects

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- EPC reports: EGAPP, USPSTF, NIH, CMS, CDC, clinical societies etc.
- RCTs: Warfarin gene-based dosing, two PROSPECT projects
- Computer-based CDS tool for assessing BRCA mutation risk in primary care
- Existing infrastructure to ascertain utilization and outcomes of gene-based applications
- Analytic validity, quality rating and evaluation frameworks of genetic and lab tests



# EHC Investments in Electronic Infrastructure

- Distributed research networks: Funded '07
  - New: DARTNet (PBRN, link several EMRs)  
[http://www.effectivehealthcare.ahrq.gov/ehc/products/53/151/2009\\_0728DEcIDE\\_DARTNet.pdf](http://www.effectivehealthcare.ahrq.gov/ehc/products/53/151/2009_0728DEcIDE_DARTNet.pdf)
  - Enhancement of existing: HMORN  
<http://www.effectivehealthcare.ahrq.gov/index.cfm/search-for-guides-reviews-and-reports/?pageaction=displayproduct&productID=150>

Published in Annals of Internal Medicine (2009; 151)





# A Focus on Prospective Electronic Clinical Data

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- Build on successes and lessons learned from DRN projects
- Goal: Build multi-purpose, dynamic, electronic prospective data systems leveraging current HIT investments
- Spans several AHRQ portfolios:
  - Comparative Effectiveness
  - Health Information Technology
  - Prevention and Care Management
  - Patient Safety



# Four ARRA RFAs (> \$100 M Investment)

- CER in ARRA (2009):  
<http://www.hhs.gov/recovery/programs/cer/index.html>
- PROSPECT: Prospective Outcome Systems using Patient-specific Electronic data to Compare Test and therapies (AHRQ-ARRA; Six R01s)
- Scalable DRNs (OS-ARRA; Three R01s)
- Enhanced registries for QI and CER (OS-ARRA; Two R01s)
- Electronic Data Methods (EDM) Forum (AHRQ-ARRA; One Cooperative Agreement)



# Common Themes Across R01 Projects

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- Link multiple healthcare delivery sites
- Connect multiple databases
- Focus on priority populations and conditions
- Prospective, patient-centered outcomes
- Conduct CER
- Valid and generalizable conclusions
- Focus on governance and stakeholder involvement
- Sustainable



# Additional Features of Registry and DRN Projects

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- Registry: Build on existing registry, QI and CER project, sustainable and scalable
- Scalable DRN: Multiple cohorts (at least four pairs of increasing complexity), at least two unrelated priority conditions, near-real time, sustainable and scalable



# Electronic Data Methods (EDM) Forum

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- A central repository and resource for information on the use of prospective electronic clinical data (PECD) for CER
- The EDM Forum:
  - Collects, synthesizes, and shares lessons learned from efforts to build infrastructure and conduct CER
  - Engages stakeholders in the science and also to understand the needs and challenges in this area
  - Builds resources and tools to advance the science and understanding of CER



