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

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Examples from Medical Care and Public Health

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PUBLIC HEALTH SERVICES & SYSTEMS RESEARCH
PRACTICE-BASED RESEARCH NETWORKS
National Coordinating Center

Acknowledgement

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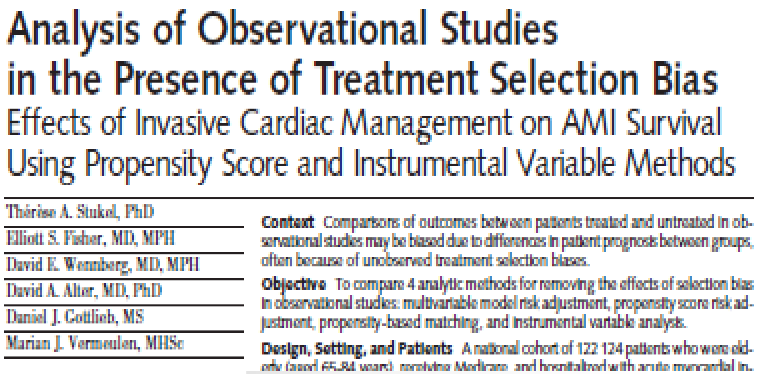
Questions of interest

- 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

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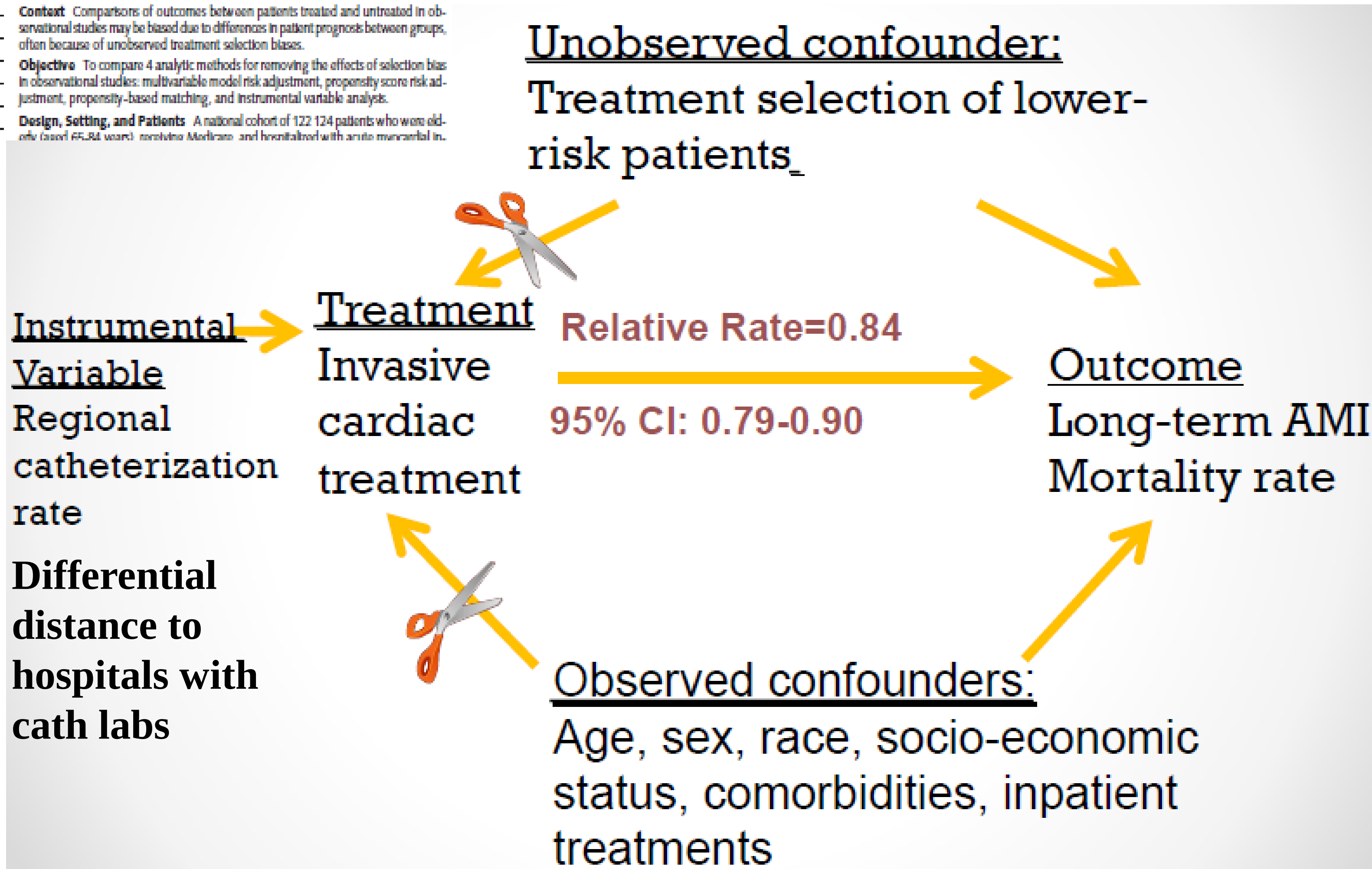
Context Comparisons of outcomes between patients treated and untreated in observational studies may be biased due to differences in patient prognosis between groups, often because of unobserved treatment selection biases.

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 eld-



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***)?

