U.S. Department of Health and Human Services
National Institutes of Health

National Heart Lung and Blood Institute

Addressing Sickle Cell Care

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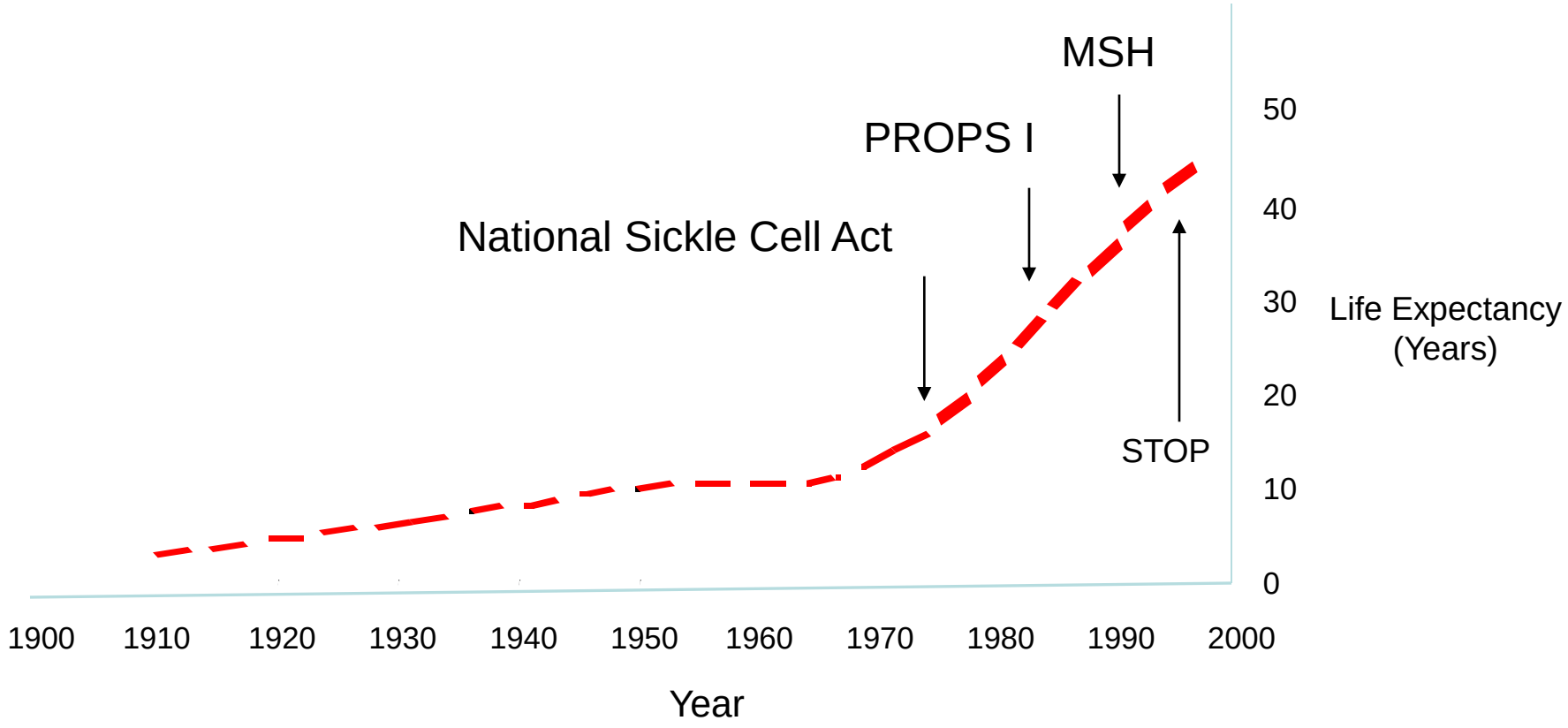
Increases in Life Expectancy of Patients with Sickle Cell Anemia



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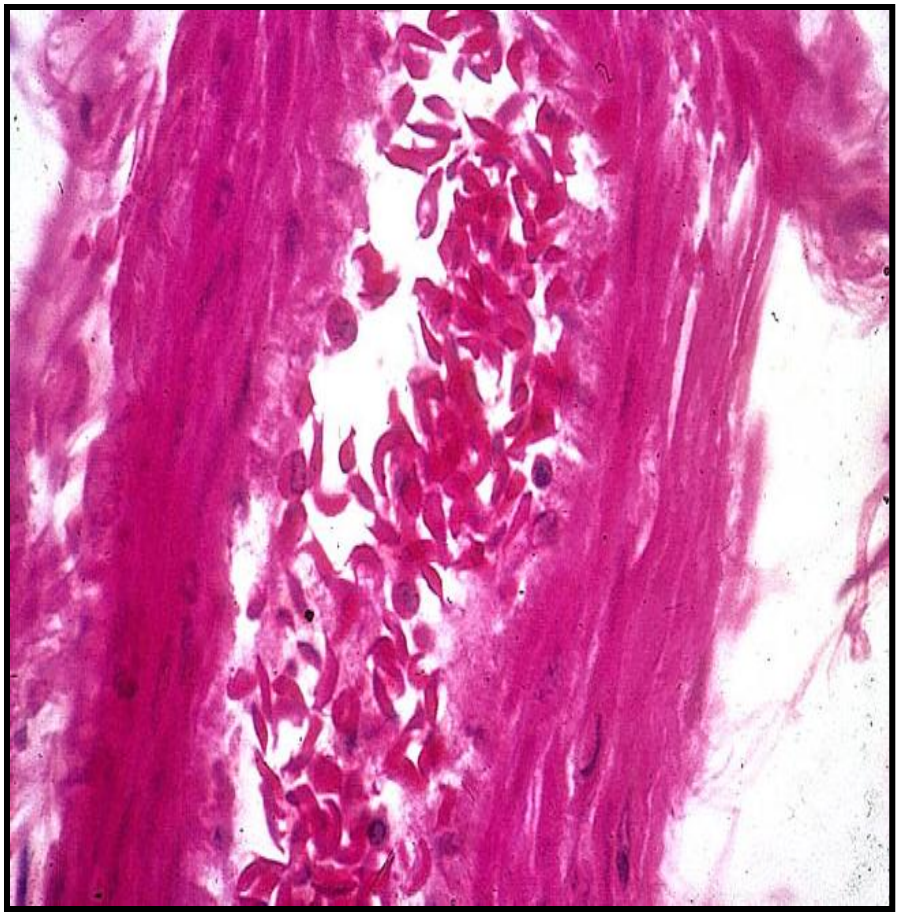
Advancements in Sickle Cell That Have Improved Life Expectance

