Public Health Perspective of Sickle Cell Disease

Richard Lottenberg, MD
University of Florida

New England Pediatric Sickle Cell Consortium
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Presentation Outline

- Evidence-based Public Health
  - Newborn Screening for Hemoglobinopathies
- Barriers to Care for Adolescents and Young Adults with SCD
- Health Care System Reform
  - Elements pertinent to SCD
  - Patient-Centered Medical Home

Evidence-based Public Health

Domains of Evidence-based Public Health Policy

Domain	Objective	Data Sources
Process	To understand approaches to enhance the likelihood of policy adoption	• Key informant interviews • Case studies • Surveys of setting-specific political contexts
Content	To identify specific policy elements that are likely to be effective	• Systematic reviews • Content analysis
Outcome	To document the potential impact of policy	• Surveillance systems • Natural experiments tracking policy-related endpoints

Brownson RC et al. Am J Public Health 2009; 99:1576-1583

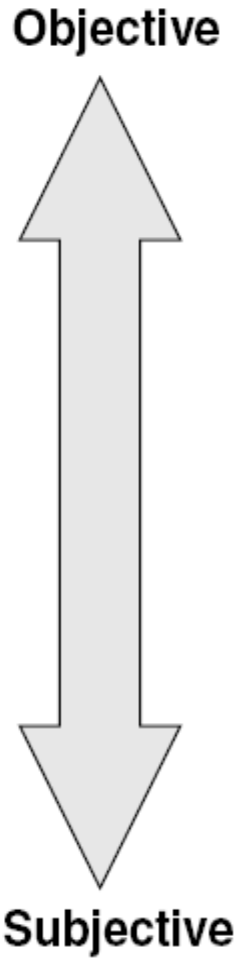
Components of Evidence-based Public Health

- Evaluation of the evidence
- Systematic use of data and information systems
- Applying program-planning frameworks
- Engaging the community in assessment and decision making
- Evaluation
- Dissemination