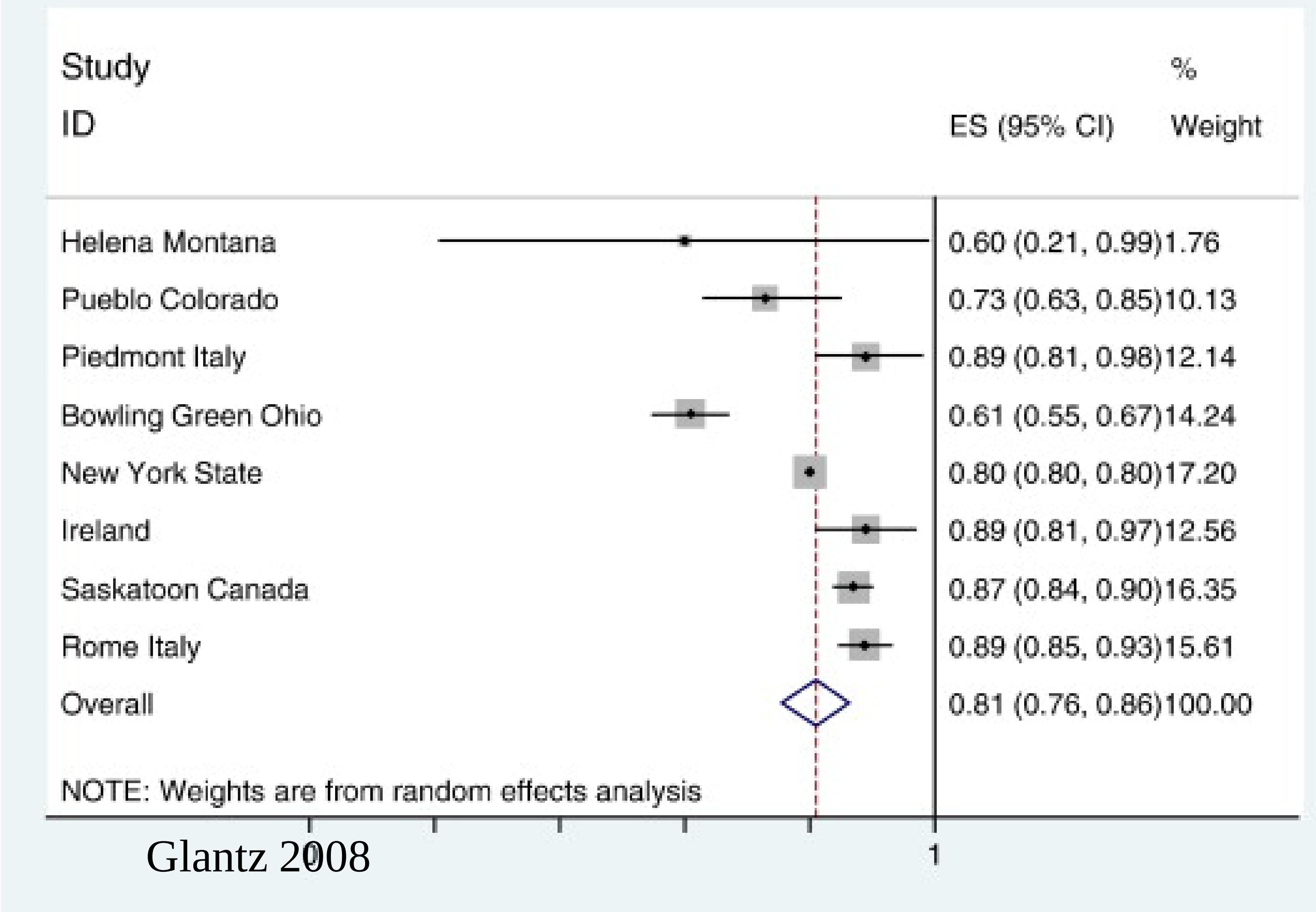
Pawson and Tilley 1997

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



Glantz 2008

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

Heckman et al. 2006; Basu et al 2007

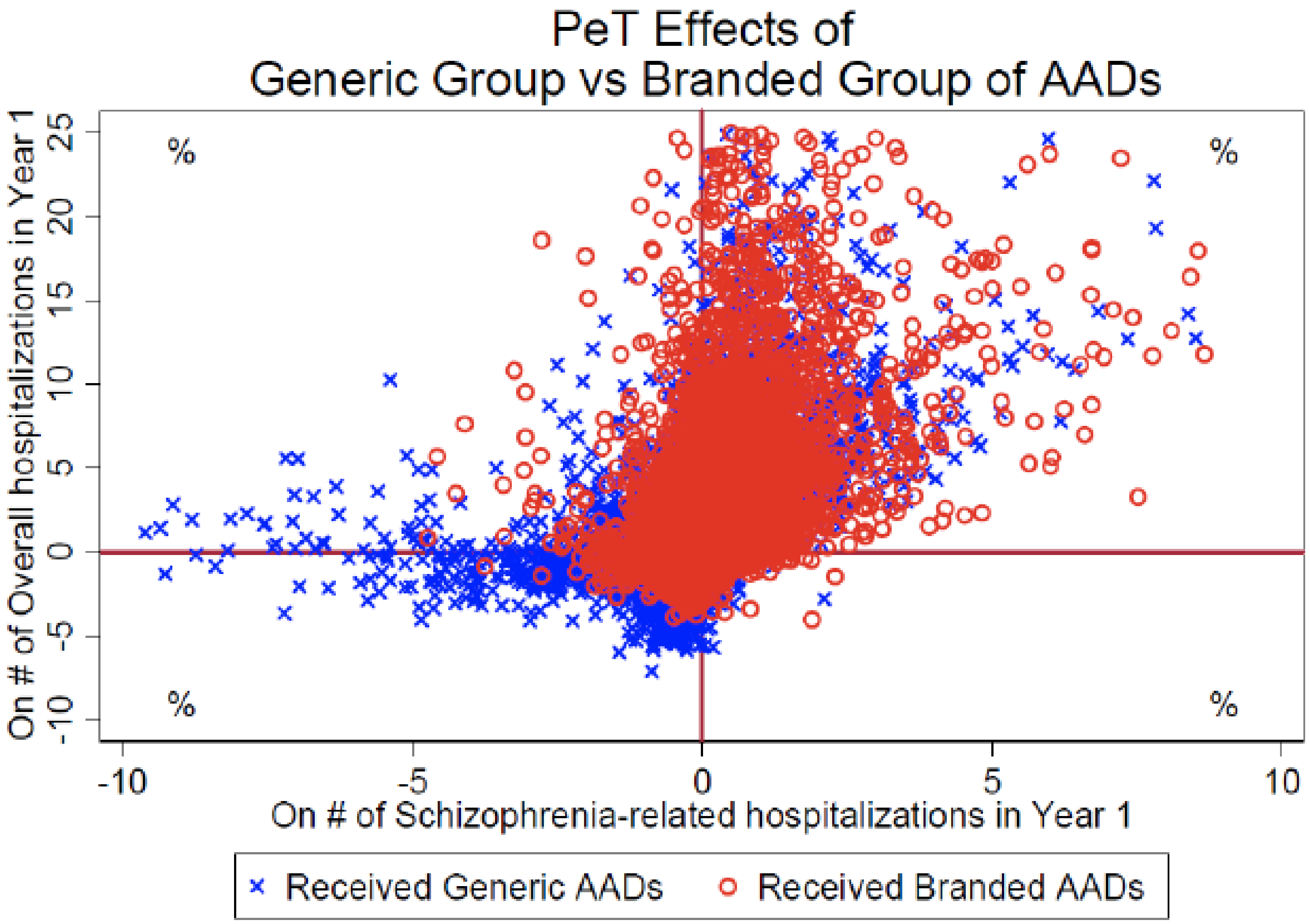
Person-centered treatment effect estimation

- 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



Basu et al. 2013

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.