- Sickle cell screening
- Penicillin Prophylaxis
- Stoke Screening (Transcranial Doppler Ultrasound)
- Blood Transfusion
- Bone Marrow Transplantation
- Hydroxyurea



Agencies of Health and Human Services, HHS

- Administration for Children & Families (ACF)
- Administration on Aging (AoA)
- *Agency for Healthcare Research & Quality (AHRQ)*
- Agency for Toxic Substances & Disease Registry (ATSDR)
- *Centers for Disease Control & Prevention (CDC)*
- *Centers for Medicare & Medicaid Services (CMS)*
- Food & Drug Administration (FDA)
- *Health Resources & Services Administration (HRSA)*
- Indian Health Service (HIS)
- *National Institutes of Health (NIH)*
- Substance Abuse & Mental Health Services Administration (SAMHSA)

*Members of HHS Hemoglobinopathies Federal Partners Initiative

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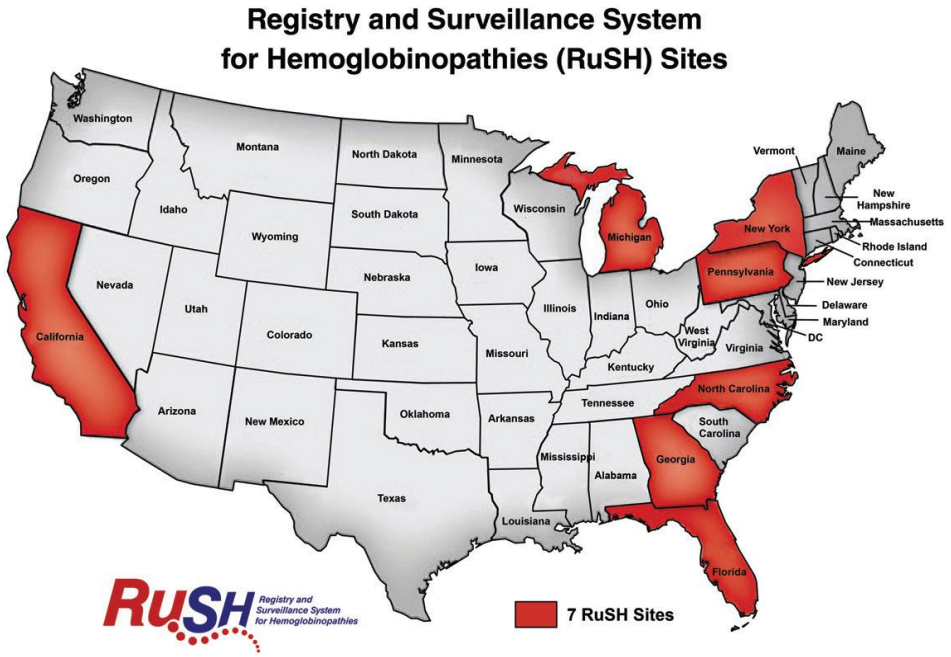
Agency for Healthcare Research and Quality

- Charged with improving quality, safety, efficiency and effectiveness of healthcare
- US Preventative Task Force releases recommendations
- Evidence Based Practice Centers support development of evidence reports and technology assessments to assist public- and private-sector organizations to improve quality of health care in the United States.
- In 2008, released report supporting use of Hydroxyurea as an effective therapy in sickle cell disease
- AHRQ provides grants to study the improvement of sickle cell care, including a grant given to Dr. Paula Tanabe in 2011 to study improving emergency care for sickle cell patients.

Center for Disease Control and Prevention

- The federal agency responsible for protecting the nations health through health promotion, prevention of disease, injury and disability, and preparedness for new health threats
- The National Center on Birth Defects and Developmental Disabilities has a Blood Disorders division which produces sickle cell materials and works to improve sickle cell care
- The Blood Disorders division is leading the RuSH project, (Registry and Surveillance for Hemoglobinopathies)



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More on RuSH

Project Goals

- **Determine How Many People Have SCD and Thalassemia**
- **Increase Knowledge and Awareness**
- **Demonstrate the Value of Surveillance Data**

State-based information often includes:

- **Vital records—births, deaths, pregnancies, and pregnancy outcomes**
- **Immunization records—vaccinations or shots given to prevent diseases**
- **Newborn screening results**
- **Health care statistics—hospitalizations, emergency room visits, and other sources of medical care**

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Health Resources and Services Administration

- The federal agency responsible for improving access to health care services for people who are uninsured, isolated, or medically vulnerable
- HRSA works with Federally Qualified Health Centers, visiting nurses programs, nursing home programs, and other community based programs designed to reach vulnerable populations
- The Maternal and Child Health Bureau within HRSA is responsible for the National Sickle Cell Disease Newborn Screening Program and the National Sickle Cell Disease Treatment Demonstration Program
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National Sickle Cell Disease Newborn Screening Program