Brownson RC et al. Annu Rev Public Health 2009; 30:175-201

Types of Evidence

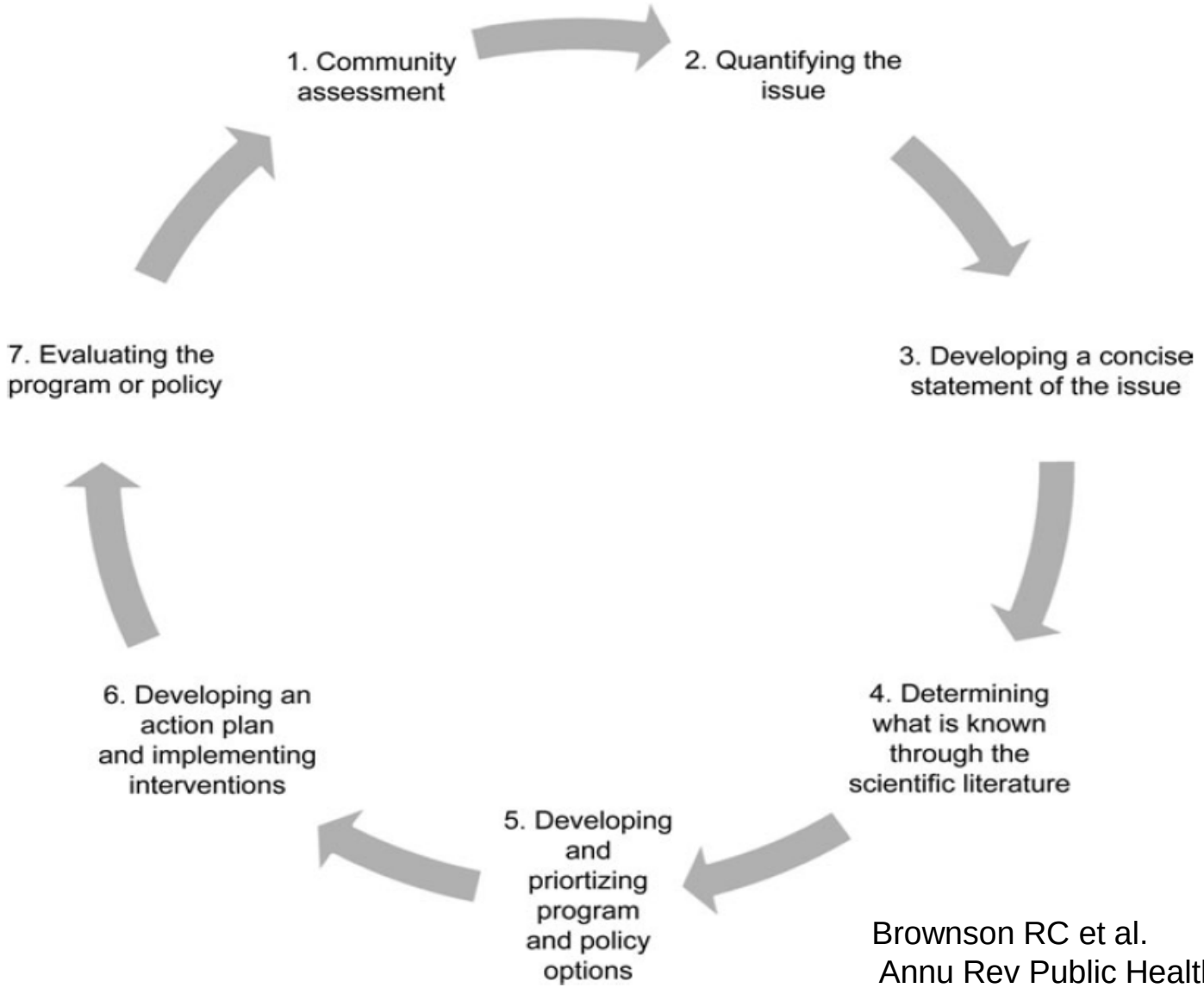
- Scientific literature in systematic reviews
- Scientific literature in one or more journal articles
- Public health surveillance data
- Program evaluations
- Qualitative data
  - Community members
  - Other stakeholders
- Media/marketing data
- Word of mouth
- Personal experience



Brownson RC et al. Annu Rev Public Health 2009; 30:175-201

Factors Impacting Policy/Program Recommendations

Brownson RC et al.
Annu Rev Public Health
2009; 30:175-201



Brownson RC et al.
Annu Rev Public Health 2009; 30:175-201

Newborn Screening for Hemoglobinopathies

Major RCTs In Sickle Cell Disease

Clinical trial	Outcome
Penicillin Prophylaxis in Sickle Cell Disease	Oral PCN greatly reduces the incidence of invasive pneumococcal infections
Prophylactic Penicillin Study II	Discontinuation of PCN can be considered at age 5
Multicenter Study of Hydroxyurea in Sickle Cell Anemia	Hydroxyurea reduces the frequency of painful episodes, acute chest syndrome transfusions, and hospitalizations
Preoperative Transfusion in Sickle Cell Anemia	Conservative tx to increase the Hb level to 10 g/dL is as effective as aggressive transfusion to reduce Hb S<30%
Prophylactic Transfusion in Pregnancy	Prophylactic tx to Hb level of 10 g/dL compared to tx at 6 g/dL did not improve outcomes
Stroke Prevention Trials in Sickle Cell Anemia (STOP I and STOP II)	Children at risk for stroke on the basis of an abnormally elevated TCD benefit from prophylactic blood transfusions

Newborn Screening Programs

- Goals of screening for sickle cell disease
  - Initiate penicillin prophylaxis and immunizations within 2 months of age
  - Parent/family education and counseling
  - Enrollment in comprehensive care
- Outcomes
  - No prospective clinical trials
  - Historically benefits of early intervention provided justification for screening
  - Contemporary infant cohort data

The Dallas Pediatric Sickle Cell Program

- Evidence-based medical care
- Prophylactic PCN for children with Hb SS and Hb S/β^0thal until age 5
- Patients instructed to seek medical attention for T > 38.5^0 C; home antibiotics available for low grade fever
- Administer heptavalent-conjugated pneumococcal vaccine; 23-valent pneumococcal vaccine at ages 2 and 5 yrs
- Administer *H. influenzae* b vaccination
- Periodic scheduled clinic visits
- Chronic transfusion program and hydroxyurea therapy