Basu et al. 2013

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

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

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

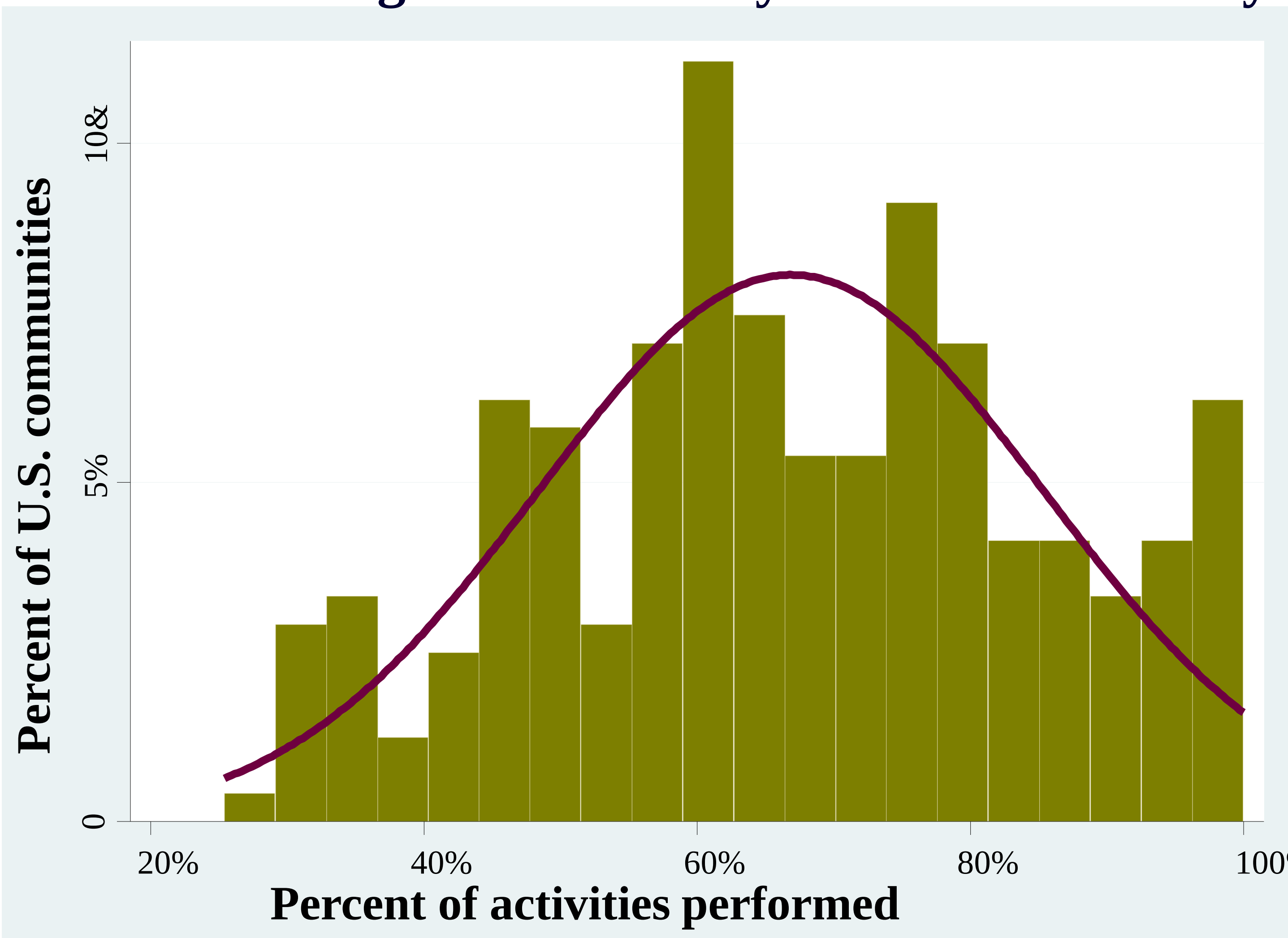
All models control for type of jurisdiction, population size and density, metropolitan area designation, income per capita, unemployment, racial composition, age distribution, educational attainment, and physician availability.

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

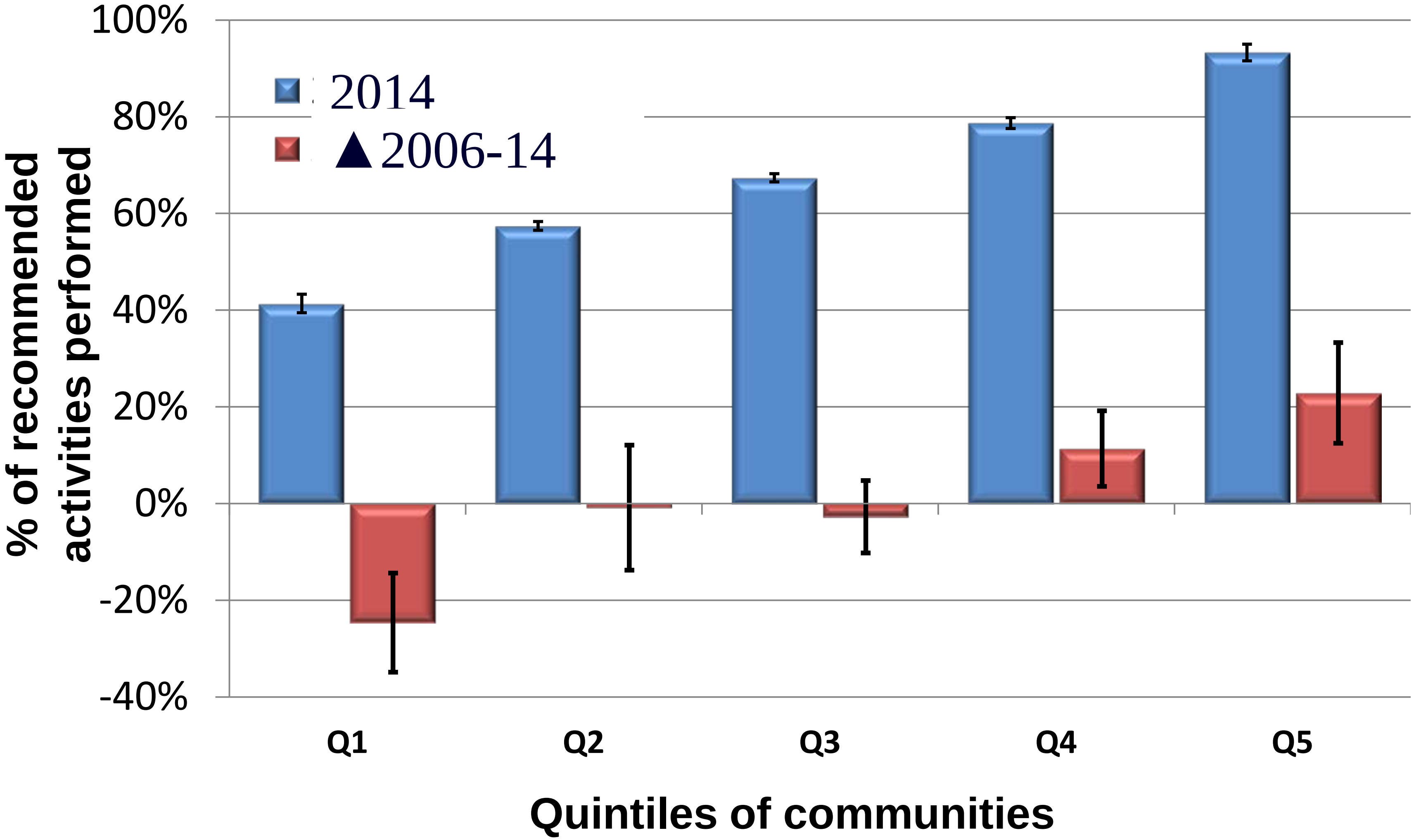
National Longitudinal Survey of Public Health Systems