Goals and Activities

- Improve follow-up of individuals detected through newborn screening and other screening approaches with sickle cell disease, sickle cell trait, and other hemoglobinopathies
- Assure that individuals identified with sickle cell disease and other hemoglobinopathies receive the highest quality of health care and supportive services throughout their lifespan
- Assure that individuals with sickle cell disease, trait and other hemoglobinopathies—including those in “emerging populations”—receive appropriate education and counseling to enable them to make informed health-related decisions, including, but not limited to, those related to reproductive choices

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National Sickle Cell Disease Treatment Demonstration Program

- Program is designed to establish practice models for the prevention and treatment of sickle cell disease through the coordinated efforts of providers, key stake holders, affected individuals and their families.
- Networks have two main goals:
  - support the provision of coordinated, comprehensive, culturally competent and family-centered care for individuals with sickle cell disease.
  - work collaboratively with partners such as Federally Qualified Health Centers (FQHC); nonprofit hospitals or clinics; university health centers offering primary care; subspecialty comprehensive sickle cell centers; and community-based organizations that provide resources to people with sickle cell disease.

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National Heart, Lung and Blood Institute

- Has been involved in sickle cell research since 1950's
- NHLBI mandated to research sickle cell with passage of Sickle Cell Disease Treatment Act of 1972
- The Division for Blood Disorders and Resources sponsors bench and clinical research to improve sickle cell care
- The Division for the Application of Research Discoveries is sponsoring the development of practice guidelines
- This year, DARD supported the establishment of the National Blood Disorders Program

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National Blood Disorders Program* Coordinating Committee

Professional and Non-Profit Organizations:

1. American Academy of Emergency Medicine
2. American Academy of Family Physicians
3. American Academy of Pediatrics
4. American Academy of Physician Assistants
5. American Association of Blood Banks
6. American College of Medical Genetics
7. American College of Physicians
8. American Osteopathic Association
9. American Society of Hematology
10. American Society of Pediatric Hematologist/Oncologist
11. International Association of Sickle Cell Nurses and Physician Assistants
12. National Initiative for Children's Healthcare Quality
13. National Medical Association
14. Sickle Cell Disease Association of America
15. Society for Academic Emergency Medicine

Federal Agencies:

19. Agency for Healthcare Research and Quality (AHRQ)
20. Centers for Disease Control and Prevention (CDC)
21. Centers for Medicaid and Medicare Services (CMS)
22. Food and Drug Administration (FDA)
23. Health Resources and Services Administration (HRSA)
24. National Institute of Minority Health and Health Disparities (NCMHD)
25. National Heart, Lung and Blood Institute (NHLBI)
26. Office of the Assistant Secretary for Planning and Evaluation
27. Office of Minority Health, HHS (OMH)

* Convened by the NHLBI

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Infrastructure of NHLBI-Sponsored National Programs

- Wider circles indicate more partners
- A Coordinating Committee is the leadership body of the Program
- Work groups address targeted topics and projects
- Wider involvement is through the internet via a “Knowledge Network” and other means

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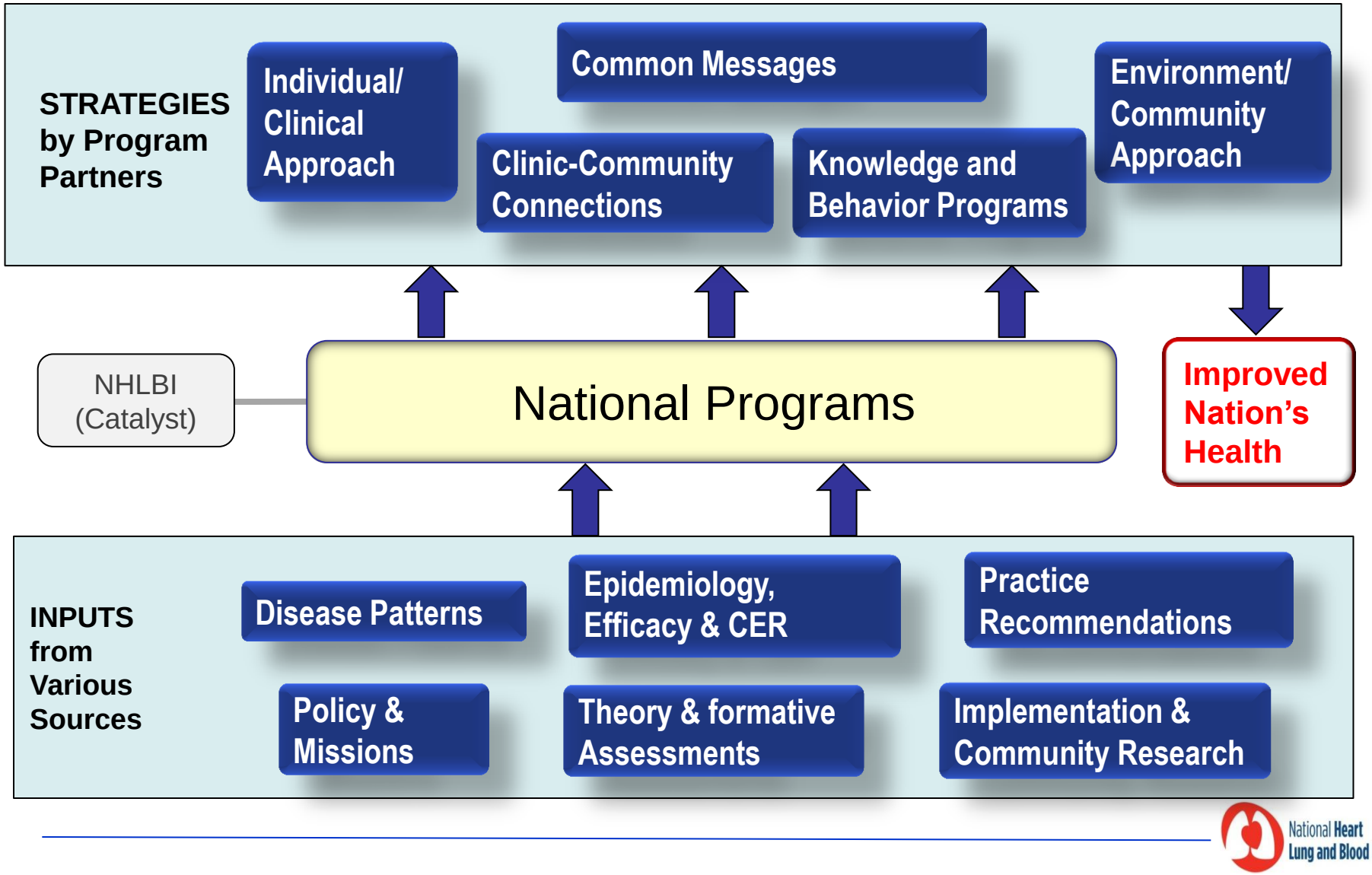
Functioning of National Partnership Programs