Quinn CT et al. Blood 2004; 103:4023-4027
Quinn CT et al. Blood 2010; 115:3447-3452

SICKLE CELL DISEASE IN CHILDREN AND ADOLESCENTS: DIAGNOSIS, GUIDELINES FOR COMPREHENSIVE CARE, AND CARE PATHS AND PROTOCOLS FOR MANAGEMENT OF ACUTE AND CHRONIC COMPLICATIONS*

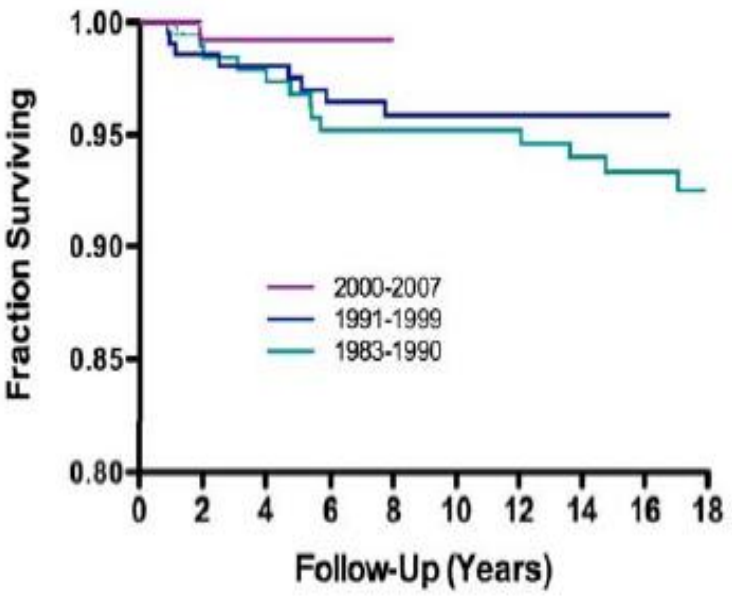
Peter A. Lane, George R. Buchanan, John J. Hutter, Robert F. Austin, Howard A. Britton, Zora R. Rogers, James R. Eckman, Michael R. DeBaun, Winfred C. Wang, Prasad Mathew, Sarah Iden, Michael Recht, Jesse D. Cohen, Ernest Frugé, Leanne Embry, Lewis Hsu, Brigitta U. Mueller, Robert Goldsby, Charles T. Quinn, Marie Mann, and Michele A. Lloyd-Puryear for the Sickle Cell Disease Care Consortium

Supported in part by the Mountain States Genetics Network, by the Texas Genetics Network and Texas Newborn Screening Hemoglobinopathy Grant (Texas Department of Health), and by Project #5H46 MC00132 and a contract from the Maternal and Child Health Bureau (Title V Social Security Act), HRSA, DHHS

*Revised at the Annual Meeting of the Sickle Cell Disease Care Consortium, Sedona, AZ, 11/10-12/2001
http://www.dshs.state.tx.us/newborn/pdf/sedona02.pdf

The Dallas Newborn Cohort

- Newborn inception cohort
  - 940 subjects
  - 8,857 pt-yrs of follow up
  - Mean F/U: 9.4 yrs
- Survival at 18 yrs of age
  - Hb SS and Hb S/β^0thal
    - 93.9% (90.3-96.2)
  - Hb SC and Hb S/β^+thal
    - 98.4% (94.4-99.5)



Survival for Hb SS and Hb S/β^0thal

Quinn CT et al. Blood 2010; 115:3447-3452

Newborn Screening for Hemoglobinopathies in the US

- Screening for sickle cell disease mandated by 50 states and Washington DC
- Guidelines
  - Council of Regional Networks for Genetic Services (CORN) guidelines
  - American Academy of Pediatrics Task Force on Newborn Screening
- HRSA SCD and Newborn Screening Program (2002)
  - State Title V and NBS programs
  - local community-based sickle cell disease organizations
  - SCD treatment centers and community-based primary care providers