National Longitudinal Survey of Public Health Systems, 2014

Variation and Change in Delivery

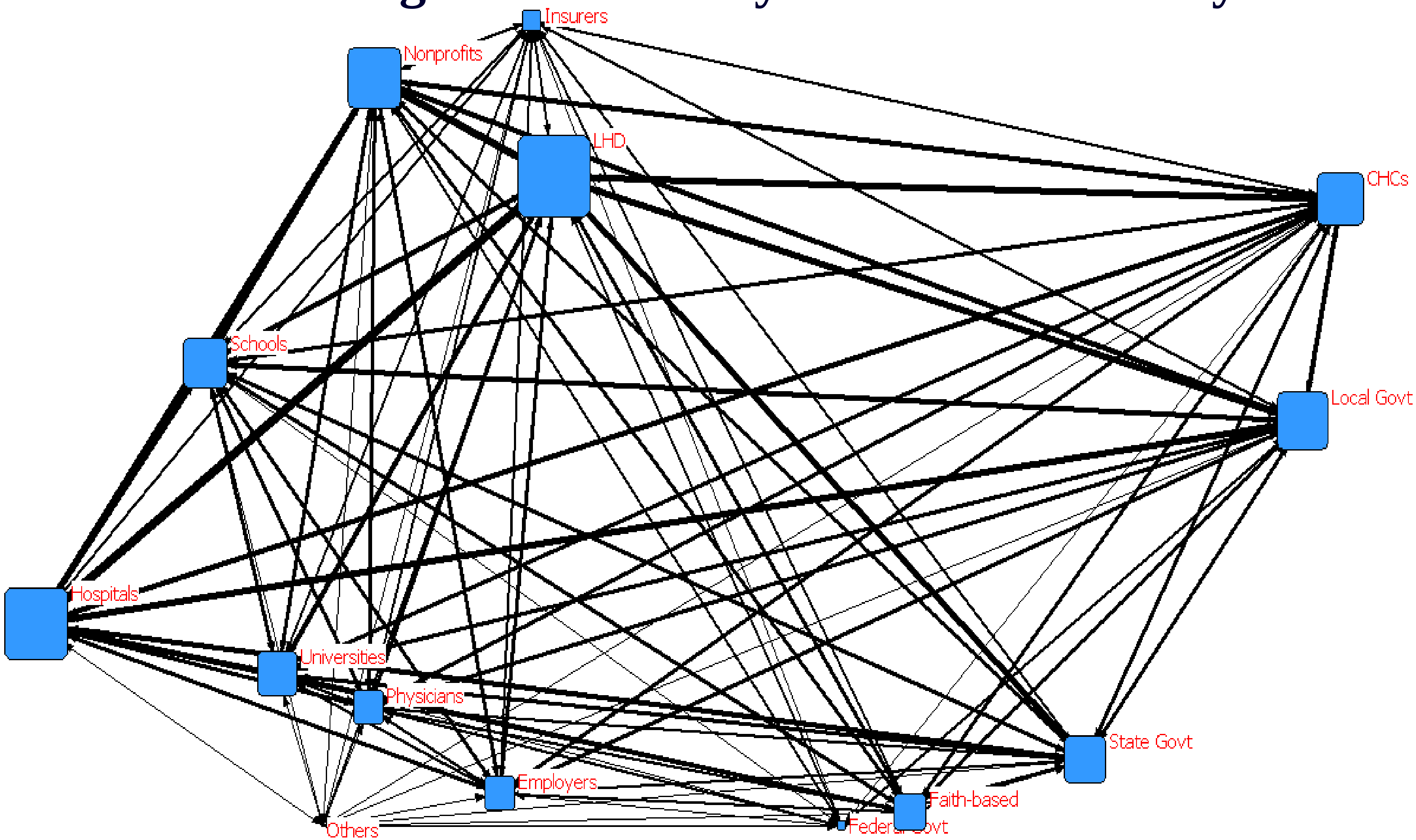
Delivery of recommended public health activities, 2006-14



National Longitudinal Survey of Public Health Systems, 2014

Delivery System Structures for Public Health Services

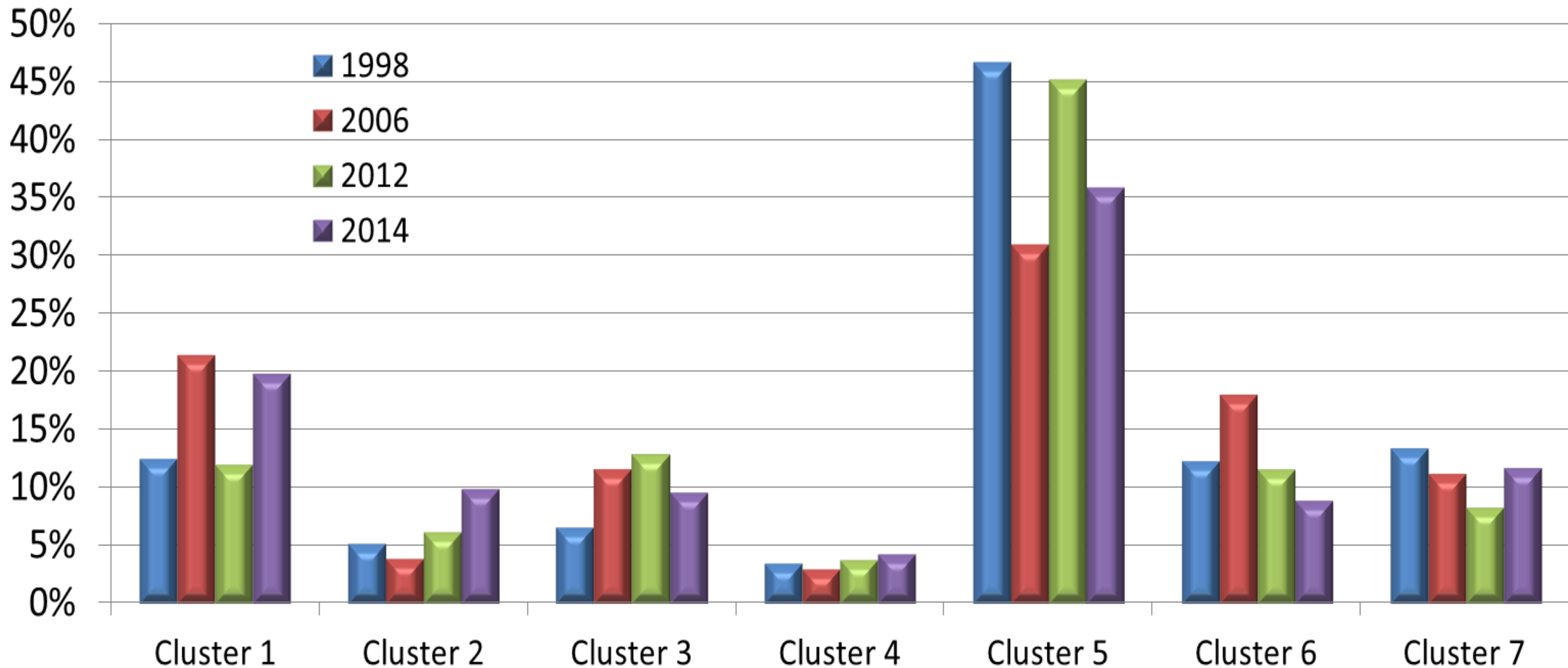
National Longitudinal Survey of Public Health Systems



Node size = centrality

Line size = % activities jointly contributed (tie strength)

