Rationale for CER

- Large gaps in knowledge of the impact of therapeutics and diagnostics on patient outcomes in the real-world
  - Lack of alignment in goals of researchers and clinicians; focus on short-term outcomes; one-off projects; information and expertise is in silos; end-users not involved to ask “right” questions
  - Limitations in existing data infrastructure and research methods
Limitations of RCTs

- Often highly selected patient populations and clinical settings, evaluate surrogate outcomes (efficacy trials)
- Not useful for detecting rare events or long-term outcomes
- Not suitable for many clinical and policy questions: rapidly evolving technology, large number of variables, utilization, adherence, preferences
- Expensive, one-off studies
Administrative Claims Data: Cheap, Convenient but Flawed

- 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 (PROs)
- Increased risk of erroneous conclusions due to selection bias, confounding etc.
- Data obtained after considerable lag
ARRA Investment: Enhance CER Data Infrastructure

- Link EHRs to diagnostic (lab tests, imaging studies), pharmacy, claims databases
- Improve collection of PROs
- Capture patient information across diverse delivery sites: inpatient, ambulatory, long term care
- Add survey data
Four ARRA RFAs ($100 M Investment)


- 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)

- EDM Forum (AHRQ-ARRA; One Cooperative Agreement)
ARRA Funding for Electronic Clinical Data Infrastructure: EDM Forum Analysis

<table>
<thead>
<tr>
<th>Evidence development and synthesis</th>
<th>Translation and dissemination</th>
<th>Infrastructure and methods development</th>
<th>Priority Setting</th>
<th>Stakeholder Engagement</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Total ARRA-CER Funding</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>$1.1 billion</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Governance</th>
<th>Data</th>
<th>Methods</th>
<th>Training</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Infrastructure &amp; Methods Development</strong></td>
<td></td>
<td>$417.2 million (37.9% of ARRA-CER Funding)</td>
<td></td>
</tr>
<tr>
<td>Governance</td>
<td>Data</td>
<td>Methods</td>
<td>Training</td>
</tr>
</tbody>
</table>

| Clinical and claims databases, electronic health records, and data warehouses | Patient Registries | Distributed and federated data networks | Informatics platforms, systems and models to collect, link and exchange data |
|---------------------------------------------------------------------------|-------------------|----------------------------------------|---------------------------------
| **Electronic Clinical Data Infrastructure**                                |                   |                                        |                                  |
| $276 million (25.1% of ARRA-CER Funding)                                  |                   |                                        |                                  |

<table>
<thead>
<tr>
<th>AHRQ Awards to Build Infrastructure Using Prospective Electronic Clinical Data for CER, PCOR, and QI</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>$100 million (9% of ARRA-CER Funding)</strong></td>
<td></td>
</tr>
<tr>
<td><strong>PROSPECT</strong></td>
<td></td>
</tr>
<tr>
<td>Enhanced Registries for QI &amp; CER</td>
<td></td>
</tr>
<tr>
<td>Scalable Distributed Research Networks</td>
<td></td>
</tr>
<tr>
<td>EDM Forum</td>
<td></td>
</tr>
</tbody>
</table>
Common Objectives Across Projects

- 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 (stakeholder engagement)
- Sustainability
National Distribution of CER Infrastructure Projects

- Networks include between 12,000 and 7.5 million patients each; more than 21 million in total
- 38 CER studies are underway or to be conducted
- Address most of AHRQ’s priority populations and conditions
Electronic data infrastructure: 11 HMORN sites
- Largest civilian diabetes registry (about 1.1 million patients; > 400,000 incident cases, longitudinal data from 2001-11)

CER: Interventions to reduce primary non-adherence to drugs in diabetes patients; effectiveness of strategies in gestational DM

“Rethinking Adherence” (by John Steiner)
- Adherence is cluster of behaviors, not one construct
- Physician-patient counseling inadequate
- Coordinated system-wide intervention needed

CER: RCT evaluating oral appliance therapy vs. PAP in obese, hypertensive patients with sleep apnea

Outcomes: blood pressure, CV functioning (imaging), CV genomic/biomarkers, PRO
Indiana PROSPECT

- Builds on Indiana Network of Patient Care: Nation’s largest HIE
- CER: RCT of 3 drugs for Alzheimer’s
- Electronic tools to collect patient-centered outcomes from patients and care givers
- Real-time patient recruitment through an alert triggered in EHR
- Also analyzing genetic information
Automated Study Recruitment
WICER: Columbia U., NYC

- Integrate survey data from Washington Heights/Inwood community in NYC with data from inpatient, outpatient, home care, and long-term care to create a research data warehouse

- CER: Diagnosis & mgmt. of high blood pressure

- Use of mobile devices (iPad) to collect data

- Baseline survey of 5,500 members shows:
  - More than >80% are Latino, 51% are immigrants, 1/3 have 8th grade education or less
  - 1/3 have been told they have high blood pressure
  - Significant levels of inadequate health literacy, poor nutrition, and low physical activity
CER Hub: KPNW, OR