U.S. Preventive Services Task Force
Pass et al. J Pediatr 2000; 137:S1-46
Pediatrics 2000; 106:383-427

Pediatric Hematology Specialty Care
South Carolina Medicaid Claims:1998-2006

Independent variable	Hydroxyurea-treated cohort (*N* = 175)	Control cohort (*N* = 348)	*P*
Ethnicity:			
African American	115 (65.7%)	228 (65.5%)	0.96
Non-African American (i.e., Hispanic, Middle Eastern, Asian Indian, or mixed)	60 (34.3%)	120 (34.5%)	
Gender			
Female	73 (41.7%)	146 (42.0%)	0.96
Male	102 (58.3%)	202 (58.0%)	
Adenotonsillectomy			
Yes	21 (12.0%)	41 (11.8%)	0.94
No	154 (88.0%)	307 (88.2%)	
Years in Medicaid	9.5 (SD = 2.3)	9.6 (SD = 2.3)	0.92
Percent receiving care at specialty clinic	11.8 (SD = 14.1)	9.5 (SD = 11.4)	0.02
Hydroxyurea mean days prescribed	869.8 (SD = 770.3)	0	
Penicillin days received	797.4 (SD = 757.3)	742.8 (SD = 731.5)	0.58
Transfusions/year total	3.4 (SD = 9.8)	5.3 (SD = 12.9)	0.30

Stallworth JR et al. Am J Hematol. 2010 May 24 [Epub]

Healthy People 2020
New Objectives

- Increase the proportion of persons with hemoglobinopathies who receive disease-modifying therapies.
- Increase the proportion of persons with a diagnosis of hemoglobinopathies who receive early and continuous screening for complications.
- Increase the proportion of persons with hemoglobinopathies and their families who are referred for evaluation and treatment.
- Increase the proportion of children with SCD who receive penicillin prophylaxis from 4 months until 5 years of age.
- Increase the proportion of persons with blood disorders who receive recommended vaccinations.

Barriers to Health Care Access for Young Adults with Sickle Cell Disease

Potential Impact of Health Care Reform

Barriers to Health Care for Young Adults with Sickle Cell Disease

- Inadequate insurance coverage to access appropriate care in many health care settings
- Inadequate number of adult-oriented physicians with skills or interest in SCD
- Limited timely access to outpatient services
- Excessive reliance on care through emergency departments

Consequences of the Health Inequity

- Suboptimal attention to health maintenance and management of acute illness
  - Increased complications from SCD and comorbidities
- Decreased quality of life
  - Inadequate pain management
  - Psychosocial impairment
- Decreased productivity
  - Education
  - Employment capacity
- Negative impact on family members
  - Financial costs
  - Interruption of normal family life

Dallas Newborn Cohort
Circumstances of Death: 18 Years of Age and Older

Patient no.	Age at transition, y	Age at death, y	Chronic complication of sickle cell disease	Circumstances of death
1	N/A*	18.8	Renal failure; dialysis	Found dead at home
2	17.9	19.2	None known	Acute chest syndrome
3	18.0	18.3	None known	Acute chest syndrome−refused transfusion (Jehovah's Witness)
4	18.2	20.6	None known	Fell in hospital while pregnant−cerebral hemorrhage
5	18.3	19.3	None known	Acute chest syndrome complicated by multiorgan failure syndrome
6	18.4	23.7	None known	Presumed stroke†
7	18.5	18.7	None known	Multiorgan failure syndrome

*Not applicable: transition delayed beyond 18 years of age because of chronic renal failure.
†This was the first patient in the Dallas Newborn Cohort.

Quinn CT et al. Blood 2010; 115:3447-3452

Racial and Ethnic Disparities in Health Care ACP Policy Paper

The health care delivery system must be reformed to insure that patient-centered medical care is easily accessible to racial and ethnic minorities and physicians are enabled with the resources to deliver quality care. (Position 5)

- African Americans are less likely than whites to have access to a regular source of care
- Physicians serving primarily African Americans have greater difficulty in referring patients to specialists
- Minority patients are more likely than whites to receive care in an environment other than a doctor's office or private clinic

American College of Physicians. Racial and Ethnic Disparities in Health Care; 2010: Policy Paper.