Prevalence of Public Health System Configurations, 1998-2014



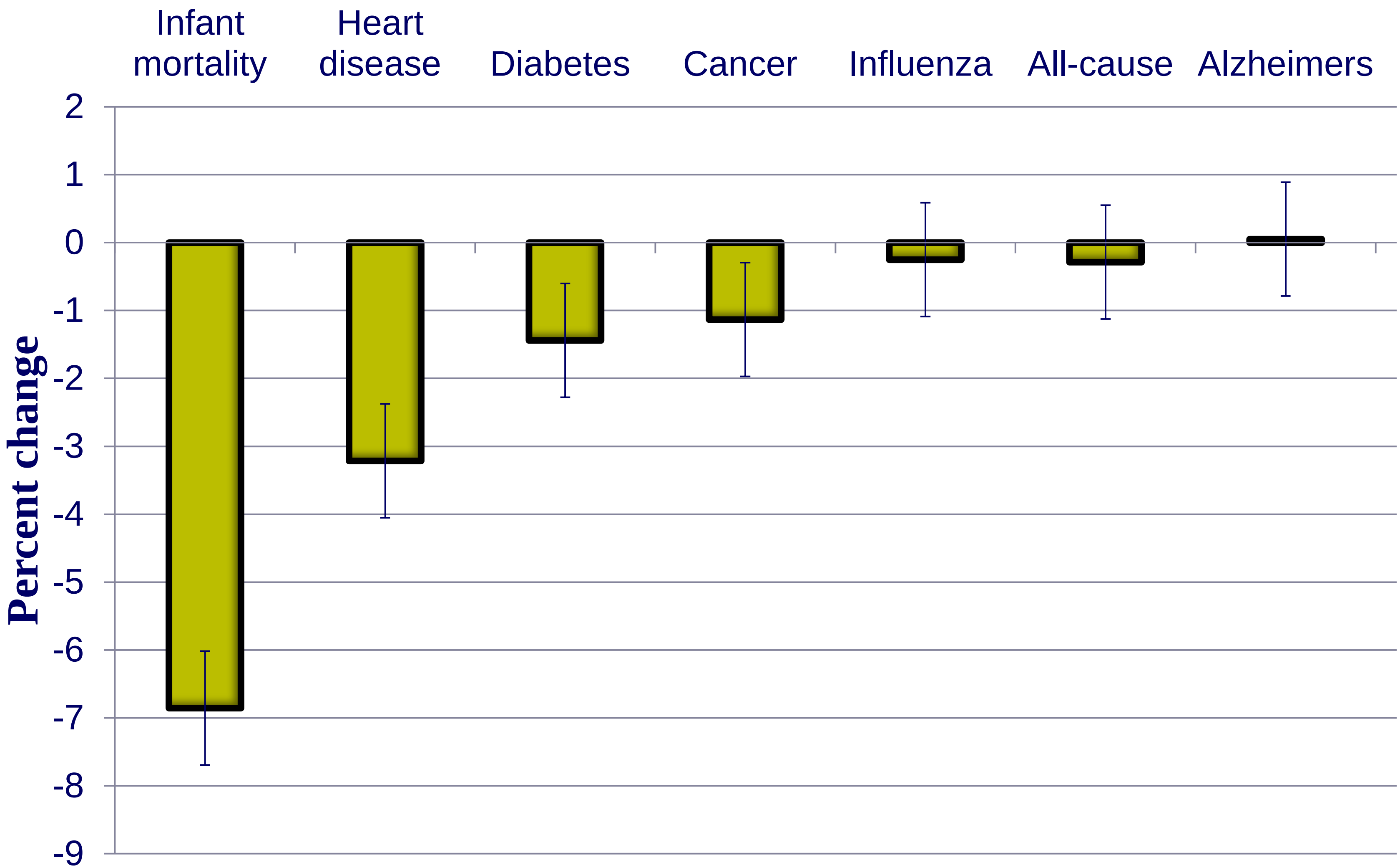
Scope	High	High	High	Mod	Mod	Low	Low
Centrality	Mod	Low	High	High	Low	High	Low
Density	High	High	Mod	Mod	Mod	Low	Mod

Comprehensive
(High System Capital)

Conventional

Limited

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

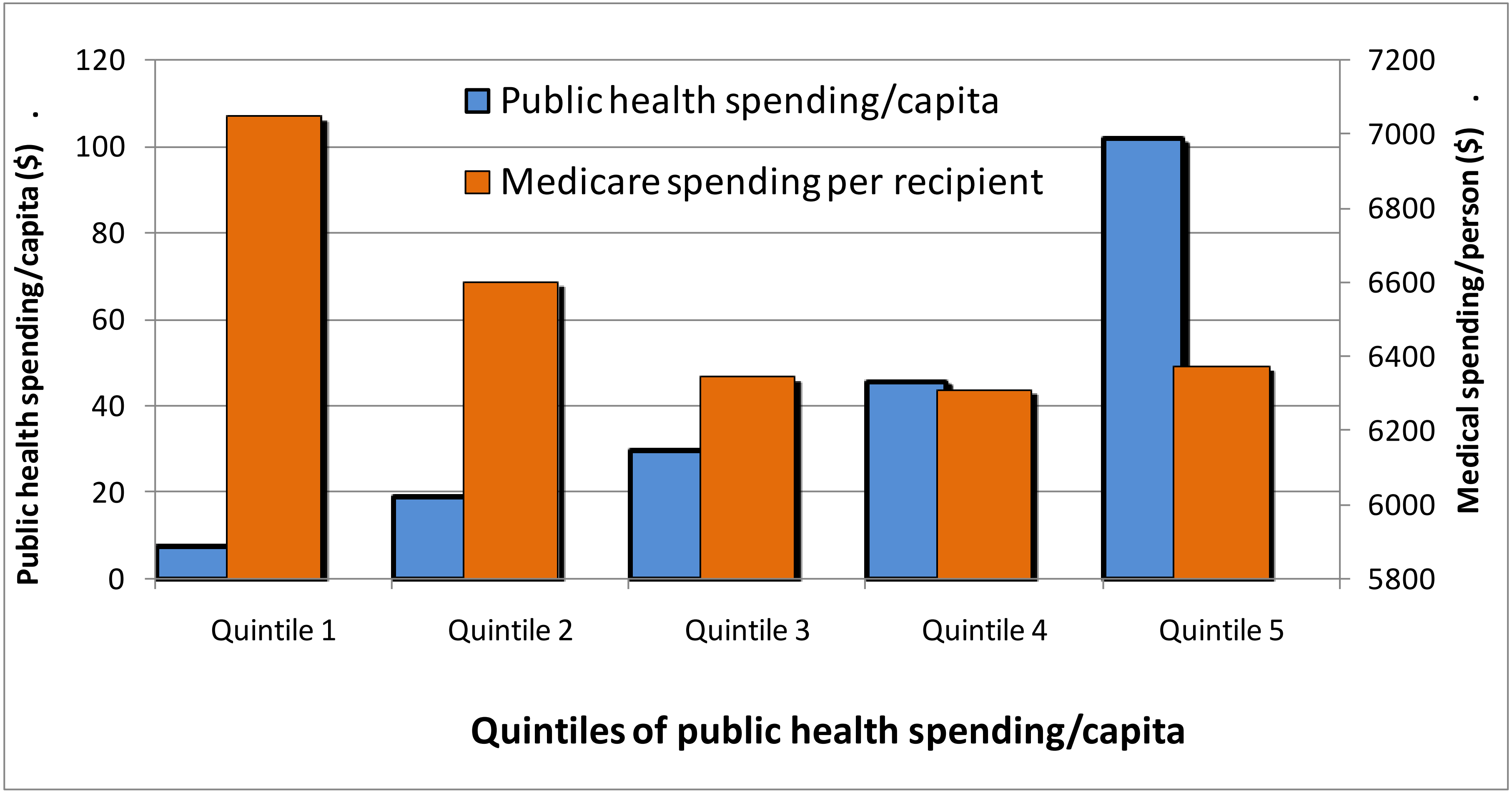


Hierarchical regression estimates with instrumental variables to correct for selection and unmeasured confounding