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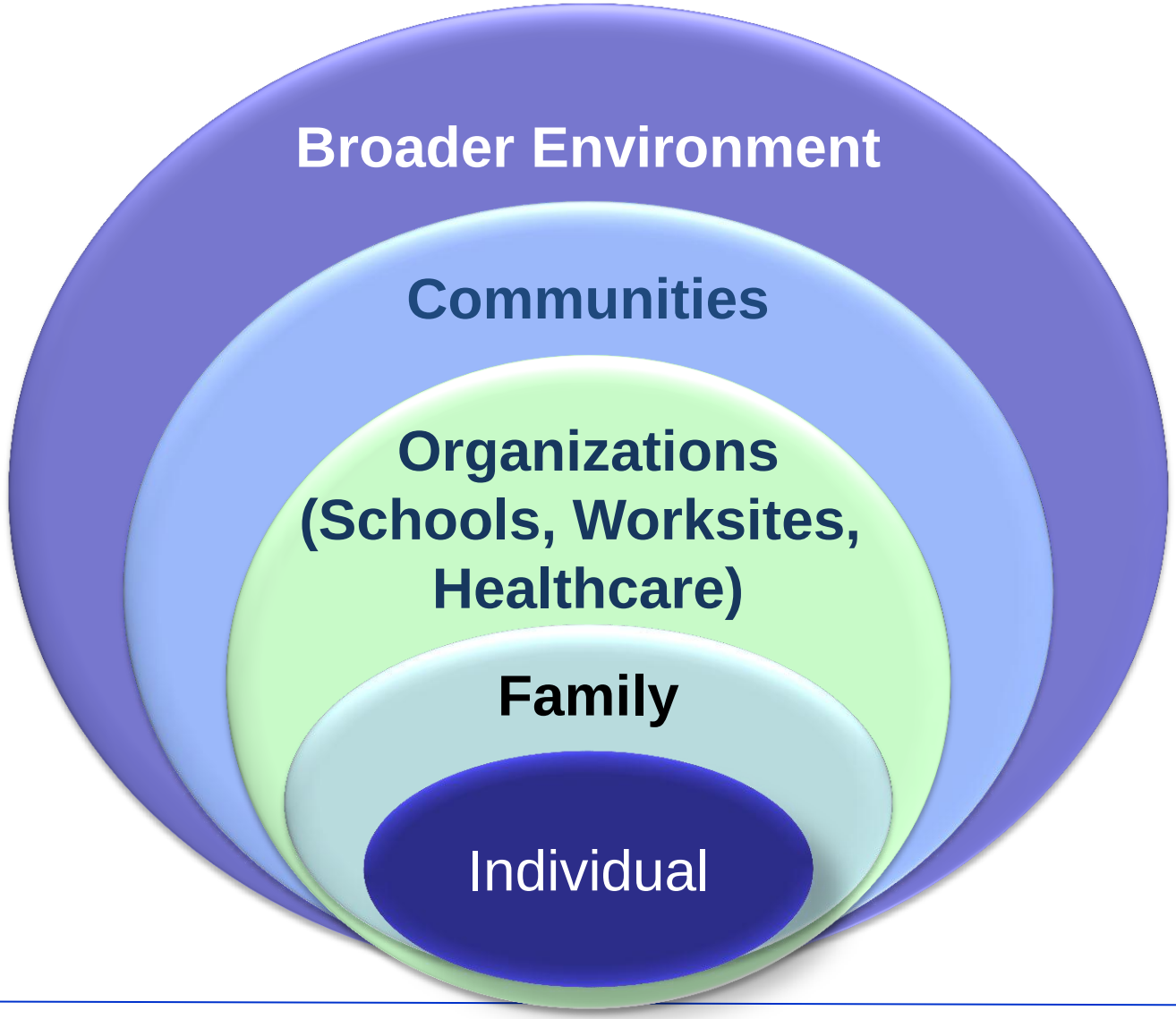
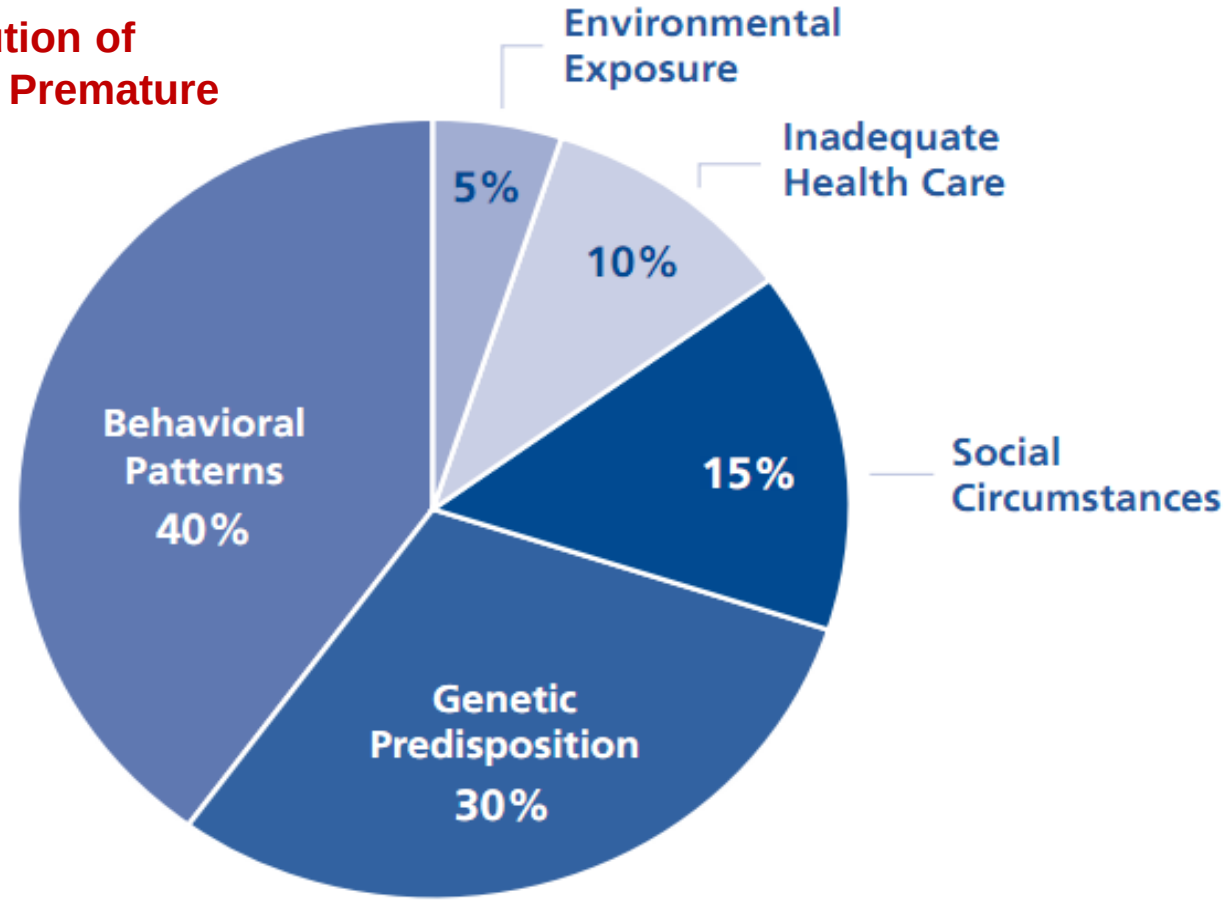
Social-Ecological Model



