



# DEFINING AND MEASURING HEALTH ACROSS THE LIFE COURSE

**Presenters:** Neal Halfon, MD MPH  
Christopher B. Forrest, MD PhD  
Richard Gershon, PhD

Wednesday, August 24, 2011 Natcher Center, NIH  
Bethesda, MD

August 24, 2011

# National Academy of Sciences Review of the NCS Protocol: Critique



“While invoking a dynamic, multifactorial, life-course model, the research plan does not specify any particular model or models to guide decisions on which conditions and potential causal factors are chosen for study, the timing of data collection points, the types of data to be collected, and the overall analytical approach.”

*NAS Review of the National Children’s Study Research Plan (2008)*



# National Academy of Sciences Review of the NCS Protocol: Recommendation



“The NCS should clearly define the key constructs of child health and development and more fully develop a conceptual framework for understanding child health and development over the course of infancy, childhood, and adolescence.”

*NAS Review of the National Children's Study Research Plan (2008)*



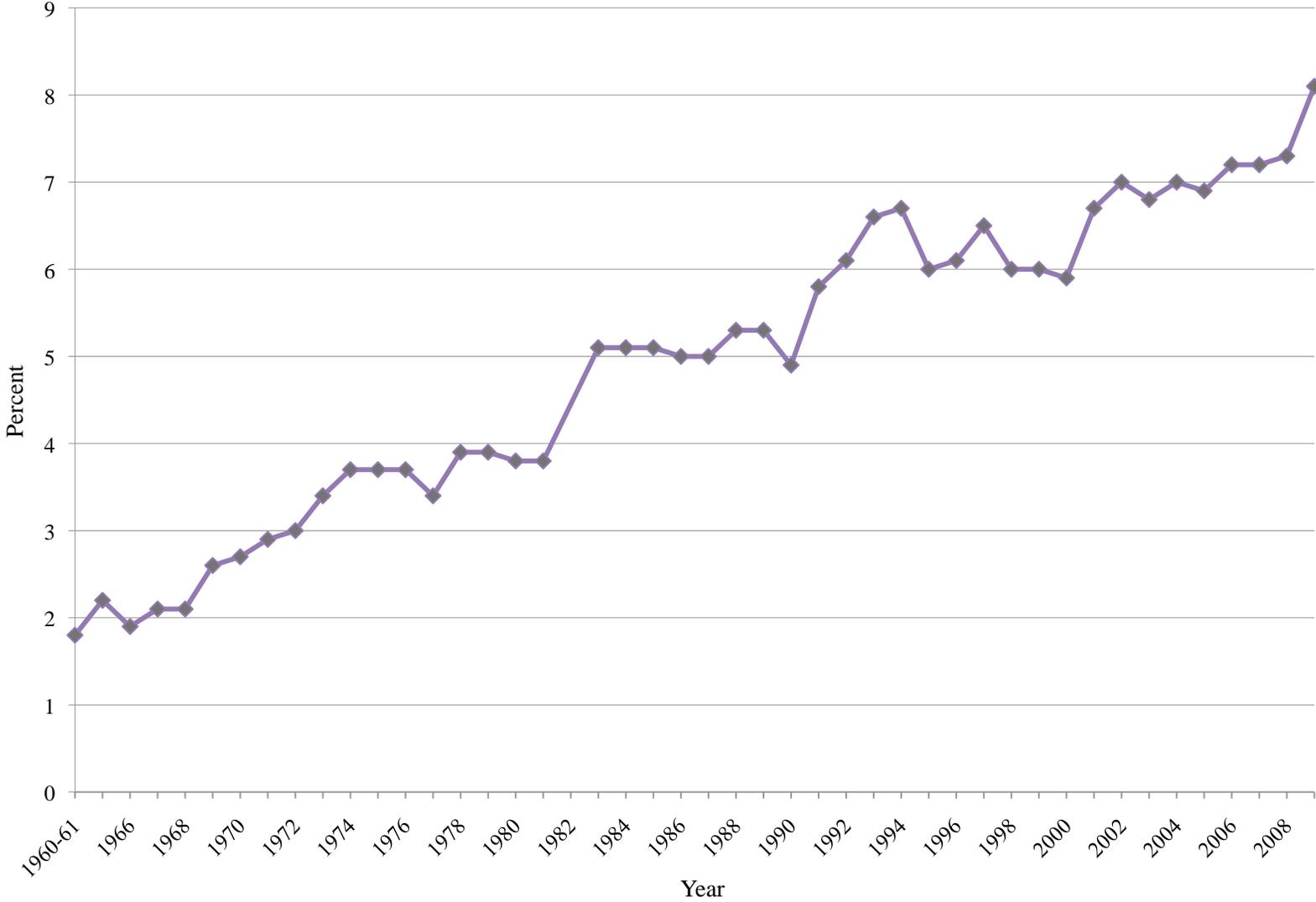
# Trends in Child Health



- Mortality rates continue to decrease
- Disability and rates of disparities have increased
- Emergence of new morbidities and concerns
- Patterns of exposure and risk are continuously changing
- Compared to other nations the US does not look so good



