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Estimating Patient-Centered and Community-Centered Treatment Effects: Examples from Medical Care and Public Health

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Estimating Patient-Centered and Community-Centered Treatment Effects:

Examples from Medical Care and Public Health

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Acknowledgement



The Public Health Services & Systems Research Program and the Public Health Practice-Based Research Networks Program are national programs of the Robert Wood Johnson Foundation.

Funding for this research was provided by the Robert Wood Johnson Foundation

Questions of interest

- Do the effects of interventions vary across patient and community subgroups based on health needs, vulnerabilities and risks?
- How can we estimate treatment heterogeneity at the level of the individual patient or community?
- Can we achieve larger and more equitable impacts with this knowledge, e.g. through enhanced targeting and tailoring of interventions?
 - **Precision medicine**
 - **Precision public health**

Instrumental variables: a review

- IVs influence treatment choices/exposures but are independent of factors that determine outcomes
- IVs serve as natural randomizers: they approximate RCTs with observational studies
- IVs can be used to estimate causal treatment effects while accounting for both observed and hidden confounding and selection bias

IVs: a classic example



Analysis of Observational Studies in the Presence of Treatment Selection Bias Effects of Invasive Cardiac Management on AMI Survival Using Propensity Score and Instrumental Variable Methods

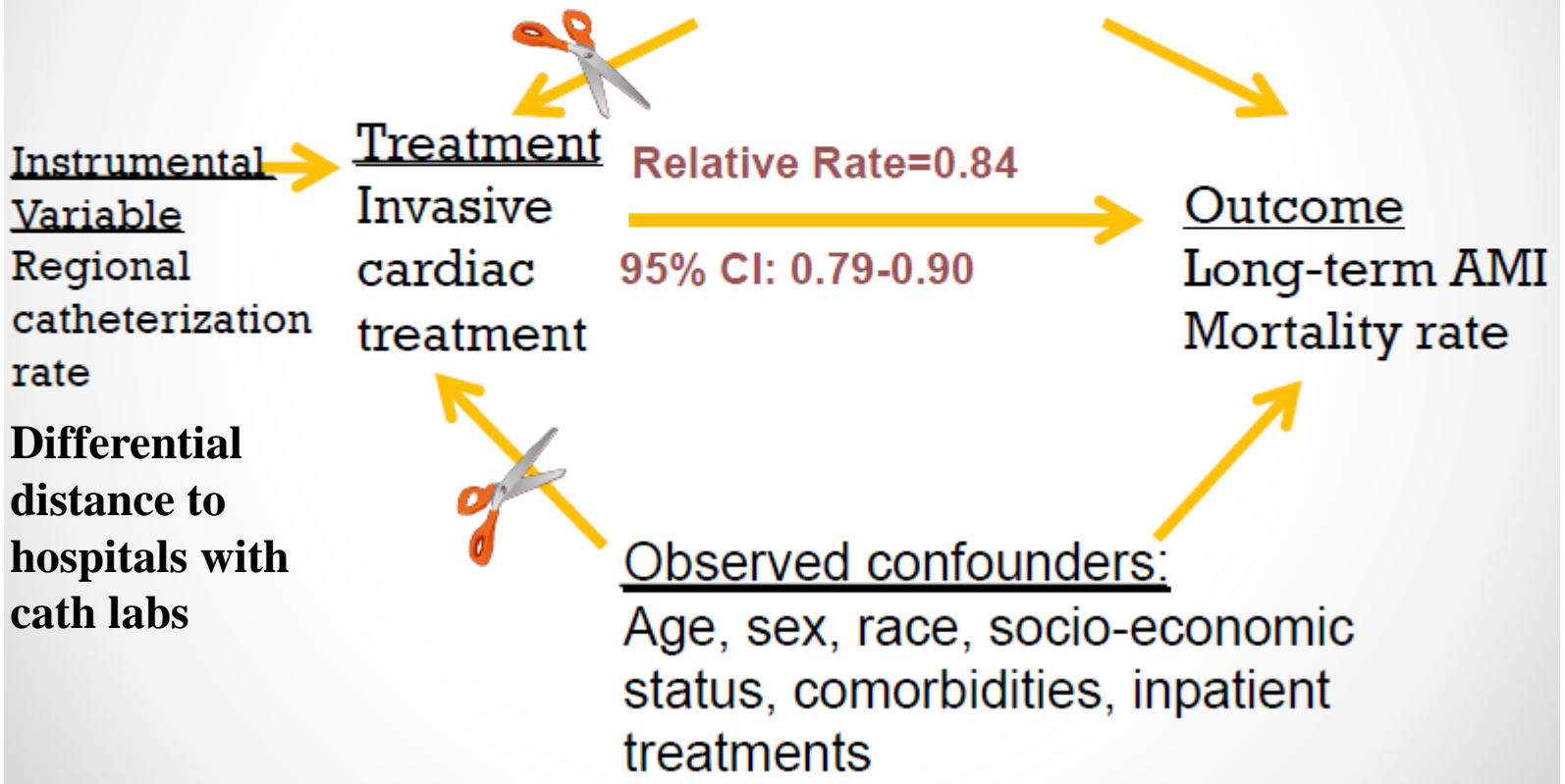
Thérèse A. Stukol, PhD
Elliott S. Fisher, MD, MPH
David E. Wennberg, MD, MPH
David A. Alter, MD, PhD
Daniel J. Gottlieb, MS
Marian J. Vermeulen, MHS

Context Comparisons of outcomes between patients treated and untreated in observational studies may be biased due to differences in patient prognosis between groups, often because of unobserved treatment selection biases.

Objective To compare 4 analytic methods for removing the effects of selection bias in observational studies: multivariable model risk adjustment, propensity score risk adjustment, propensity-based matching, and instrumental variable analysis.

Design, Setting, and Patients A national cohort of 122 124 patients who were elderly (aged 65-84 years), enrolled in Medicare, and hospitalized with acute myocardial in-

Unobserved confounder:
Treatment selection of lower-risk patients



Treatment effect heterogeneity: fundamental empirical questions



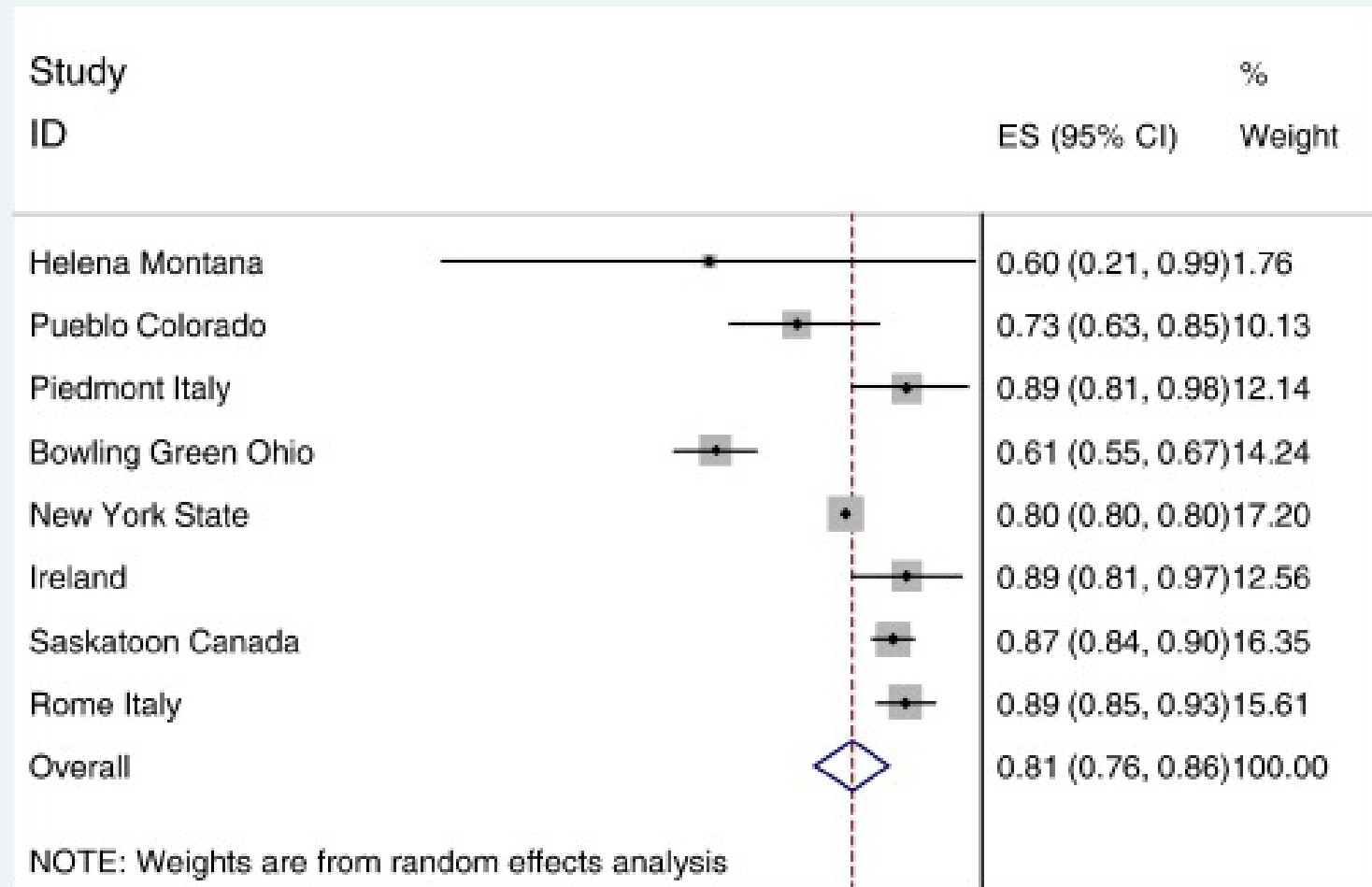
- Which programs, interventions, policies, strategies (*mechanisms*)....
- Work best (*outcomes*)...
- In which institutional & community settings (*contexts*)...
- For whom (*populations and subgroups*)?