Contribution of Determinants of Health

Proportional Contribution of Determinants of Health to Premature Death



Source: Steven A. Schroeder, "We Can Do Better-Improving the Health of the American People," New England Journal of Medicine, 327, no 12 (September 20, 2007), p. 1222

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Barriers Identified from Hydroxyurea Conference (2008)

Lack of knowledge about hydroxyurea as a therapeutic option
Difficulty in communication between patients and their caregivers regarding the use of hydroxyurea
Need for frequent monitoring of response to hydroxuyrea
Lack of adherence to treatment regimen
Provider bias and negative attitudes toward patients with sickle cell disease and their treatment
Lack of clarity of hydroxyurea treatment regimens and under treatment in adults
Limited number of physicians who have expertise in the use of hydroxyurea for sickle cell disease
Failure to engage patients /caregivers in treatment decision-making in a developmentally appropriate manner

Expert Panel Recommendation on the Management of Sickle Cell Disease

- 12-Member Multi-disciplinary Panel
  - Family Physician
  - Nurse Practitioner
  - Transfusion Specialist
  - Obstetrics/gynecology
  - Hematologists: Internal Medicine, Pediatric
  - Psychiatrist

- Five Chapters
  - Health Maintenance
  - Management of Acute Complications
  - Management of Chronic Complications
  - Hydroxyurea Therapy
  - Transfusion

Expert Panel Recommendations Focus

- Target audiences:
  - Prime: Primary care clinicians
  - Secondary: Specialty physicians, healthcare systems, policymakers, others

- Goals:
  - Implementable evidence-based recommendations on managing sickle cell disease in clinical practice
  - Identification of gaps in knowledge for additional research