Mays et al. 2011

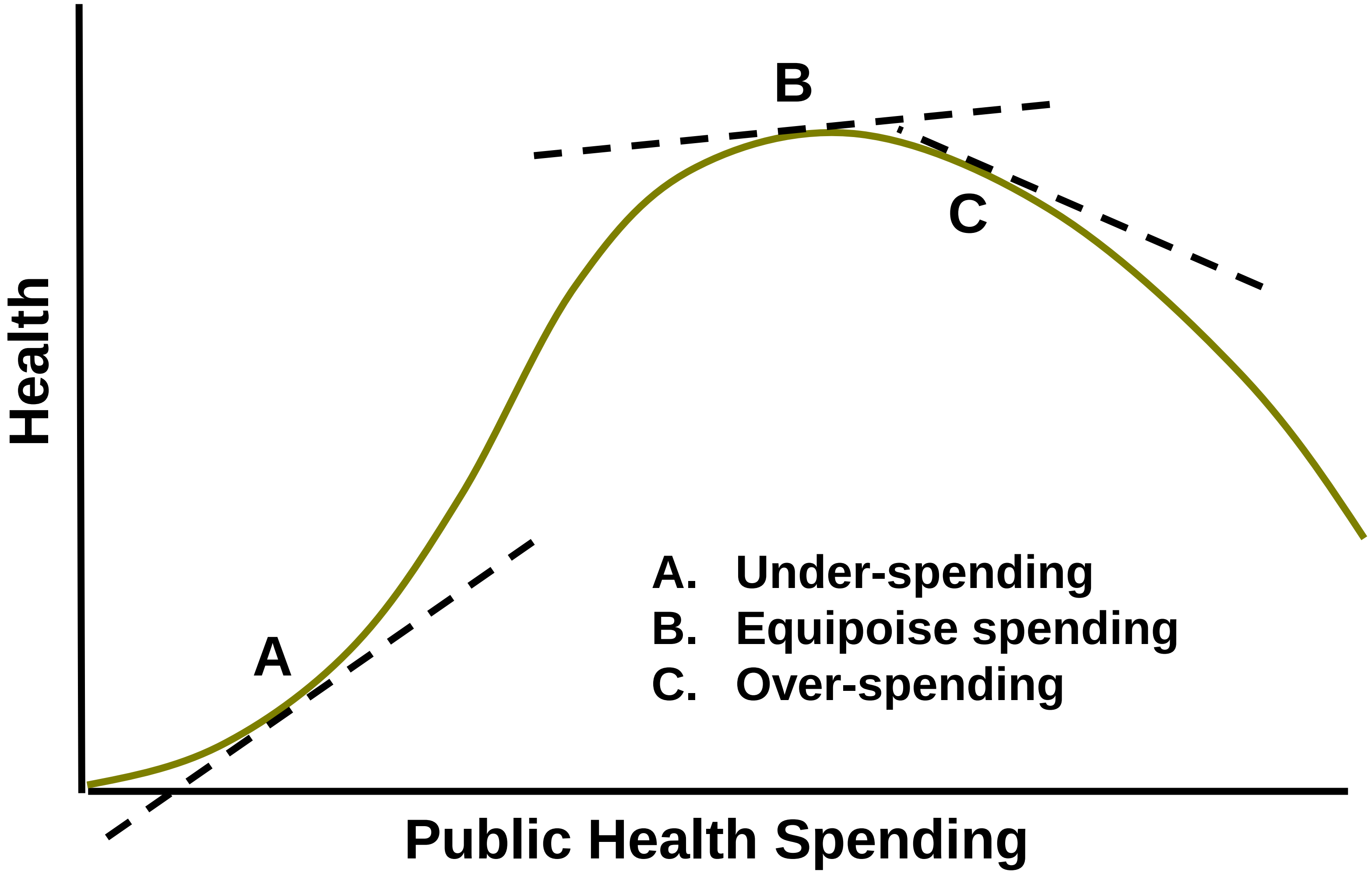
Prior Research: Medical cost offsets attributable to local public health spending 1993-2008

Offset elasticity = −0.088



Mays et al. 2013

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

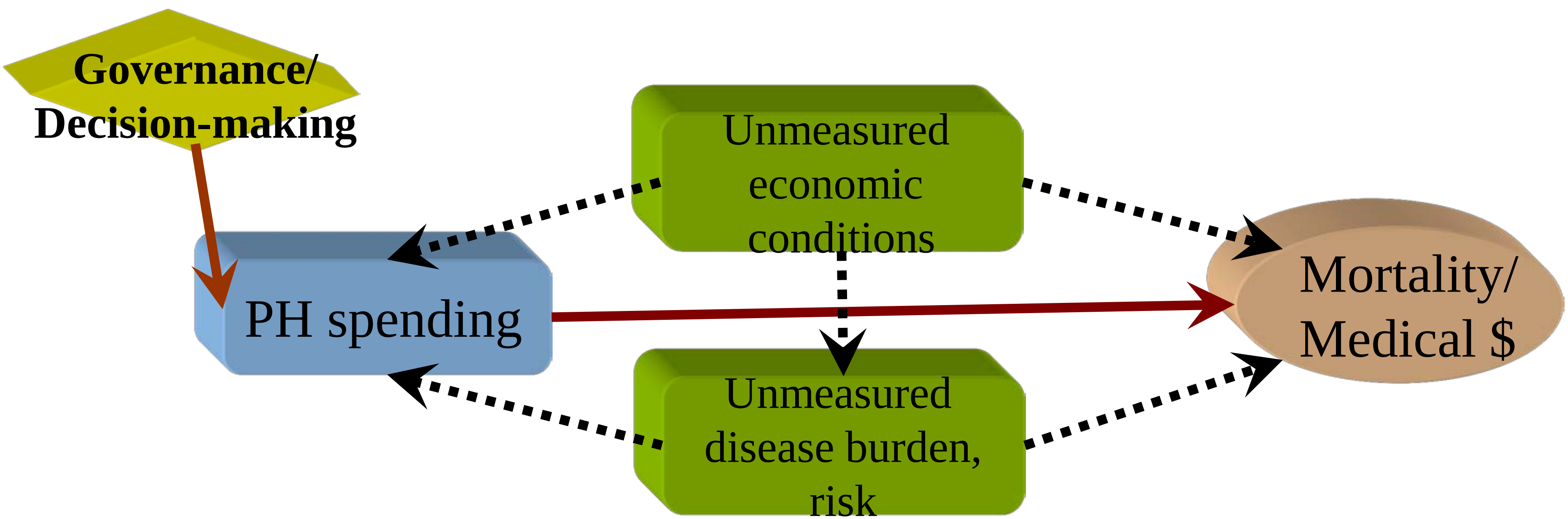
- Estimate predicted spending (P) as a function of all measured covariates (X) and instruments (Z)
- Model outcome (O) as nonlinear function of P(X,Z) and X
- Estimate $\partial O / \partial P$ the effect of a change in predicted spending on the outcome
- Find the distribution of P(X,Z) for the subset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

Governance/Decision Authority	Elasticity Coefficient	95% CI
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