- Building customizable standardized clinical data processors (MediClass)
- Six sites: 3 within HMORN, 3 external (VA, Baylor, OCHIN)
- CER: Asthma and smoking cessation
Pediatric IBD Registry: Cincinnati, OH

- Network of pediatric care sites in 26 states that take care of more than 20% of the nation’s IBD patients

- QI registry (improveCareNow) shares best practices across sites; now automates data collection and can also conduct CER

- PRO data used for pre-visit planning, clarifies changes in patient’s disease

- CER: effectiveness of alternative treatment strategies, focus on biologics
“Data In Once” for QI: Pediatric Enhanced Registry

- EHR-integrated SmartForm that records patient symptoms
- Captures data directly from EHRs to achieve ‘data-in-once’ for QI, CER, pre-visit planning
ESCOAP-CERTAIN: Washington State Registry

- Enhancement of a Washington state-wide surgical QI program; now performing CER and QI on PAD
- Automate and enrich data collection
- Patient engagement (Patient Voices Project)
  - Collaborate with patients and caregivers
  - Collect PRO to complement clinical data for CER
  - Use PRO at POC to guide clinical decision-making
- Develop and encode a flexible policy model
  - In-depth policy review and broad stakeholder engagement to clarify policies

- Proof-of-concept CER studies:
  1. Medication surveillance – to study safety of antiplatelets (esp. prasugrel) and of anticoagulants (esp. dabigatran)
  2. Medication therapy management – co-management by physicians and pharmacists vs. usual care, for patients with diabetes or with high blood pressure
SPAN: Hub and Spoke Design
Types of Data Queries

- **Menu-driven Query Builder**
  - Create a menu-driven user interface to conduct simple queries against multi-site data for CER
  - Returns aggregate data in encrypted files
  - Preliminary analyses, power analyses, prep to research

- **SAS Queries**
  - Distribute free-form SAS to participating sites
  - Returns limited datasets in encrypted files
Challenges: Governance and Sustainability

- Data governance: who has access, for what purpose?
- IRB: QI or research?
- Variation across IRBs: federated or central?
- Network governance & sustainability: right, relevant and timely answers (meaningfully engage stakeholders), build capacity, need multiple sources of funding, manage conflicts of interest
- Legal, regulatory and proprietary issues need to be addressed; not just patient safety
- Trust, credibility, and usefulness are critical
- Sustainability example: DARTNet & PHQ-9
Challenges: CER

- Integrate information across diverse databases and delivery sites to capture complete patient experience
  - genomics and diagnostic data are a big challenge
- Improve approaches to reduce bias and confounding in observational studies
- Create better, useful PRO tools
- Balance inherent tension between internal validity and generalizability
- Identify and account for patient-level, clinician-level and system-level variations that affect patient outcomes
Challenges: Informatics

- Link diverse databases: standardization, assess and assure data quality
- Validate the new electronic tools used to collect and analyze data
- Communicate data securely using mobile devices
- Meet different information needs of clinicians, patients, and researchers (volume and type of information, impact on clinical workflow)
- Improve common data models
- Clarify, harmonize and encode policies to enable distributed research
Electronic Data Methods (EDM) Forum

- Convenes investigators and other stakeholders to understand and tackle challenges in building electronic data infrastructure

- Advance the national dialog and understanding in four domains: CER analytic methods, clinical informatics, governance, and learning healthcare system
EDM Forum Activities

- Focus on topics relevant to electronic clinical data, work guided by a steering committee
- Convenes workshops and symposia to engage scientists and diverse group of stakeholders
- Products: websites, special supplement in *Medical Care*, other journal articles, *CER Project Profiles* report, webinars, issue briefs
eGEMs: Electronic publication
Generating Evidence and Methods (to improve patient outcomes)

- New peer-reviewed electronic publication for useful, timely, focused information (not typically found in scientific journals)
- Focus on “journey” and results, facilitate bi-directional dialogue, broad audience
- Freely-accessible, diverse products:
  - Papers (methods perspectives, best practices, editorials, commentaries)
  - Visualizations
  - Other media (videos, program code)
Engage with the EDM Forum

- Visit the EDM Forum website at www.edm-forum.org for:
  - Announcements of upcoming events and relevant news;
  - New papers and presentations from the PROSPECT, DRN, and Enhanced Registry projects
- Sign up to receive updates on EDM Forum activities (send an email to edmforum@academyhealth.org) and follow the EDM Forum on Twitter, @edm_ah
- Register to participate in upcoming events, including stakeholder symposia and webinars
- Contribute comments on opportunities, challenges, and areas for the EDM Forum to address, including feedback on EDM Forum papers and special supplements
Questions?