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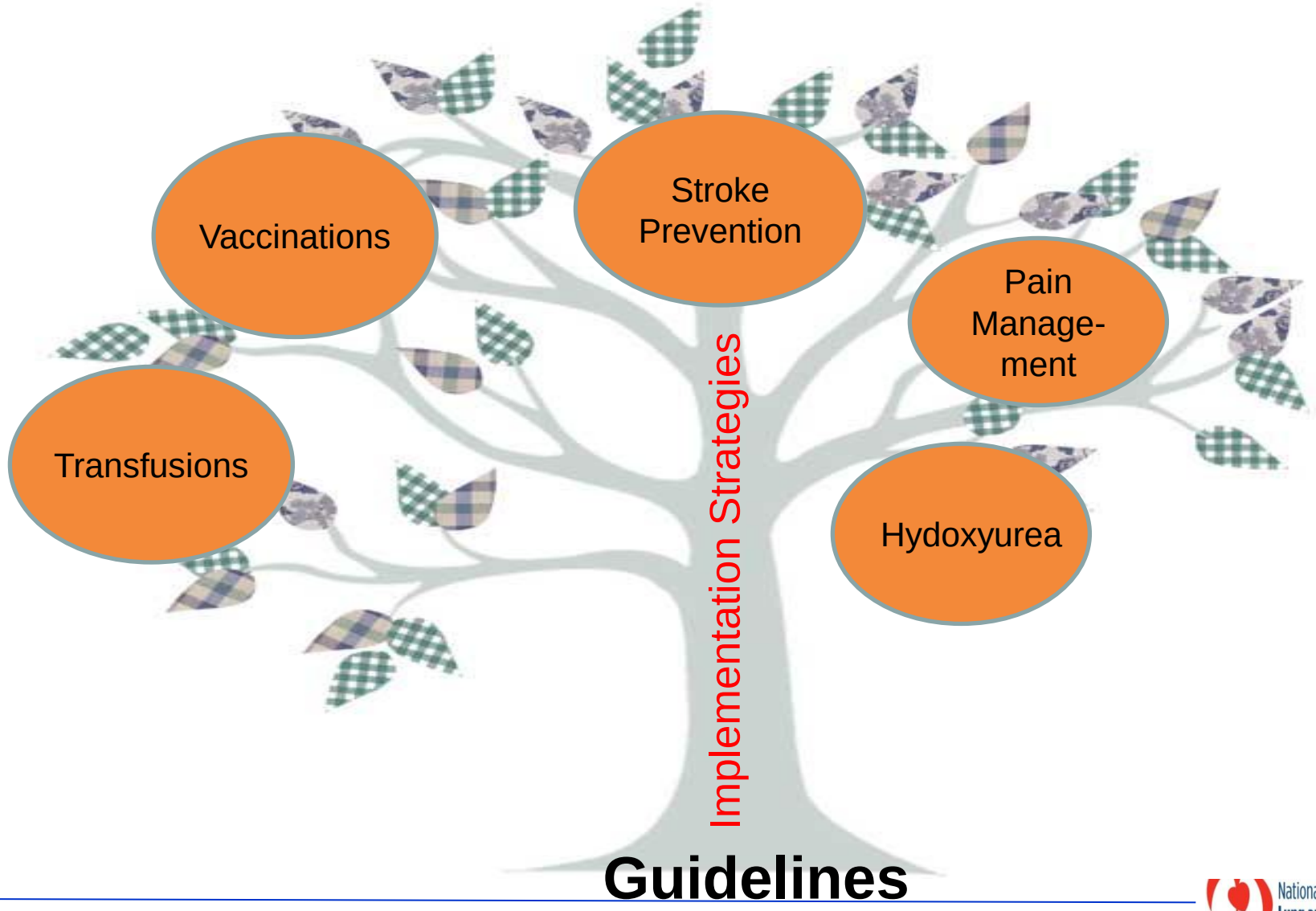
Implementation of Guidelines: A Tree that Bears Fruit



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Health Maintenance

- Includes recommendations for primary care providers and specialists
- Examples of recommendation
  - Vaccinate persons with sickle cell to prevent pneumococcal infections
  - Provide penicillin prophylaxis to children younger than age 5 to prevent sepsis
  - Screen for stroke risk in children with sickle cell disease using Transcranial Doppler Ultrasound (TCD)
  - Provide genetic counseling to potential parents

Acute Complications

- Recommendations primarily target care in the emergency room setting
- In some cases, the recommendation provide guidance but does not provide detail
- For example, the guidelines may state treat pain but does not provide detail about which pain med to use, how often or at which doses
- When available and appropriate, the guidelines uses guidance from other sources, such as the American Pain Society Guidelines
- Examples
  - Acute Pain
  - Acute Chest Syndrome
  - Worsening Anemia

Chronic Complications

- Guidelines attempt to provide recommendations for illnesses throughout the lifespan
- Due to limited resources and time, the guidelines are not meant to provide recommendations on every aspect of sickle cell disease
- Chronic chapter provides recommendations that can be used by the primary care provider the emergency provider and the specialists
- Examples
  - Chronic Pain
  - Renal Complications
  - Pulmonary complications

Hydroxyurea

- Strong evidence that sickle cell patients meeting certain criteria should be started on Hydroxyurea
- Includes recommendations for both primary care providers and specialists
- Includes a protocol for initiating and maintaining hydroxyurea therapy
- Effective use of hydroxyurea will require an approach that gets all stakeholders involved, including the patient, family, primary care providers, emergency room staff, specialists and others