# EDM Forum Activities

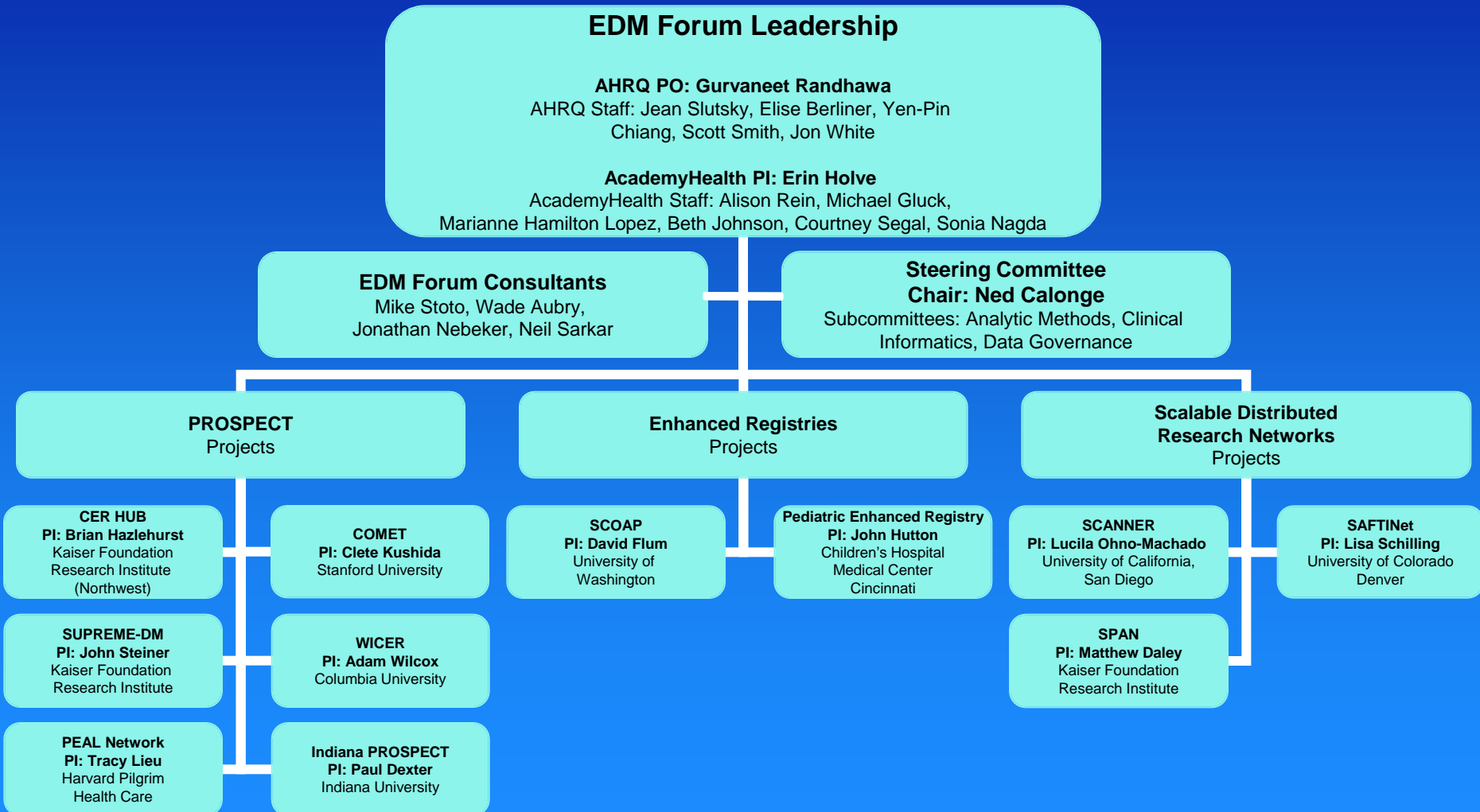
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- Analytic methods
- Clinical informatics
- Data Governance
  - Security
  - Privacy
  - Access
- CER and learning healthcare system, including:
  - Quality improvement
  - Use in clinical decision support
  - Meaningful engagement





# EDM Forum: Organizational Chart



<http://www.edm-forum.org/publicgrant/Home/>



# Questions?

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# Limitations of RCTs

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- Highly selected patient populations and clinical settings
- Not useful for detecting rare events or long-term outcomes
- Commonly report surrogate outcomes
- Not suitable for many clinical and policy questions : rapidly evolving technology, large number of variables, utilization, factors affecting adherence, preferences
- Expensive, one-off studies



# Limitations in Using Administrative Claims-based Data

- Created for billing; not research, quality improvement, or clinical decision support
- Lack details on diagnostic tests and results
- Limited clinical information: co-morbidity, disease severity, diet & supplements, type of device or procedure etc.
- Lack patient reported outcomes
- Increased risk of erroneous conclusions due to selection bias, confounding etc.
- Data obtained after considerable lag