Patient Protection and Affordable Care Act
Health Care and Education Reconciliation Act of 2010

- Health Care Coverage
- Health Care Workforce
- Payment and Delivery Systems
- Medical Liability

Public Law 111-152
Public Law 111-148
American College of Physicians: An Internist's Practical Guide to Understanding Health System Reform; June 2010

Health Care Coverage 2010

- Sliding scale tax credits to help businesses purchase health insurance for employees
- Requires all health plans to provide coverage to children up to age 19 with pre-existing conditions.
- High risk pool will be created to provide coverage for adults with pre-existing medical conditions
- Requires all new health plans to provide evidence-based preventive services with no cost-sharing, extend dependent coverage to individuals up to age 26,

  eliminate lifetime limits on coverage

American College of Physicians: An Internist's Practical Guide to Understanding Health System Reform; June 2010

Payment and Delivery System Reforms 2010 - 2011

- Comparative effectiveness research
- Medicare and Medicaid Center on Innovation to pilot test innovative payment and delivery system reforms including medical home model
- Provides a 10 percent Medicare bonus payment for designated primary care services
- Grant program to establish community-based interdisciplinary, inter-professional teams to support primary care practices

American College of Physicians: An Internist's Practical Guide to Understanding Health System Reform; June 2010

Health Care Coverage 2014

- Expand coverage to 32 million people
- Provide sliding scale tax credits to help individuals and families buy coverage
- All individuals up to 133 percent of the Federal Poverty Level will be eligible for Medicaid
- Requires individuals to buy coverage or pay a penalty, with hardship exemptions
- Requires large employers to contribute to coverage or pay the costs associated with subsidies to their employees

American College of Physicians: An Internist's Practical Guide to Understanding Health System Reform; June 2010

Improvement in the Quality of Health Care

Department of Health and Human Services is required to development a strategy to provide health plans with increased reimbursement or other incentives for implementing activities such as quality reporting, effective case management, care coordination, chronic disease management, medication and care compliance initiatives, including through use of the medical home model for treatment or services

American College of Physicians: An Internist's Practical Guide to Understanding Health System Reform; June 2010

Definition of the Medical Home

American Academy of Pediatrics policy statement

…medical care of infants, children, and adolescents ideally should be accessible, continuous, comprehensive, family centered, coordinated, and compassionate.

Pediatrics 2002; 110:184-186

Closing The Divide: How Medical Homes Promote Equity In Health Care

Insurance coverage helps people gain access to health care, but the next thing you need to ask is "access to what?"

Anne C. Beal, Michelle M. Doty, Susan E. Hernandez, Katherine Shea, and Karen Davis

The Commonwealth Fund

June 2007

The Commonwealth Fund 2006 Health Care Quality Survey

- Princeton Survey Research Associates International
- May 30, 2006 – October 19, 2006
- Random representative sample of 3,535 adults >18 years of age
  - 25-minute telephone interviews
  - Weighted analysis based on 2,837 respondents (ages 18 – 64)