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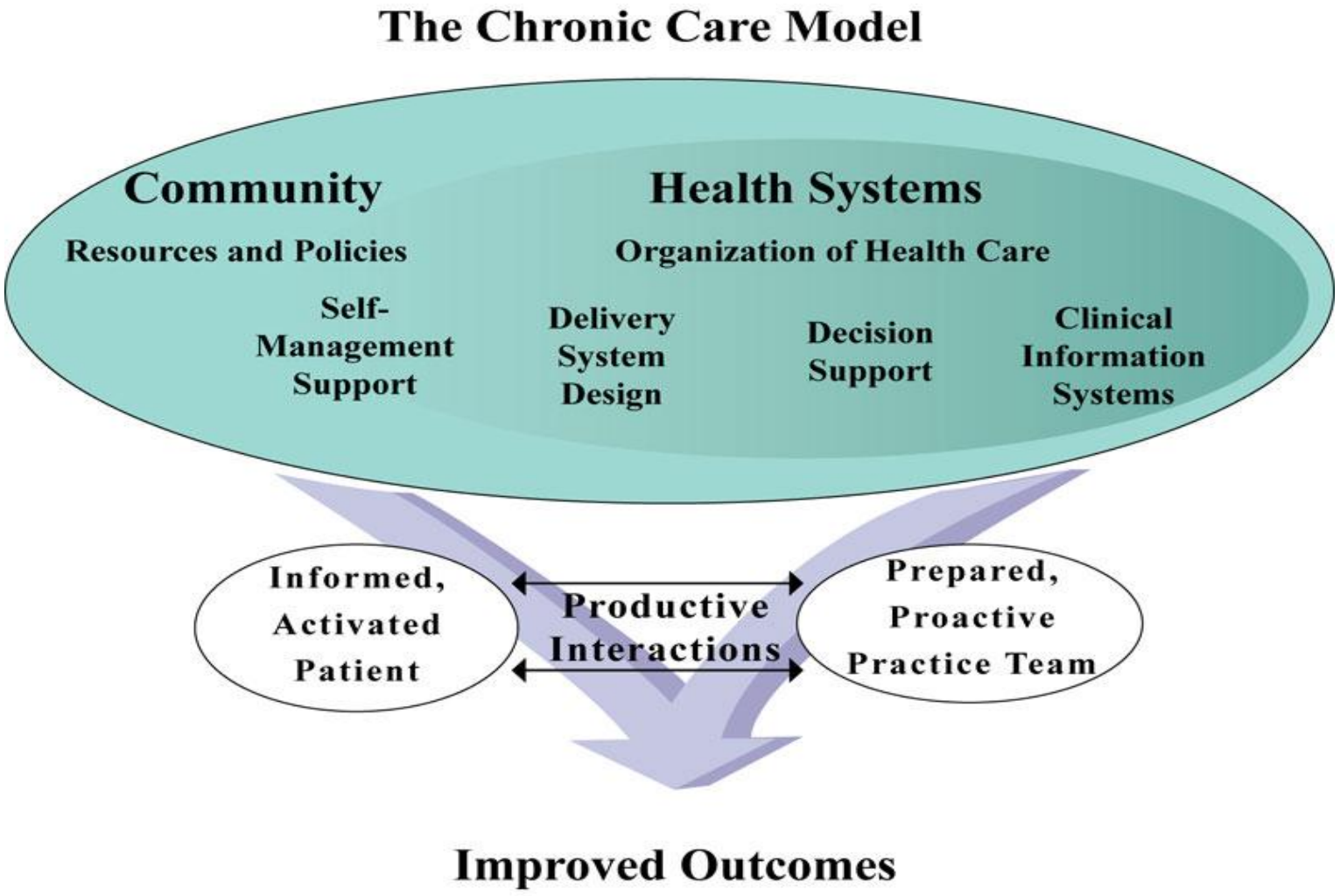
The Chronic Care Model



Developed by The MacColl Institute
® ACP-ASIM Journals and Books

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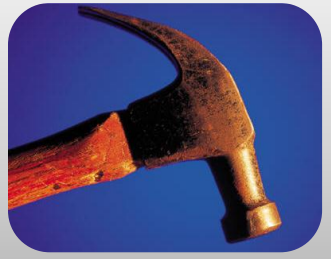
Five Key Elements of Revitalization

Adapted from White House Neighborhood Revitalization Report, July 2011

Resident engagement and community leadership

Investing in and building organizational capacity

Develop strategic and accountable partnerships



Alignment of resources to a unified and targeted impact strategy

Maintaining a results focus supported by data

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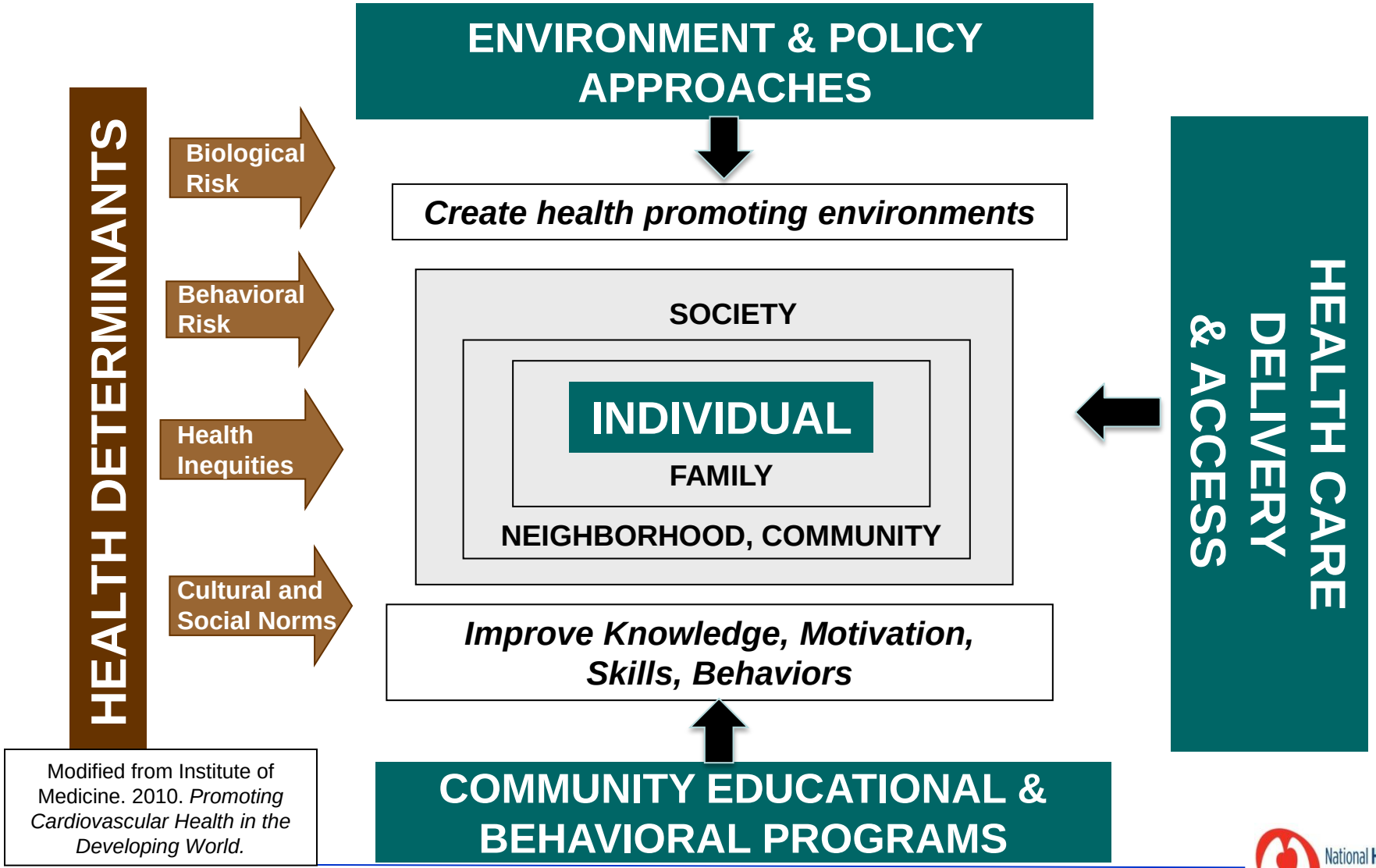
Improving Population Health