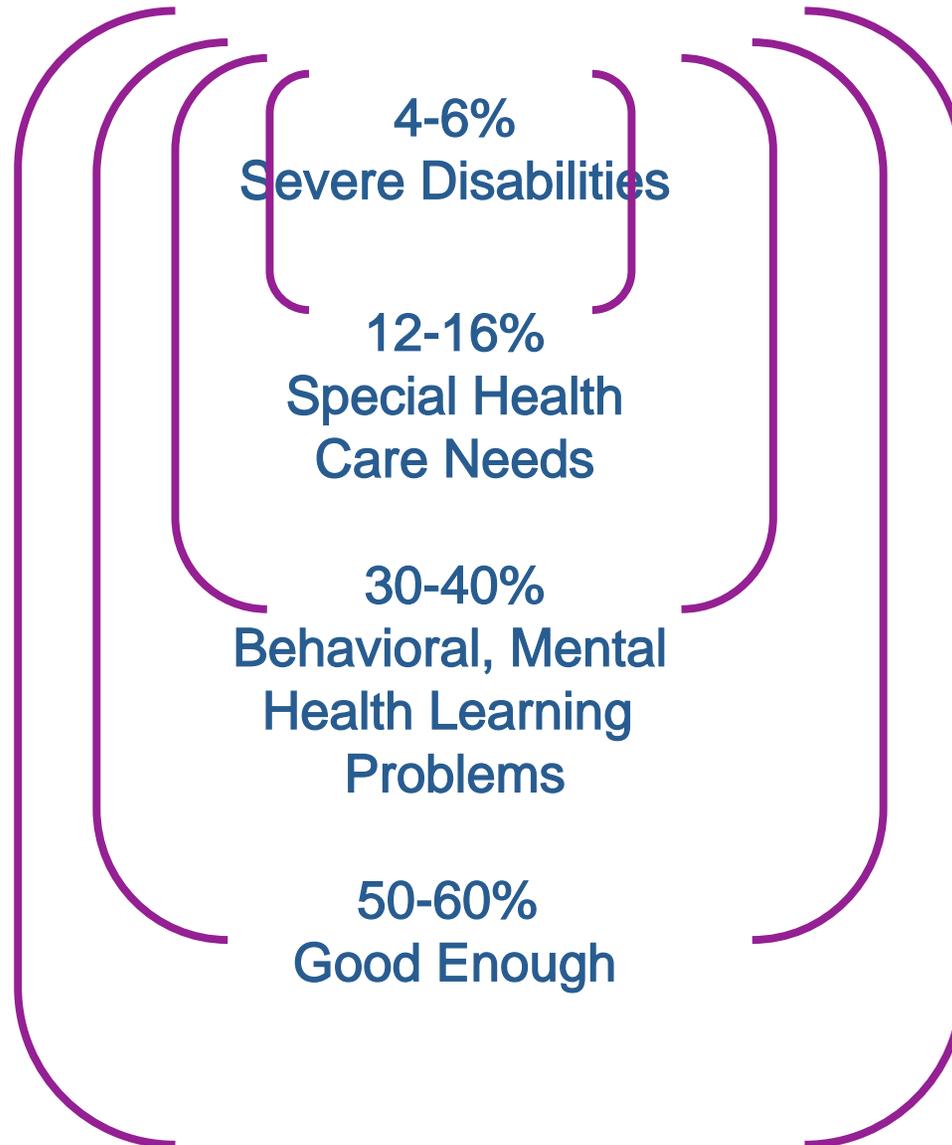
# Trends in Limitation in Activity due to a Chronic Condition US Children 1960-2009 ( NHIS)