Treatment effect heterogeneity

- Biological, behavioral, or structural mechanisms
- Average treatment effect from an RCT may not match the causal treatment effect found in observational data
- Average treatment effect may have little clinical utility and policy significance
- IV estimates may be difficult to interpret in the presence of treatment effect heterogeneity

Variations in policy design, implementation, enforcement

Estimated Effects of Smoke-free Policies on AMI admissions



Treatment effect heterogeneity: estimation problems

- Treatment effects may vary over **unobserved confounders**
- “Essential heterogeneity”
- IV estimates may vary with specific IVs used
- **Solution:** *local* IV methods to estimate marginal treatment effects (Heckman 1999, 2006)

Person-centered treatment effect estimation

- Treatment effects vary across patients based on factors observed by decision-makers
- Treatment is “sorted” across patients based in part on differential potential benefit
 - No single treatment effect
 - Average treatment effects vary across patient subgroups based on chosen treatment levels

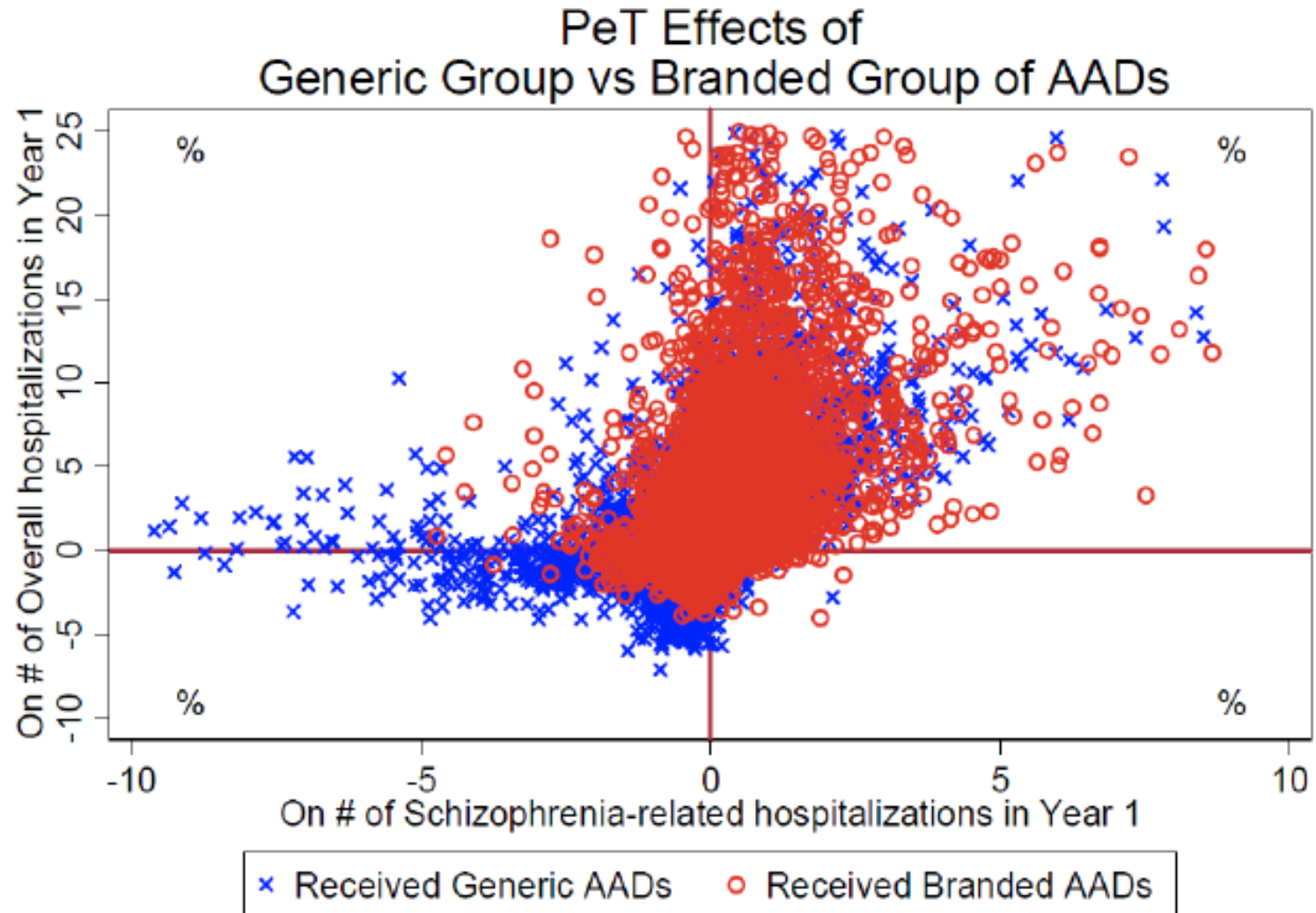
Person-centered treatment effect estimation

- PCTE is a conditional treatment effect that conditions on observed risk factors AND averages over the conditional distribution of unobserved risk factors, conditional on treatment choices
- Identifies individual-level treatment effect heterogeneity better than other methods
- Superior at identifying/controlling for self-selection
- Requires IVs to isolate distribution of unobserved risk factors

Heckman et al. 2006; Basu et al. 2007

Person-centered treatment effect estimation

Revisiting the CATIE Trial Results



Person-centered treatment effect estimation

Revisiting the CATIE Trial Results

Scenario	Average annual number of hospitalizations (95% CI)	% change from Status-quo	p-value
Status-quo	1.83 (1.81 – 1.85)	-	-
All patients started on branded group of AADs	1.73 (1.59 – 1.87)	-5.5	0.15
All patients started on generic group of AADs	2.07 (1.91 – 2.23)	13.1	0.001
All patients started on optimal predicted therapy	1.32 (1.26 – 1.40)	-27.9	<0.001

Notes: P-values reflect comparisons of average annual number of hospitalizations under various scenarios to status quo.

**Does treatment
heterogeneity extend to
public health services
at the community-level?**

Research questions of interest

- Which organizations contribute to the implementation of public health activities in local communities?
- How do these contributions change over time?
Recession, recovery, ACA implementation?
- What are the health and economic effects of these activities?
 - Heterogeneity by population and delivery system characteristics?

Data: public health production

National Longitudinal Survey of Public Health Systems

- Cohort of 360 communities with at least 100,000 residents
- Followed over time: 1998, 2006, 2012, 2014**
- Local public health officials report:
 - **Scope**: availability of 20 recommended public health activities
 - **Network**: types of organizations contributing to each activity
 - **Effort**: contributed by designated local public health agency
 - **Quality**: perceived effectiveness of each activity

** Stratified sample of 500 communities < 100,000 added in 2014 wave

Cluster and network analysis to identify “system capital”

Cluster analysis is used to classify communities into one of 7 categories of **public health system capital** based on:

- **Scope of activities** contributed by each type of organization
- **Density of connections** among organizations jointly producing public health activities
- **Degree centrality** of the local public health agency

Mays GP et al. Understanding the organization of public health delivery systems: an empirical typology. *Milbank Q.* 2010;88(1):81–111.