Modified from Institute of Medicine. 2010. *Promoting Cardiovascular Health in the Developing World.*

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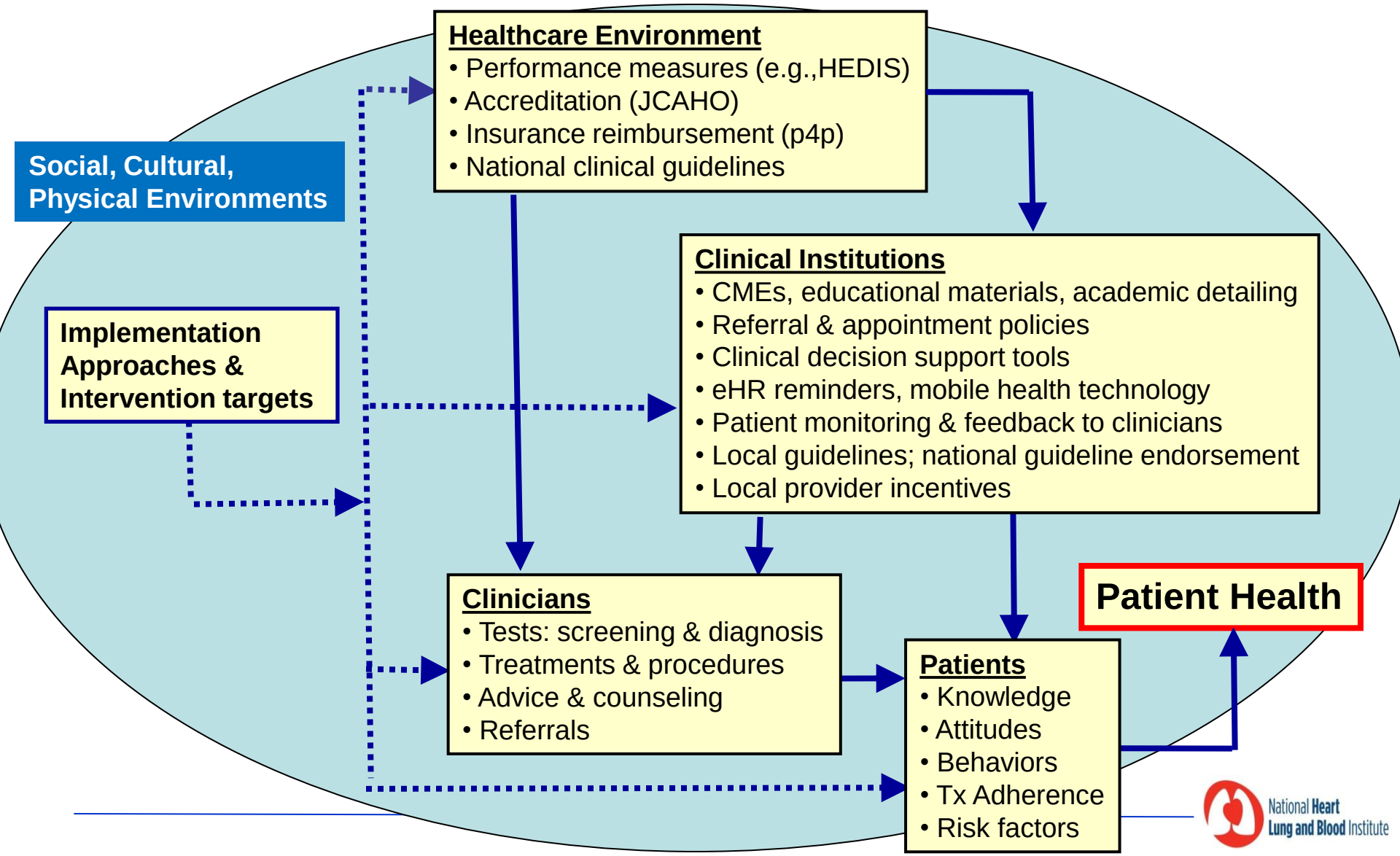
Multi-level Model for Clinical Implementation



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Principles of Patient-Centered Medical Home

- ***Personal physician*** - each patient has an ongoing relationship with a personal physician provide, continuous and comprehensive care.
- ***Physician directed medical practice*** –leads a team at the practice level who take responsibility for the ongoing care of patients.
- ***Whole person orientation*** –personal physician responsible for providing all the patient's health care needs or arranging appropriate care with other qualified professionals. This includes care for all stages of life; acute care; chronic care; preventive services; and end of life care.
- ***Care is coordinated and/or integrated*** across all elements of the complex health care system
- ***Quality and safety*** are hallmarks of the medical home:
- ***Enhanced access*** to care is available through open scheduling, expanded hours and new options for communication
- ***Payment*** appropriately recognizes the added value provided to patients who have a patient-centered medical home.

Adapted from HRSA presentation: HRSA Sickle Cell Disease Program, September 2009

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