August 24, 2011

# Health of US Children



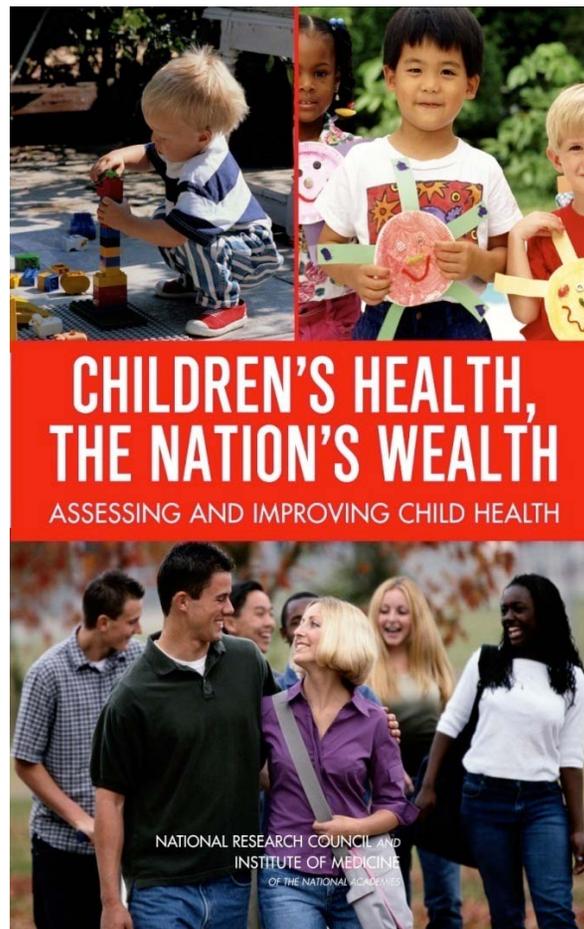
What % are thriving ?

30% ?

40% ?

50% ?

# 2004 National Research Council and Institute of Medicine Report

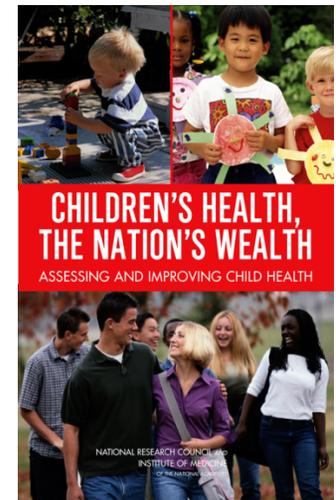


# Recommendation: Adopt a Broader Definition of Children's Health



Children's health should be defined as the extent to which individual children or groups of children are able or enabled to

- a) **Develop** and realize their **potential**,
- b) **Satisfy** their needs, and
- c) **Develop** the **capacities** that allow them to **interact successfully** with their biological, physical, and social environments.



# Three Dimensions of Health



- **Health conditions:** disorders or illnesses
- **Function:** execution of tasks and participation in desired activities
- **Health potential:** development of assets and positive aspects of health, such as competence, capacity, and developmental potential



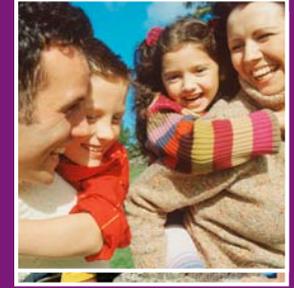
# Life Course Health Development



- Health development
  - Health Development occurs across the life course;
  - Can be represented by health trajectories; and
  - Includes critical and sensitive periods when both toxic social environments and adverse experience as well as positive influences are likely to “get under the skin” and become embedded in to the biology and brain of the developing child

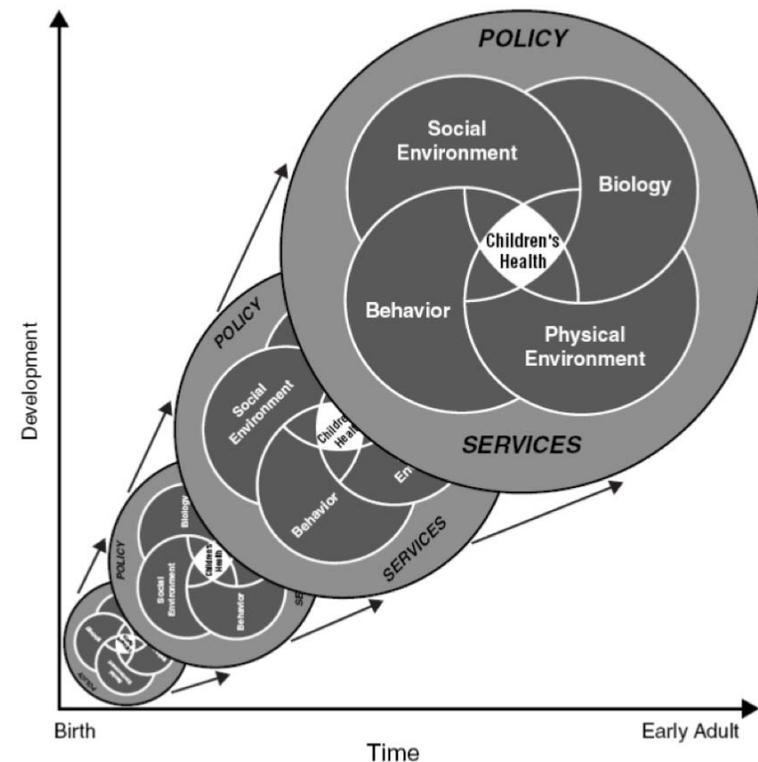


# A Dynamic Developmental Model Of Children's Health And Its Influences



## Health and development

- Do not occur randomly;
- Are affected by the prior patterns of adaption; and
- Can be represented as a kaleidoscope, where future outcomes are determined by earlier configurations.