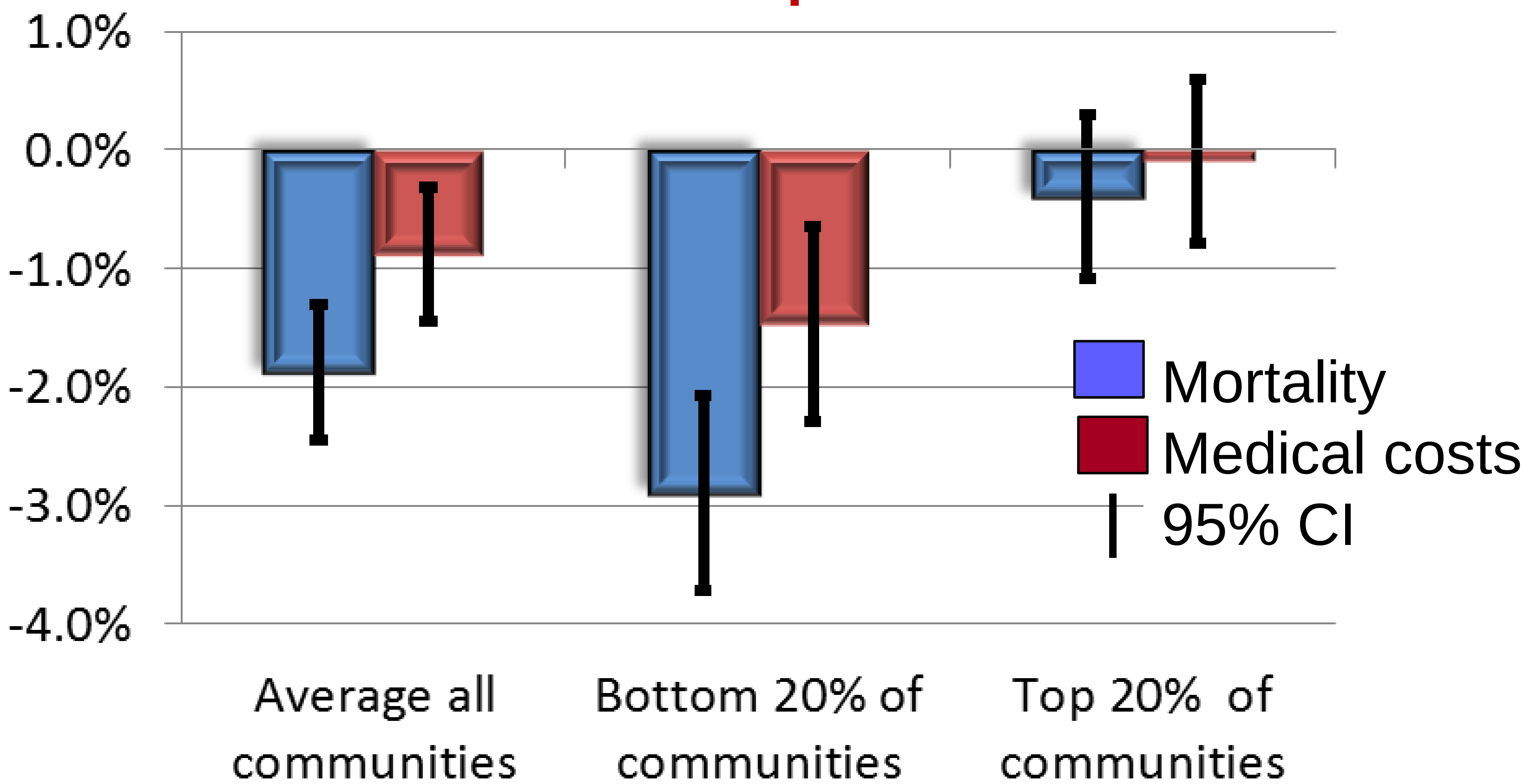
†As compared to the local board of health having the authority.

Mays et al. HSR 2009

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



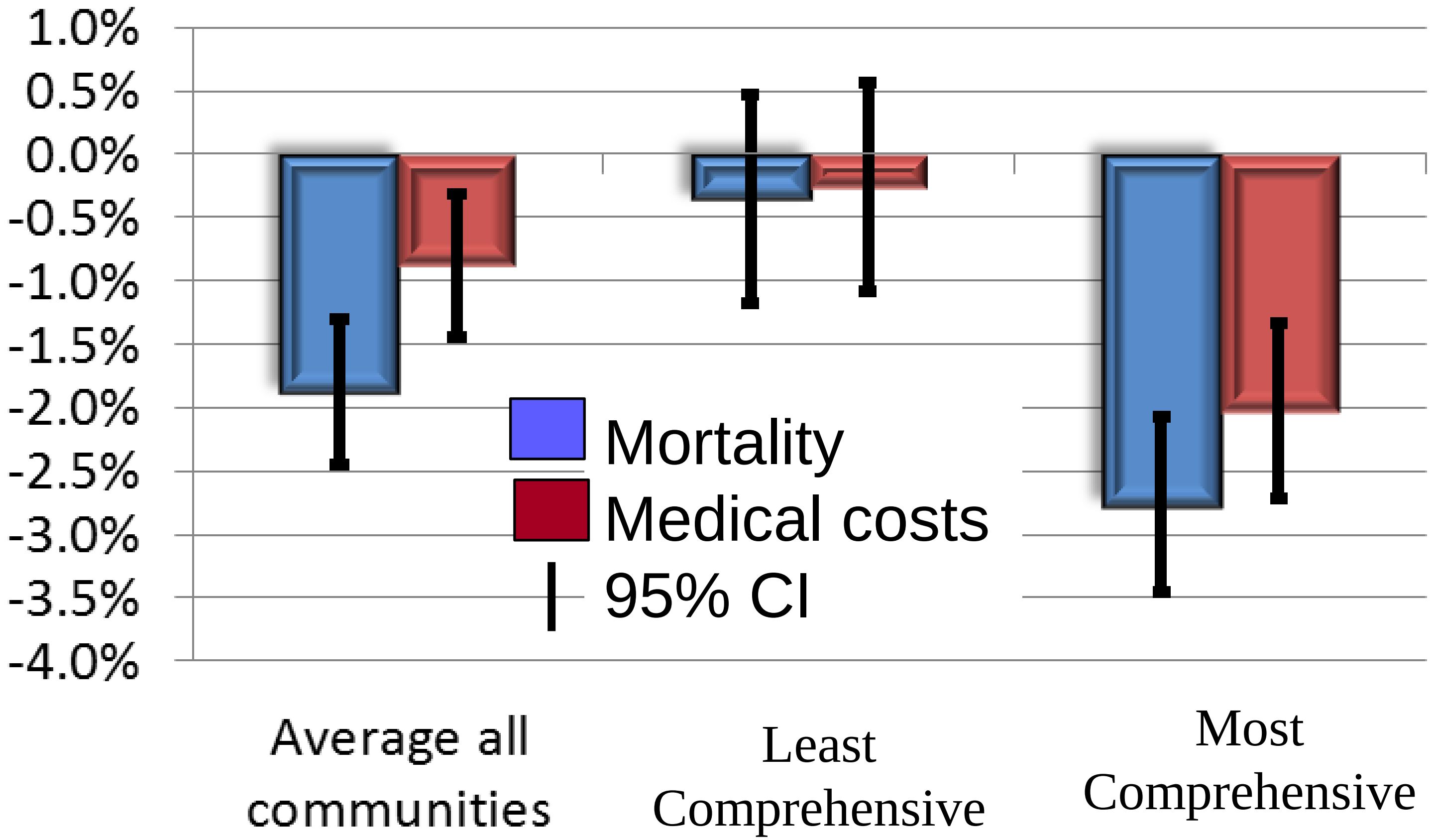
Log IV regression estimates controlling for community-level and state-level characteristics