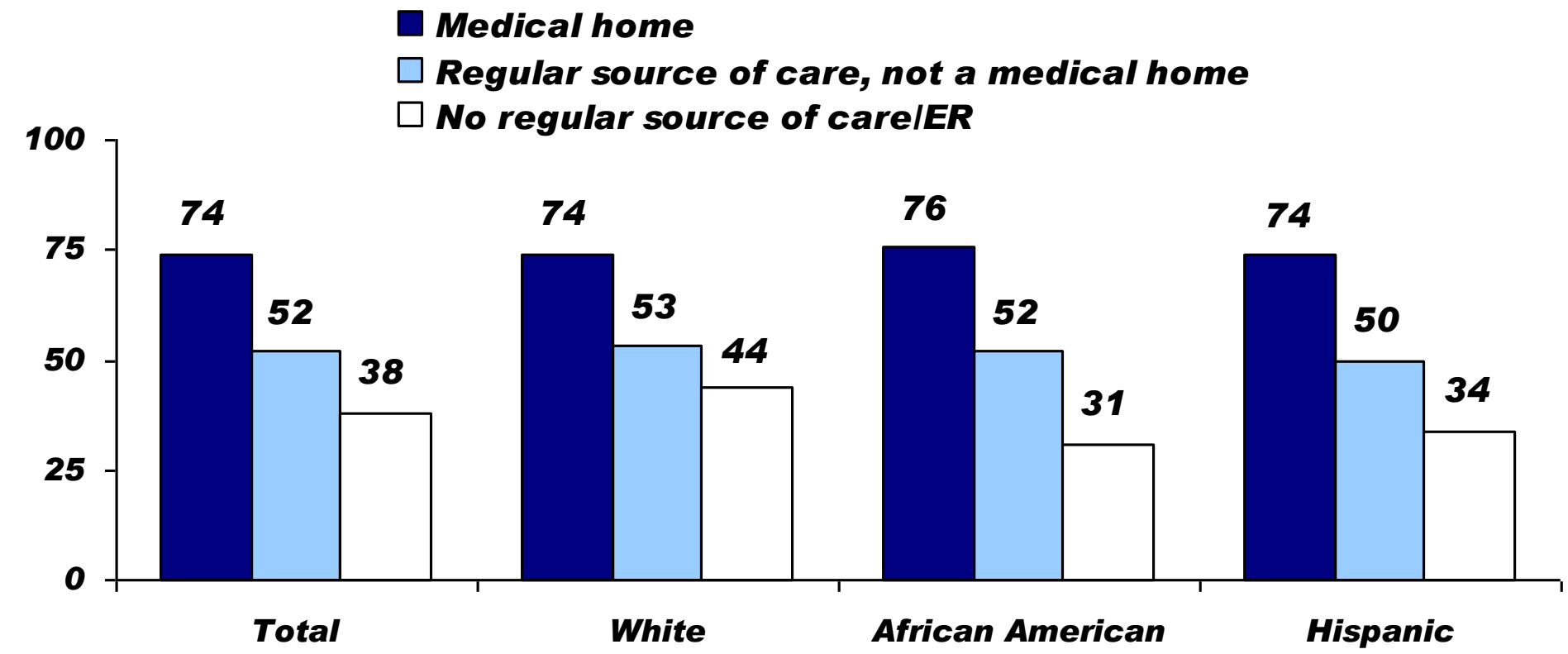
Commonwealth Fund 2006 Health Care Quality Survey
www.commonwealthfund.org

Indicators of a Medical Home

Parameter	Percent
A regular provider or place of care	80
No difficulty contacting provider by phone	85
No difficulty getting advice and medical care after hours	65
Office visits well organized and running on time	66

Commonwealth Fund 2006 Health Care Quality Survey

Racial and Ethnic Differences in Getting Needed Medical Care Are Eliminated When Adults Have Medical Homes



Percent Reporting Always Getting Care They Need

Commonwealth Fund 2006 Health Care Quality Survey

Benefits of Having a Medical Home*

- Patients more likely to check BP and keep it controlled
- Higher rates of preventive care reminders
- Higher rates of cholesterol screening, counseling on diet and exercise
- Patients more likely to receive plans on home management of medical condition