Estimating network effects

Dependent variables:

- **Quantity:** Percent of recommended public health activities performed in the community
- **Quality:** Perceived effectiveness of activities
- **Resource use:** Local governmental expenditures for public health activities
- **Health outcomes:** premature mortality(<75), infant mortality, death rates for heart disease, diabetes, cancer, influenza

Independent variables:

- **Contribution scores:** percent of activities contributed by each type of organization
- **Network characteristics:** network density, organizational degree centrality, betweenness centrality

Estimating network effects

Estimation:

- Log-transformed Generalized Linear Latent and Mixed Models
- Account for repeated measures and clustering of public health jurisdictions within states
- Instrumental variables address endogeneity of network structures

$$\text{Ln}(\text{Network}_{z,ijt}) = \sum \alpha_z \text{Governance}_{ijt} + \beta_1 \text{Agency}_{ijt} + \beta_2 \text{Community}_{ijt} + \mu_j + \varphi_t + \varepsilon_{ijt}$$

$$\text{Ln}(\text{Quantity/Quality/Cost}_{ijt}) = \sum \alpha_z \text{Ln}(\hat{\text{Network}}_z)_{ijt} + \beta_1 \text{Agency}_{ijt} + \beta_2 \text{Community}_{ijt} + \mu_j + \varphi_t + \varepsilon_{ijt}$$

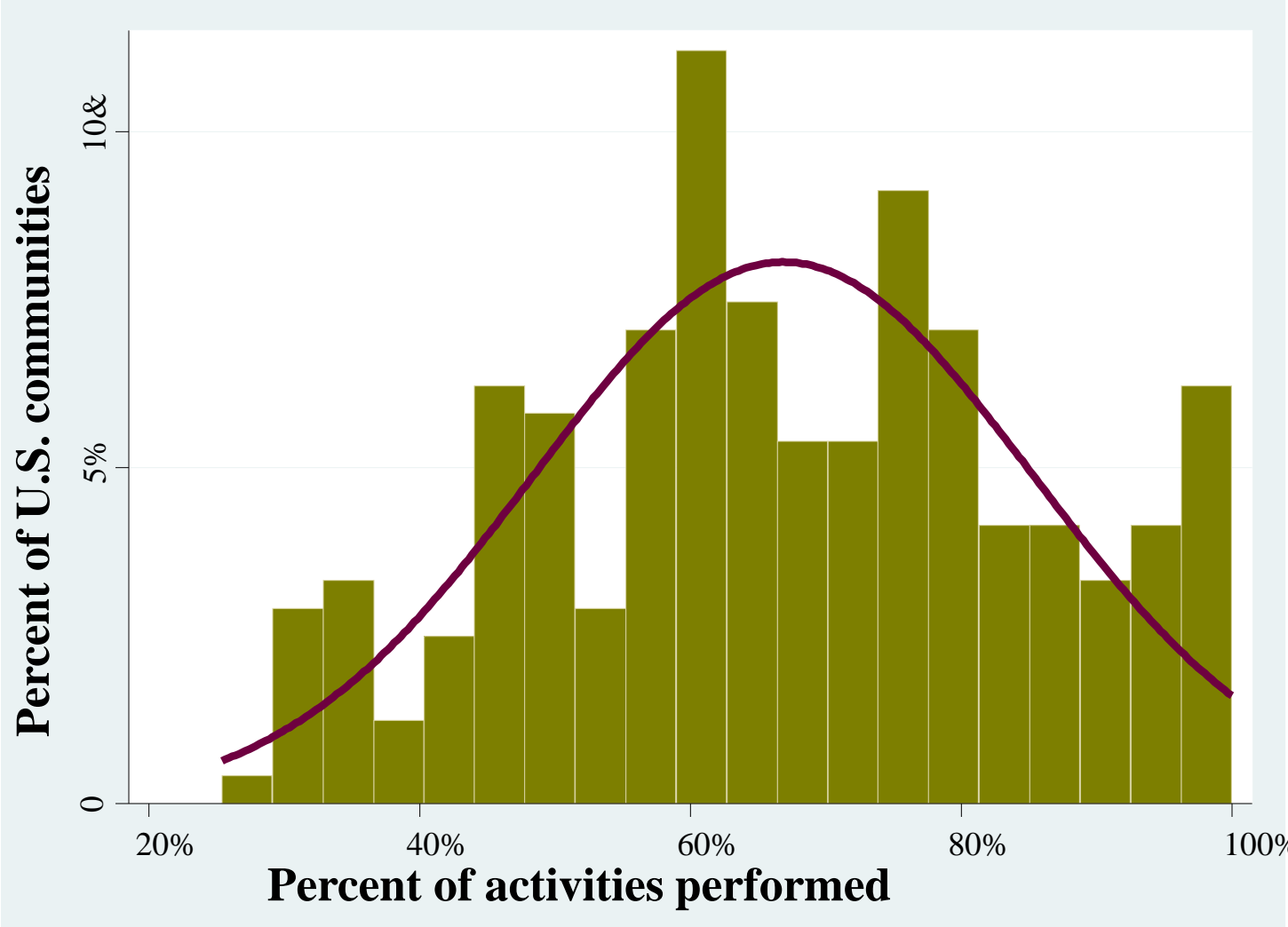
All models control for type of jurisdiction, population size and density, metropolitan area designation, income per capita, unemployment, racial composition, age distribution, educational attainment, and physician availability.

Delivery of recommended public health activities, 1998-2014

Public Health Activity	1998	2014	% Change
1 Community health needs assessment	71.5%	86.0%	20.2%**
2 Behavioral risk factor surveillance	45.8%	70.2%	53.2%**
3 Adverse health events investigation	98.6%	100.0%	1.4%
4 Public health laboratory testing services	96.3%	96.5%	0.2%
5 Analysis of health status and health determinants	61.3%	72.8%	18.7%**
6 Analysis of preventive services utilization	28.4%	39.4%	38.8%**
7 Health information provision to elected officials	80.9%	84.8%	4.8%
8 Health information provision to the public	75.4%	83.8%	11.1%*
9 Health information provision to the media	75.2%	87.5%	16.3%**
10 Prioritization of community health needs	66.1%	82.3%	24.6%**
11 Community participation in health improvement planning	41.5%	67.7%	63.0%**
12 Development of community health improvement plan	81.9%	86.2%	5.2%
13 Resource allocation to implement community health plan	26.2%	43.2%	64.9%**
14 Policy development to implement community health plan	48.6%	57.5%	18.4%*
15 Communication network of health-related organizations	78.8%	84.8%	7.6%
16 Strategies to enhance access to needed health services	75.6%	50.2%	-33.6%**
17 Implementation of legally mandated public health activities	91.4%	92.4%	1.0%
18 Evaluation of public health programs and services	34.7%	38.4%	10.8%**
19 Evaluation of local public health agency capacity/performance	56.3%	55.0%	-2.4%
20 Implementation of quality improvement processes	47.3%	49.6%	5.0%
Composite availability of assessment activities (1-6)	66.7%	77.6%	16.4%**
Composite availability of policy development activities (7-15)	60.2%	72.5%	20.4%
Composite availability of assurance activities (16-20)	64.4%	52.8%	-18.0%*
Composite availability of all activities (1-20)	63.8%	67.6%	6.0%*

Variation in Delivery of Recommended Public Health Services

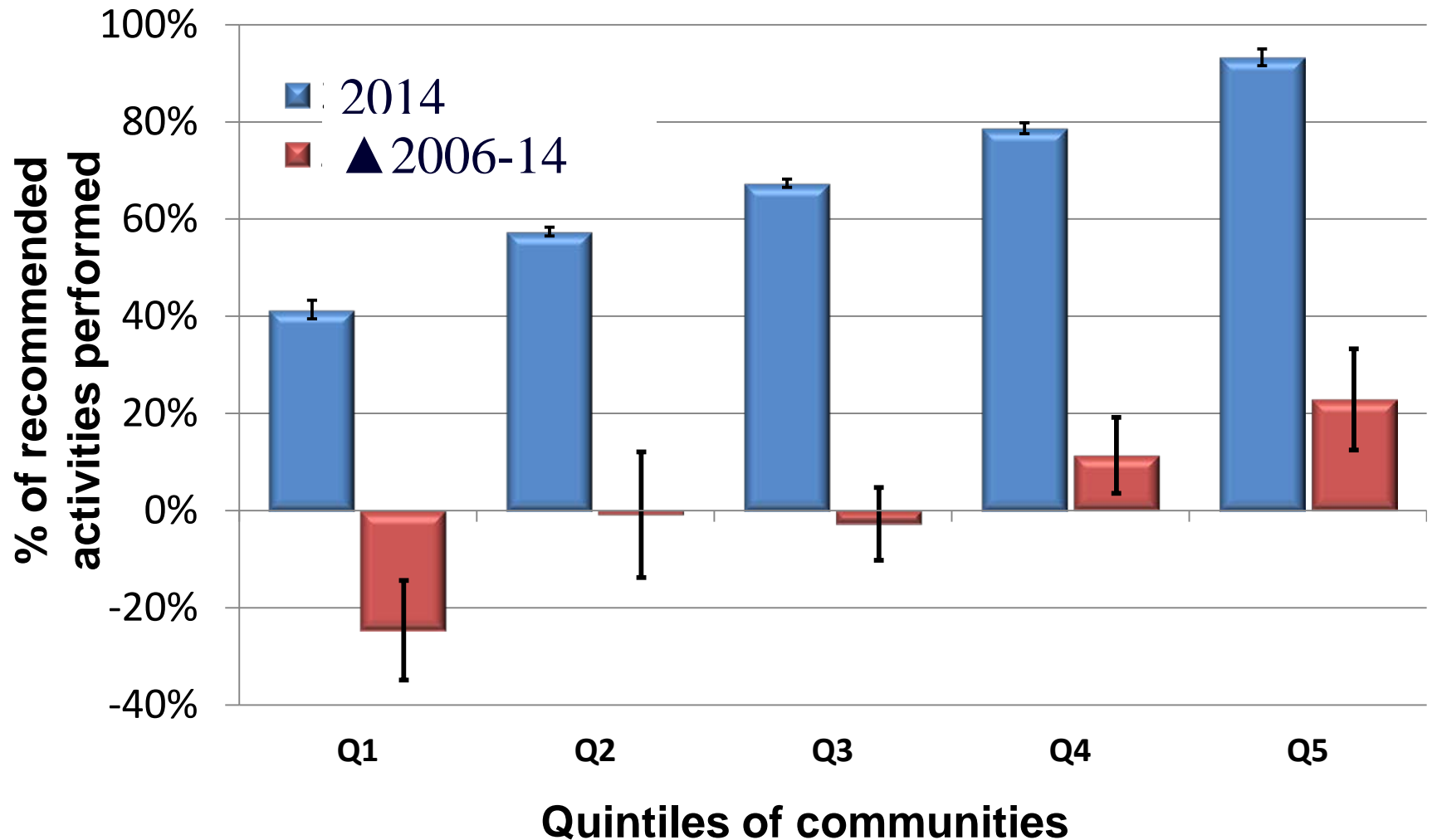
National Longitudinal Survey of Public Health Systems