Mays et al. forthcoming 2013

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

Impact of 10% Increase in Public Health Spending/Capita

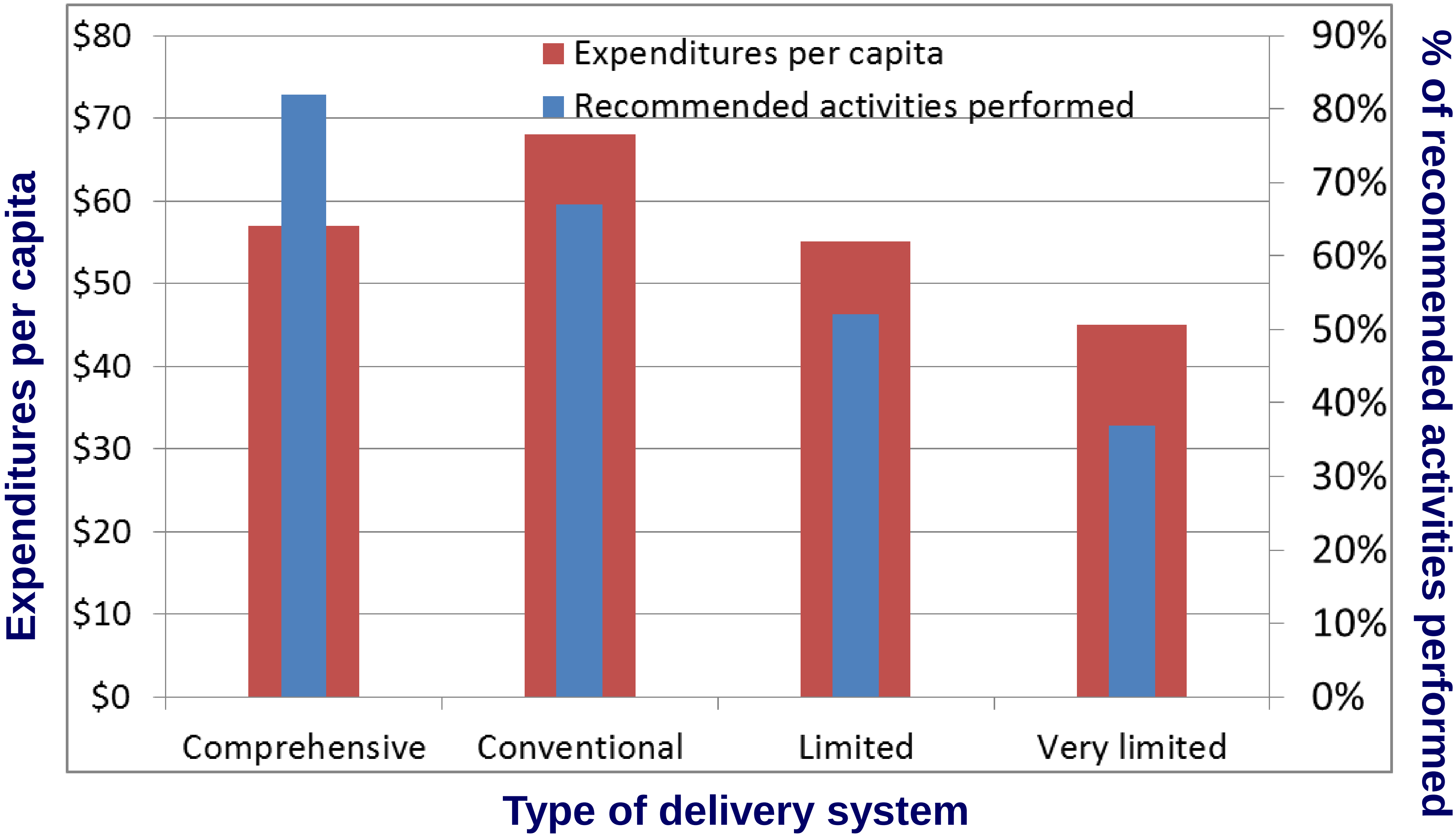
Based on Delivery System Comprehensiveness



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

Mays et al. forthcoming 2013

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

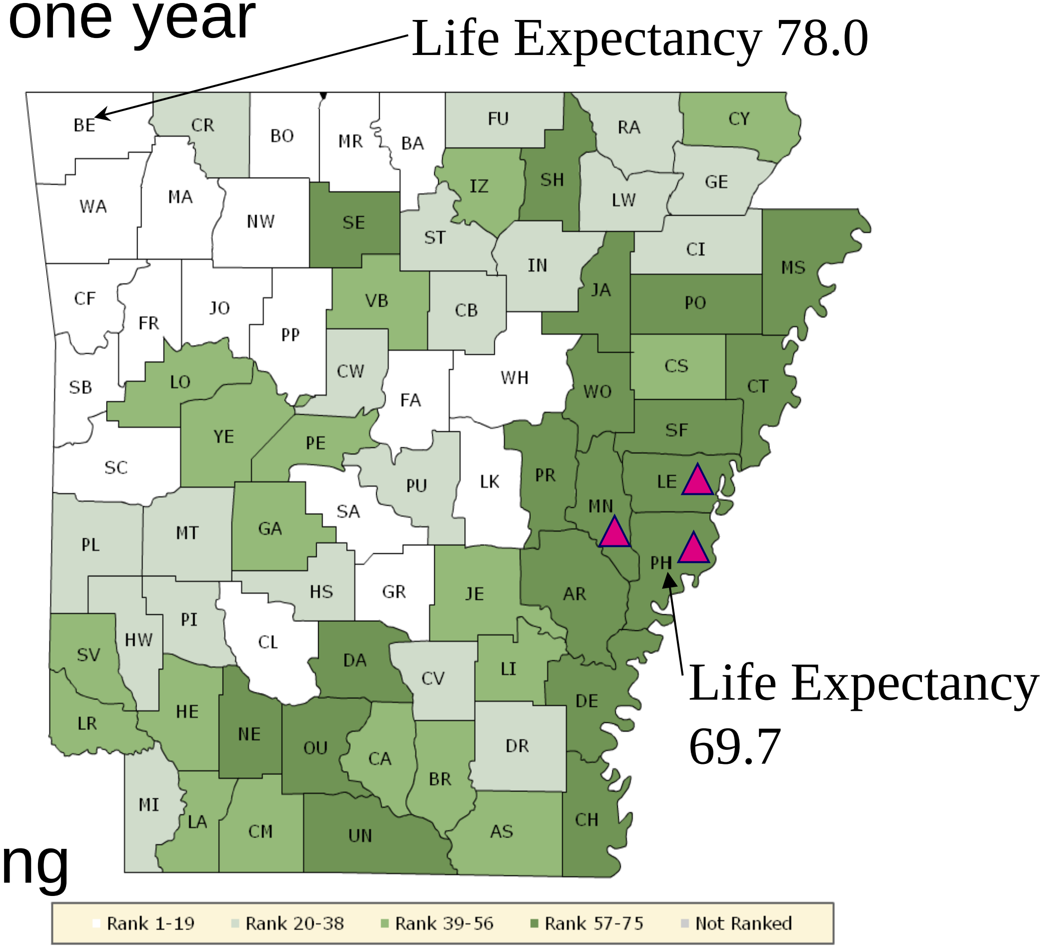
- Use community health workers & public health infrastructure to identify people with unmet social support needs
- Connect people to home and community-based services & supports
- Link to hospitals and nursing homes for transition planning
- Use Medicaid and SIM financing, savings reinvestment
- Costing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



Source: RWJF University of Wisconsin County Health Rankings 2014

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

Time Period*	Average Spending Change from Baseline	PET Spending 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

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  - 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.
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  - Quick Strike rapid-cycle studies

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