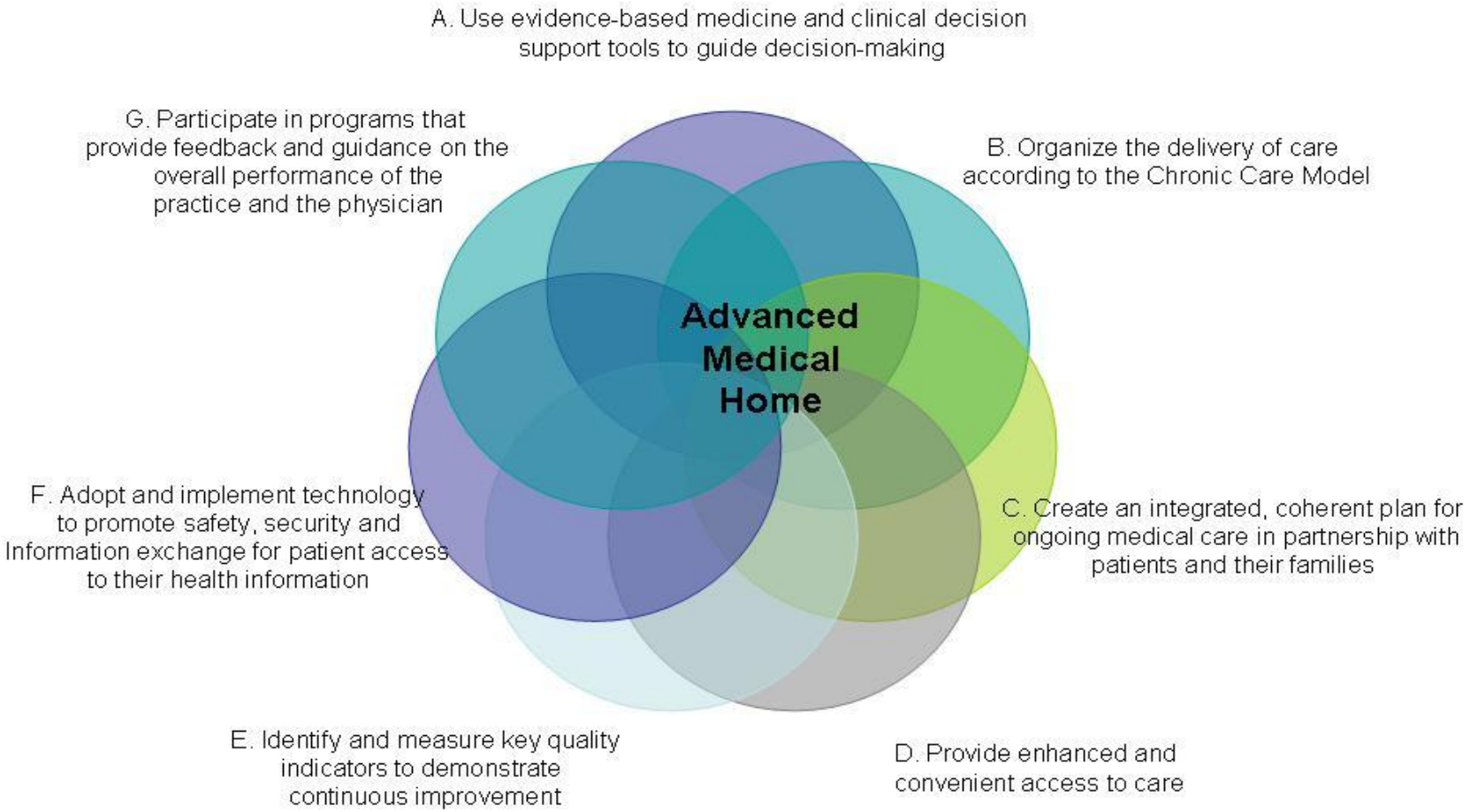
*Independent of Income or Insurance

The Advanced Medical Home: A Patient-Centered, Physician-Guided Model of Health Care

American College of Physicians

- Call for a public policy initiative
- Changes in health care reimbursement
- Changes in workforce and training policies
- Conduct further research on the Advanced Medical Home

ACP Policy Monograph
January 22, 2006
www.acponline.org/hpp/adv_med.pdf



ACP Policy Monograph January 22, 2006

Application of the Advanced Medical Home Model to Adult SCD Health Care

- Primary care physicians as principal care providers
  - Based on patient needs
  - Link with sickle cell consultative services
- Emphasis on continuous rather than episodic care
- Incorporation of evidence-based medicine
  - Health maintenance
  - Enhance appropriate use of hydroxyurea
- Preventive measures as a priority
- Increased opportunity for patients to benefit from innovative therapies