National Longitudinal Survey of Public Health Systems, 2014

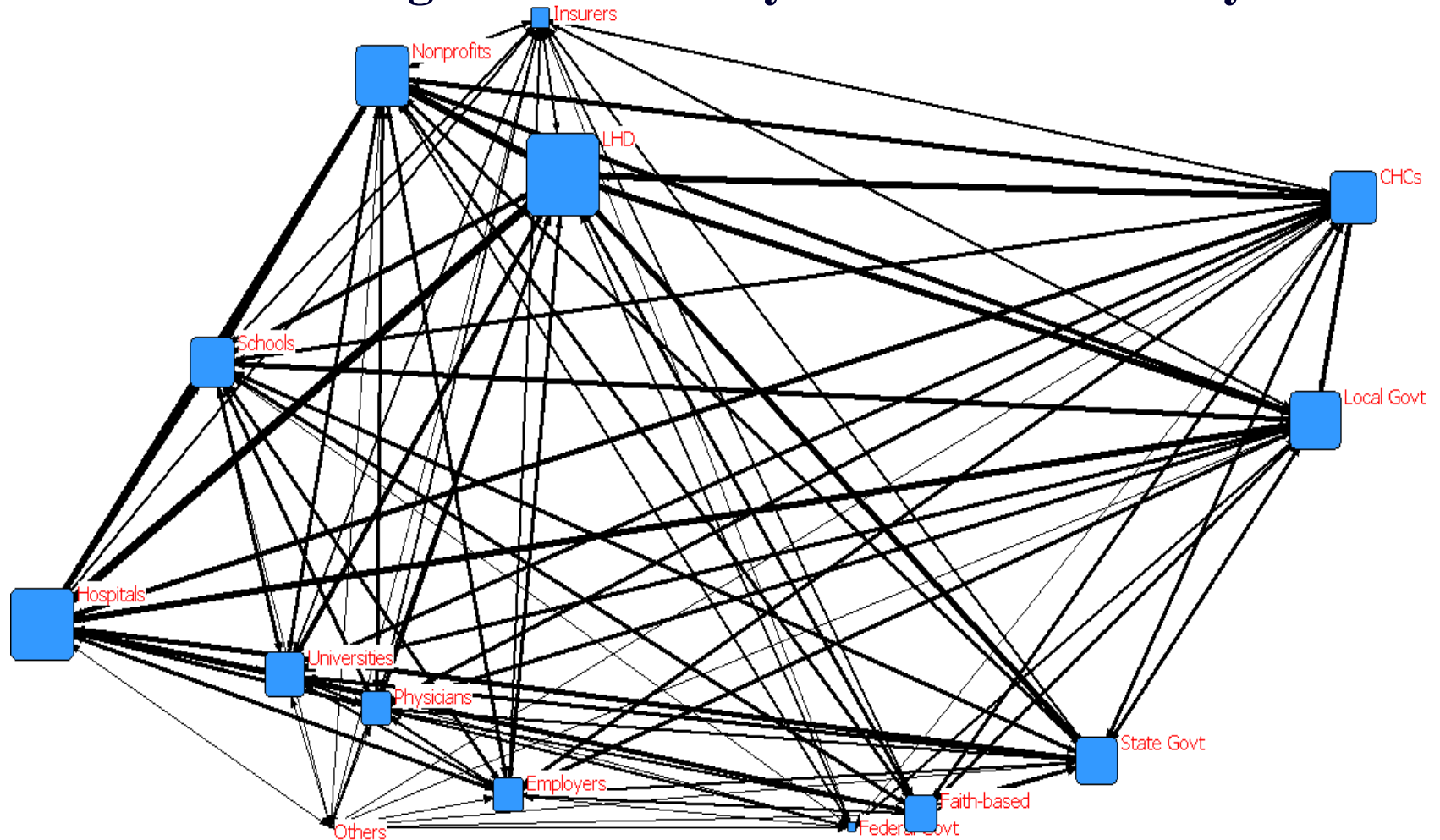
Variation and Change in Delivery

Delivery of recommended public health activities, 2006-14



Delivery System Structures for Public Health Services

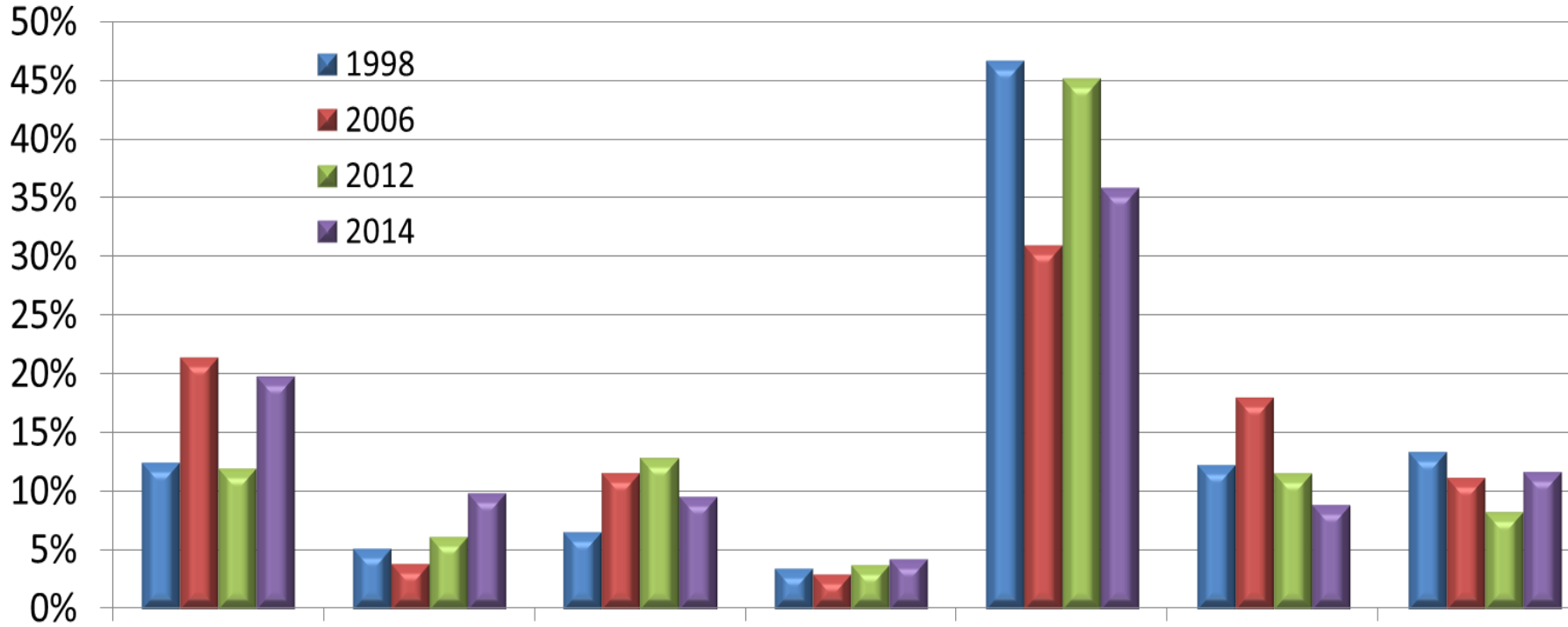
National Longitudinal Survey of Public Health Systems



Node size = centrality

Line size = % activities jointly contributed (tie strength)

Prevalence of Public Health System Configurations, 1998-2014



Scope
Centrality
Density

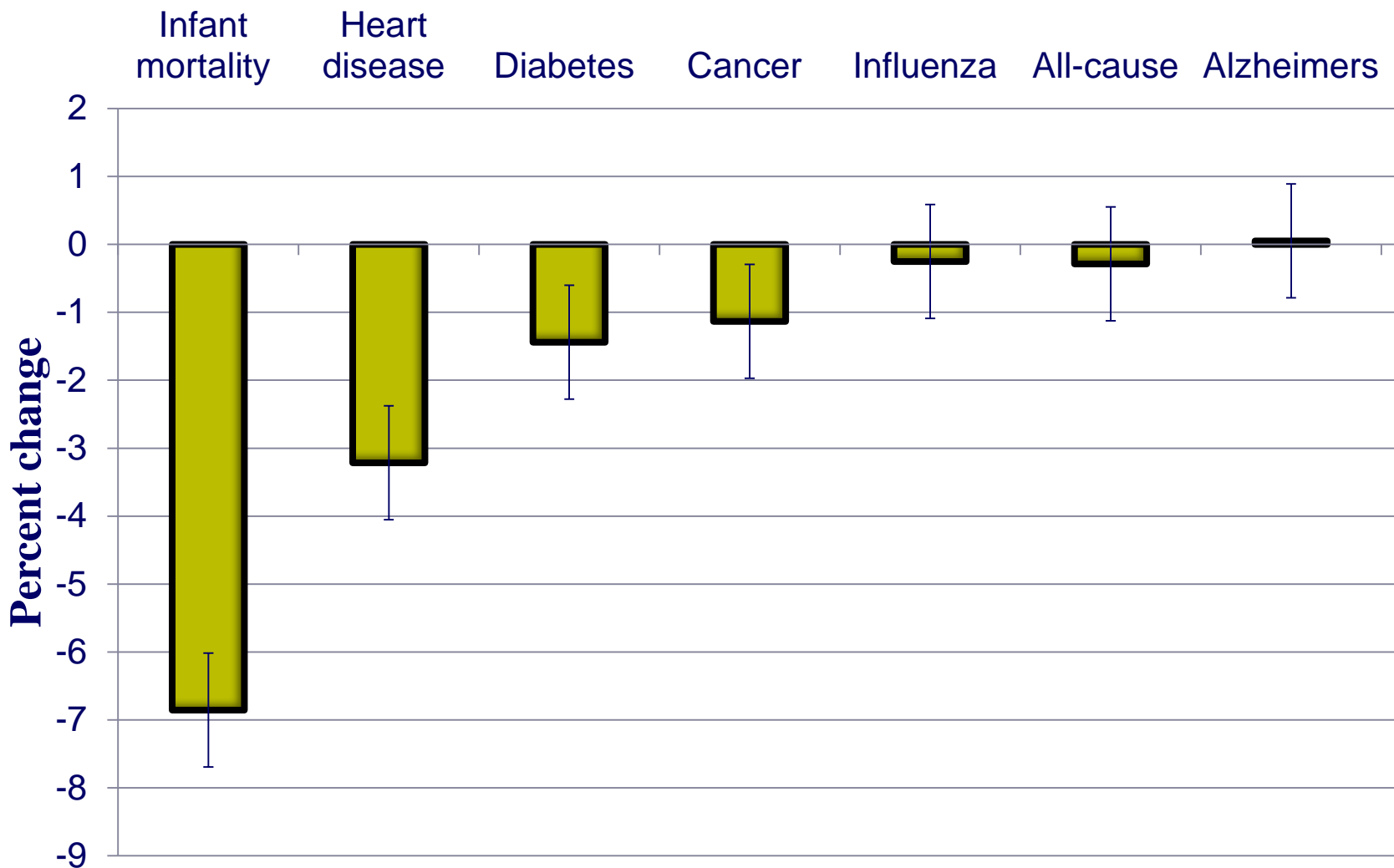
Cluster	1	2	3	4	5	6	7
Scope	High	High	High	Mod	Mod	Low	Low
Centrality	Mod	Low	High	High	Low	High	Low
Density	High	High	Mod	Mod	Mod	Low	Mod

Comprehensive
(High System Capital)

Conventional

Limited

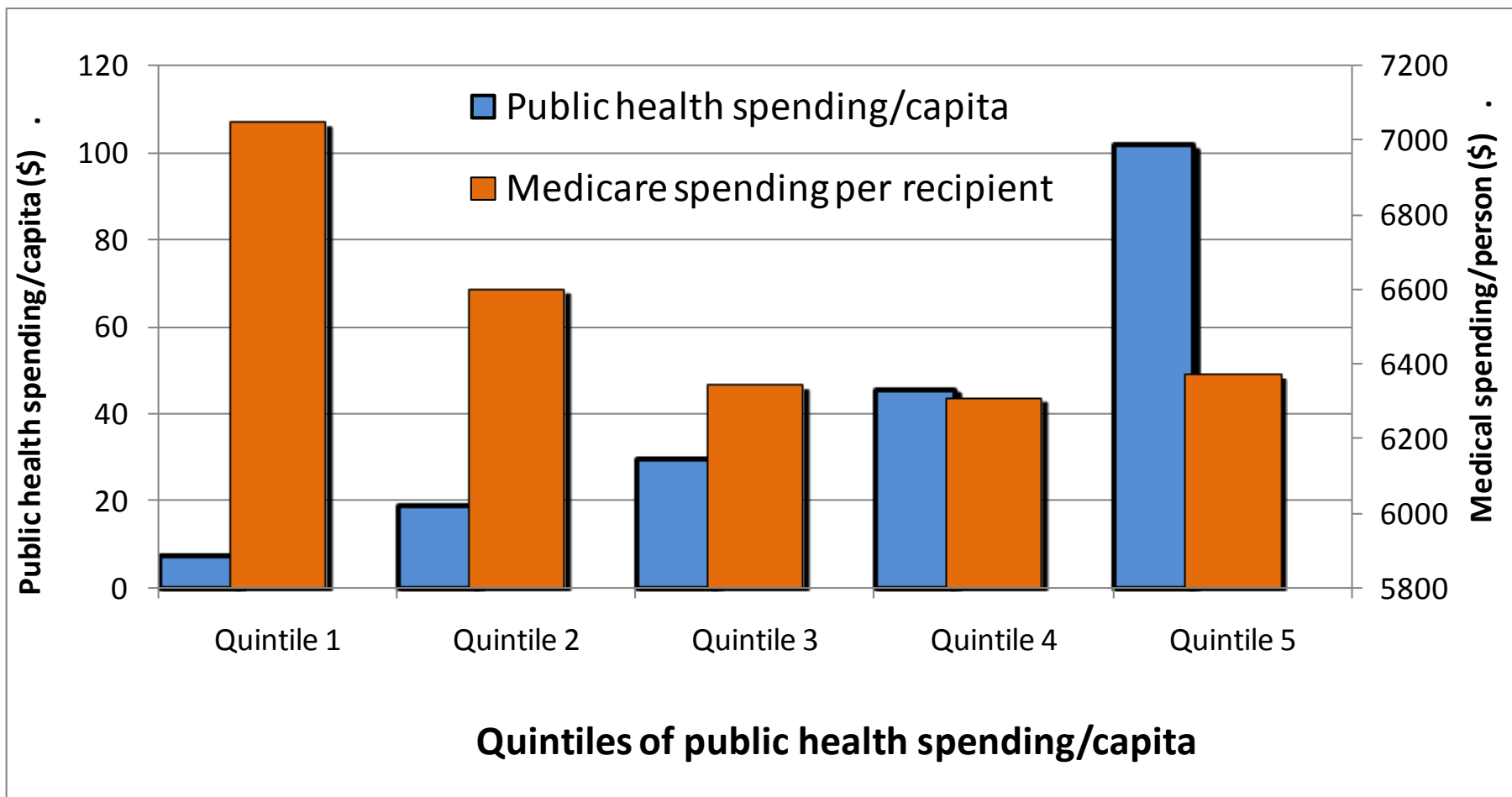
Prior Research: Mortality reductions attributable to local public health spending, 1993-2008



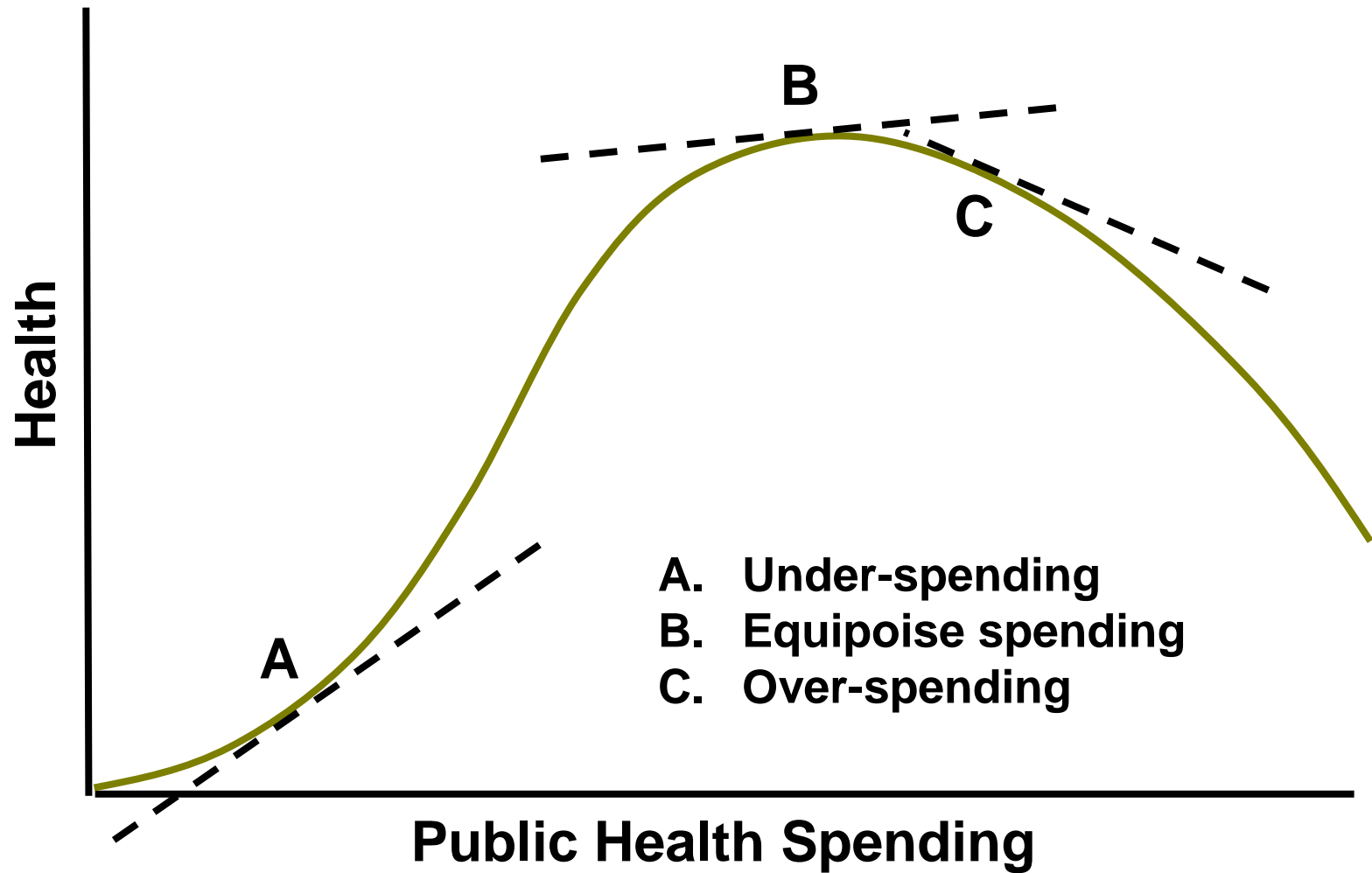
Hierarchical regression estimates with instrumental variables to correct for selection and unmeasured confounding

Prior Research: Medical cost offsets attributable to local public health spending 1993-2008

Offset elasticity = -0.088



Value of an additional dollar in public health



Analytic Approach

- Use the technique of local instrumental variables (LIV) estimation to estimate **community-specific effects** of public health spending
- Compare the health & economic impact of increases public health spending between:
 - Low-income vs. higher-income communities
 - Agencies that deliver broad vs. narrow scope of public health activities

Heckman JJ, Vytlacil EJ. 1999. Local instrumental variables and latent variable models for identifying and bounding treatment effects. *Proceedings of the National Academy of Sciences USA* 96(8): 4730–4734.

Basu A. 2013. Estimating person-centered treatment (PET) effects using instrumental variables. *Journal of Applied Econometrics*, in press.

Local IV Approach

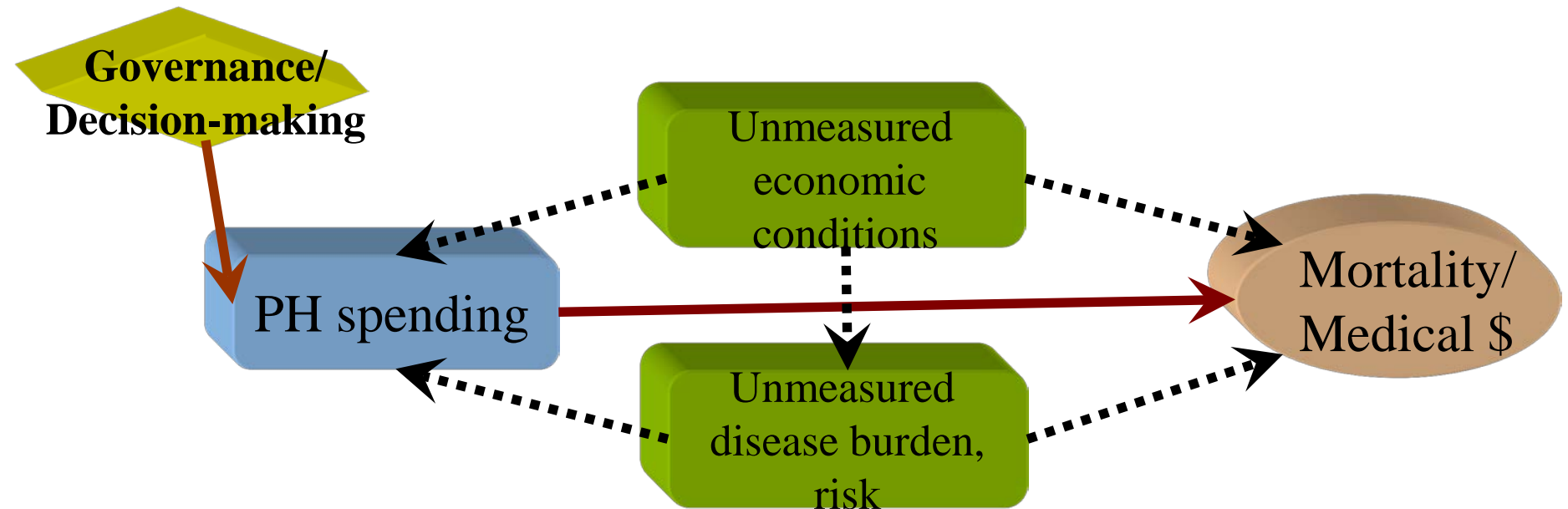
- Estimate predicted spending (P) as a function of all measured covariates (X) and instruments (Z)
- Model outcome (O) as nonlinear function of P(X,Z) and X
- Estimate $\partial O / \partial P$ the effect of a change in predicted spending on the outcome
- Find the distribution of P(X,Z) for the subset of communities of interest
- Estimate the average treatment effect for each subset as the average weighted value of $\partial O / \partial P$ across the subset

Heckman JJ, Vytlacil EJ. 1999. Local instrumental variables and latent variable models for identifying and bounding treatment effects. *Proceedings of the National Academy of Sciences USA* 96(8): 4730–4734.

Basu A. 2013. Estimating person-centered treatment (PET) effects using instrumental variables. *Journal of Applied Econometrics*, in press.

Analytical approach: IV estimation

- ◆ Identify exogenous sources of variation in spending that are unrelated to outcomes
 - Governance structures: local boards of health
 - Decision-making authority: agency, board, local, state
- ◆ Controls for unmeasured factors that jointly influence spending and outcomes



Determinants of Local Public Health Spending Levels: Local IVs

<u>Governance/Decision Authority</u>	<u>Elasticity Coefficient</u>	<u>95% CI</u>
Governed by local board of health	0.131**	(0.061, 0.201)
State hires local PH agency head†	-0.151*	(-0.318, 0.018)
Local board approves local PH budget	0.388***	(0.576, 0.200)
State approves local PH budget†	-0.308**	(-0.162, -0.454)
Local govt sets local PH fees	0.217**	(0.101, 0.334)
Local govt imposes local PH taxes	0.190**	(0.044, 0.337)
Local board can request local PH levy	0.120**	(0.246, 0.007)

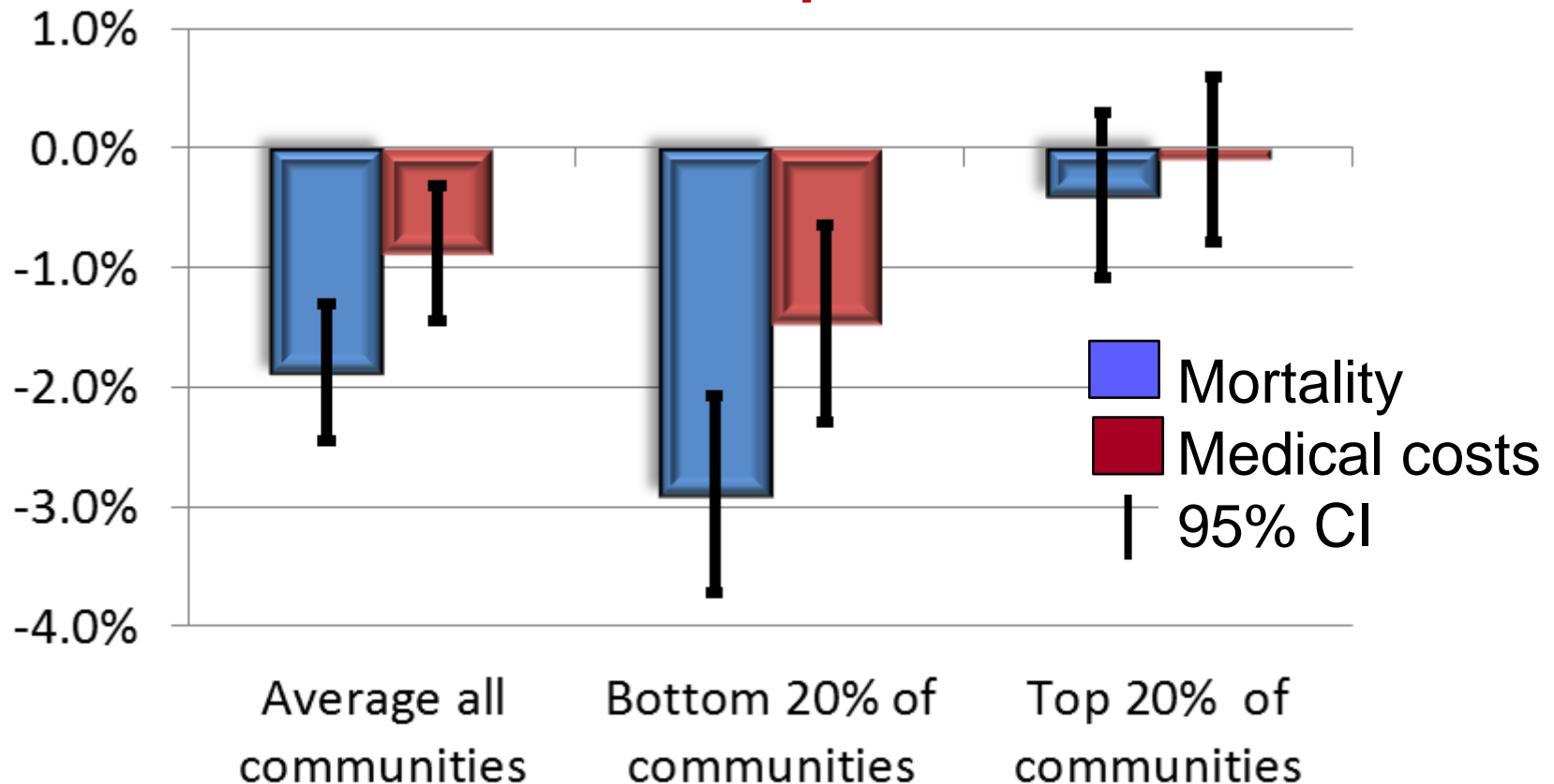
F=16.4 p<0.001

log regression estimates controlling for community-level and state-level characteristics. *p<0.10 **p<0.05 ***p<0.01

†As compared to the local board of health having the authority.

Community-specific estimates of public health spending on heart disease mortality

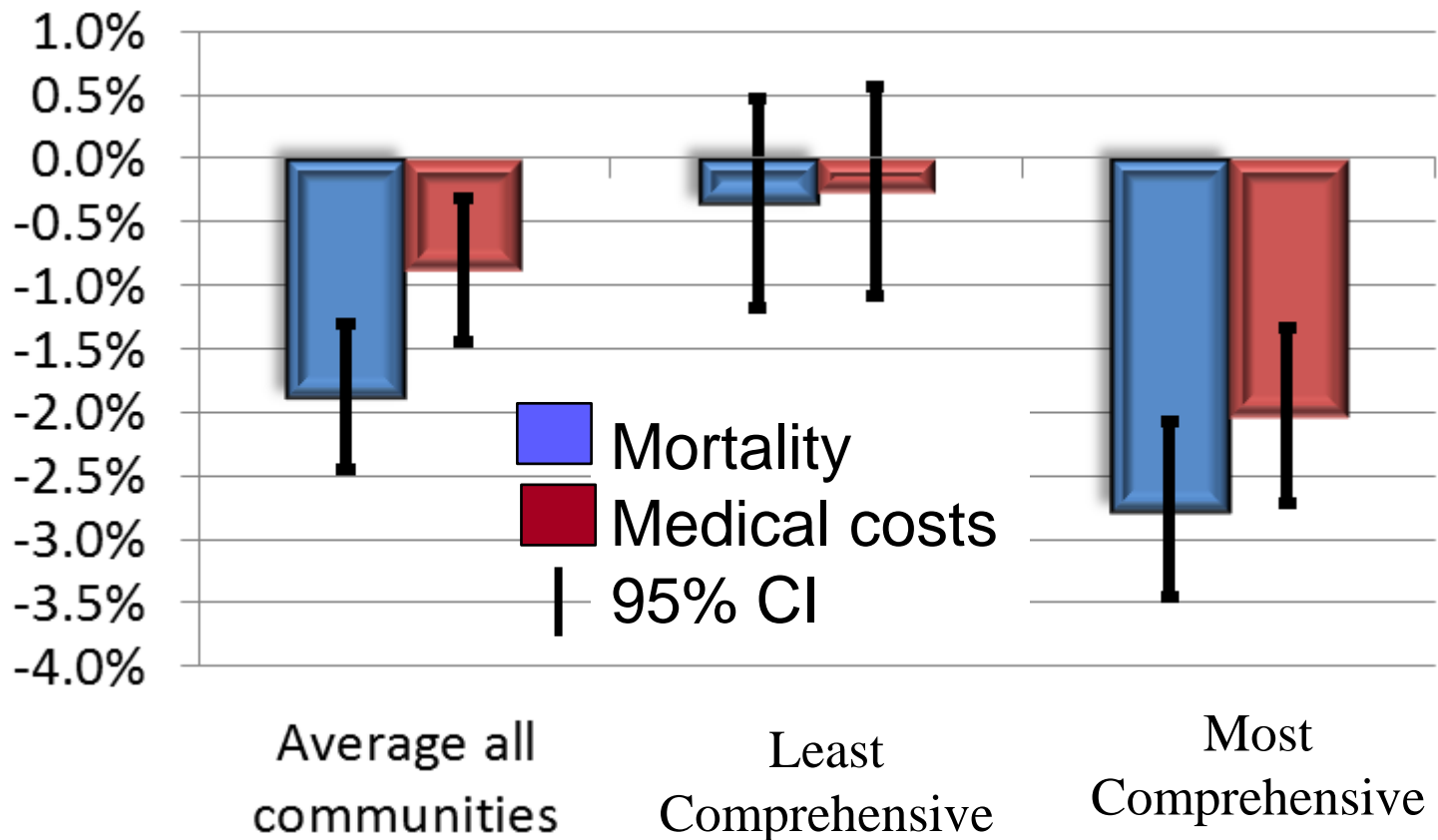
Impact of 10% Increase in Public Health Spending/Capita
Based on Income Per Capita in Communities



Log IV regression estimates controlling for community-level and state-level characteristics

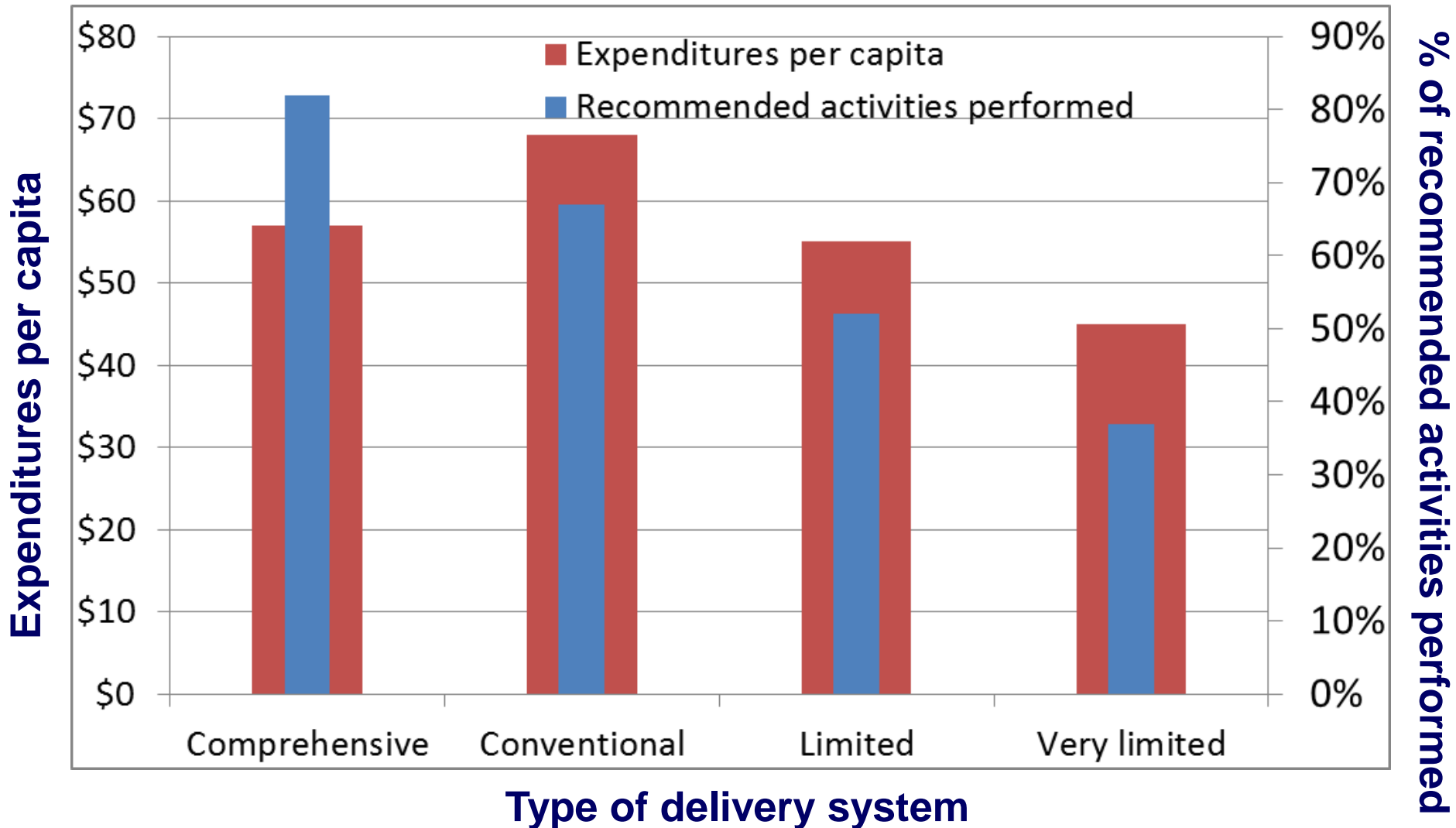
Community-specific estimates of public health spending on heart disease mortality

Impact of 10% Increase in Public Health Spending/Capita
Based on Delivery System Comprehensiveness



Log IV regression estimates controlling for community-level and state-level characteristics

Comprehensive systems do more with less



Conclusions

- Sizable health & economic gains are attributable to local public health expenditures
- Gains are 21-44% larger in low-income communities
- Gains are 17-38% larger for communities with comprehensive delivery systems
- No evidence of over-spending

Implications for policy & practice

Increase the value of public health investments through:

- ***Enhanced targeting***: low-resource, high-need communities
- ***Enhanced infrastructure***: broad scope of core public health activities
 - Accreditation standards
 - Minimum package of services

**Can Patient-Centered
Treatment Estimation
Help to Evaluate
Community-level
Programs?**

Estimating Program ROI

Arkansas Community Connector Program

- Use community health workers & public health infrastructure to identify people with unmet social support needs
- Connect people to home and community-based services & supports
- Link to hospitals and nursing homes for transition planning
- Use Medicaid and SIM financing, savings reinvestment
- Costing with electronic time logs



Felix, Mays et al. 2011

<http://content.healthaffairs.org/content/30/7/1366.abstract>

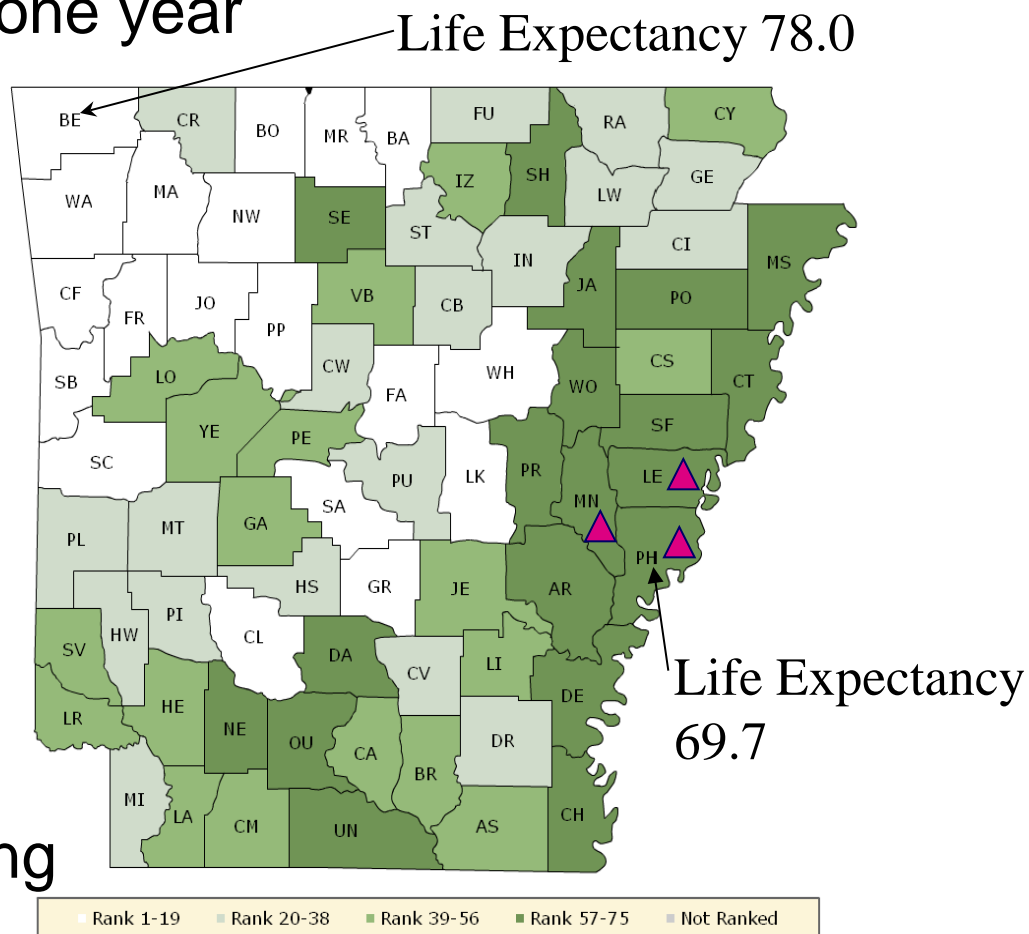
The Community Connector Program (CCP)

- Quasi-experimental research design

- Measured expenditures one year before participation and up to 3 years after participation

- Statistically-matched comparison group of Medicaid recipients not served by CCP

- Difference-in-difference estimates of impact, controlling for time-varying covariates



Estimates of Program Impact

By Holly C. Felix, Glen P. Mays, M. Kathryn Stewart, Naomi Cottoms, and Mary Olson

THE CARE SPAN

**Medicaid Savings Resulted When
Community Health Workers
Matched Those With Needs
To Home And Community Care**

HealthAffairs

Regression-Adjusted, Difference-in-Difference Estimates

PET Spending

Time Period*	Average Spending Change from Baseline	Change for Multi- morbidity patients
Year 1	-6.0%**	-9.6%**
Year 2	-13.4%**	18.2%**
Year 3	-15.3%**	21.4%**

After adjusting for baseline and time-varying differences between groups

*Reference year is one year prior to CCP participation

**p<0.05

Estimated Program ROI

Three Year Aggregate Estimates

➤ Combined Medicaid spending reductions:	\$3.515 M
➤ Program implementation costs:	\$0.896 M
➤ Net savings:	\$2.629 M
➤ ROI:	\$2.92
➤ ROI for multi-morbidity	\$5.17

Felix, Mays et al. 2011

<http://content.healthaffairs.org/content/30/7/1366.abstract>

PCT References

Basu A, Meltzer D. Value of information on preference heterogeneity and individualized care. *Medical Decision Making* 2007; 27(2):112-127.

Basu A, Heckman J, Navarro-Lozano S, et al. Use of instrumental variables in the presence of heterogeneity and self-selection: An application to treatments of breast cancer patients. *Health Econ* 2007; 16(11): 1133 -1157.

Basu A. 2009. Individualization at the heart of comparative effectiveness research: The time for i-CER has come. *Medical Decision Making*, 29(6): N9-N11.

Basu A, Jena AB, Philipson TJ. The impact of comparative effectiveness research on health and health care spending. *J Health Econ*. 2011;30(4):695-706.

Basu A. Economics of individualization in comparative effectiveness research and a basis for a patient-centered health care. *J Health Econ*. 2011; 30(3):549-559.

Basu A. Person-Centered Treatment (PeT) effects using instrumental variables: An application to evaluating prostate cancer treatments. *Journal of Applied Econometrics* (In Press).

Heckman JJ, Vytlacil EJ. Local instrumental variables and latent variable models for identifying and bounding treatment effects. *Proc Nat Acad Sci* 1999; 96(8): 4730-34

Heckman JJ, Urzua S, Vytlacil E. Understanding instrumental variables in models with essential heterogeneity. *Rev Econ Stat* 2006; 88(3): 389-432.

Kaplan S, Billimek J, Sorkin D, Ngo-Metzger Q, Greenfield S. Who Can Respond to Treatment?: Identifying Patient Characteristics Related to Heterogeneity of Treatment Effects. *Medical Care* 2010; 48(6): S9-S16

About us

- Funded by Robert Wood Johnson Foundation:
\$10.5M to UK from 2011-2015
- Intramural research activities
 - **Public Health Value**: Cost estimation, economic evaluation
 - **Delivery System Reform**: ACA effects on public health delivery, population health measurement, aligning public health & health care delivery
- Extramural research programs (funded separately ≈ **\$30M**)
 - **Practice-based Research Networks** (PBRNs) across U.S.
 - Investigator-initiated research awards
 - Predoctoral/Postdoctoral & career development awards
 - Quick Strike rapid-cycle studies

For More Information



Supported by The Robert Wood Johnson Foundation

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