# Challenges in Measuring Health Potential



- Conceptual frameworks poorly developed
- Developmental modulation is difficult to capture and account for
- Existing measures have ceiling effect
- Domain definitions - Reductionist legacy
  - Mechanistic models of body function
  - Capacity-based concepts
- Measurement also involves other unanticipated challenges



# NCS Opportunity



- Evolution of conceptual frameworks
  - Resilience, responsiveness
  - Reserves
- Observational, Clinical, Biomarkers
- Measurement of epigenetic mechanisms of developmental modulation
- Existence of PROMIS, TOOLBOX, PhenX and other NIH measurement initiatives
- Worldwide development of longitudinal cohort studies & measures



# Urgency in Developing Measures of Health Potential



- Study needs strong 21<sup>st</sup> Century conceptual and measurement schema
  - Measurement schema will provide strong functional scaffolding for the study
- Data collection is about to commence
- Relevance of the NCS to evolving paradigms of health and health care delivery are important to demonstrate



# An NCS Measurement Development Learning System



- Need for NCS concepts and measures to continuously evolve in response to changing science, evidence, constructs, and needs of the Study;
  - Strategic
  - Responsive
- Don't need to plan all the measures now but need to have a *active* **Measurement Development Learning System** to assure that measures can continuously evolve, and be updated and upgraded; and
- Will capitalize upon the enormous capacity within and across the sites



# Leveraging Existing NIH Measurement Projects



Two key NIH Measurement projects will be highlighted:

- PROMIS: Patient Reported Outcome Measurement System  
Christopher Forrest, MD PhD
- TOOLBOX: Assessment of Neurological and Behavioral Function  
Richard Gershon, PhD



# Part 2



## NCS Health Measurement Project & Connections with the NIH PROMIS Initiative

Christopher B. Forrest, MD PhD  
Children's Hospital of Philadelphia  
University of Pennsylvania School of Medicine



For more information:  
[www.nihpromis.org](http://www.nihpromis.org)  
forrestc@email.chop.edu



# Currently Used Measures of Health



- Vital Statistics
- Communicable disease rates
- Biochemical assays – “lab work”
- Functional tests of capacity
- Surveys
  - Limitations in functioning
  - Risk behaviors
  - Perceived health



# Where is the science of “health” measurement?



- 1) Health ranges from poor--excellent
- 2) Health is a biologically based
- 3) Biological measures focus on poor extreme
- 4) Need better assessments of the positive end and better conceptual models of what we mean by health



# Child Health Measurement Must Address:



- Health as a positive construct | health potential
- Sensitive periods when environment may have large impacts on health
- Multiple influences that interact over time in different ways as children develop and change
- Multi-level measurement: biological, self, and environment



# NCS Health Measurement Network (HMN) : Purpose



Design and test new concepts and measures of child health and augment the existing NCS measurement approach



# The NCS Health Measurement Network: Now to Sept 2012



## Aim 1: Develop an Organizational Architecture for the NCS-Health Measurement Network

- Use principles of open-science collaboration
- Community of scholars interested in advancing science of health measurement

## Aim 2: Select and Test Existing Self-report Measures in a Population Sample of Pregnant Women

- E.g., PROMIS & NIH Toolbox measures
- Rapid cycle proof of concept



# The NCS Health Measurement Network: Now to Sept 2012



## Aim 3: Develop Theoretical and Measurement Models of Preconception/Prenatal Health and Child Health

- To drive measurement innovation and hypothesis testing
- To generate typologies of health domains for consensus-based prioritization by NCS scientists

## Aim 4: Select Preconception/Prenatal and Child Health Measures for Prioritized Health Domains

- Define selection criteria
- Identify domains needing new measures vs. modification of existing measures



# The NCS Health Measurement Network: Oct 2012 and Beyond



**Aim 5: Validate, modify and/or develop tools and instruments for selected measures and begin longer term measurement innovation work**

- Instrument Development Teams
- Measurement Innovation Team

**Aim 6: Field Test Health Measures, Assess their Validity and Develop Integrated Summary Scores and Health Profiles**

- For use in NCS and other research settings as well as for clinical purposes



# Guiding Principles



1. Concepts and measures augment existing NCS work
2. Health is 4-dimensional including a whole-person orientation, continuity, and change over the life course
3. Health status results from the interplay of environment and one's health assets and health challenges
4. Multi-modal measurement: Health needs to be measured using multiple modalities—e.g., biological assays, device-enabled assessments, observation, and self-report
5. NCS HMN will employ open-science methods to foster collaboration, transparency, and sharing



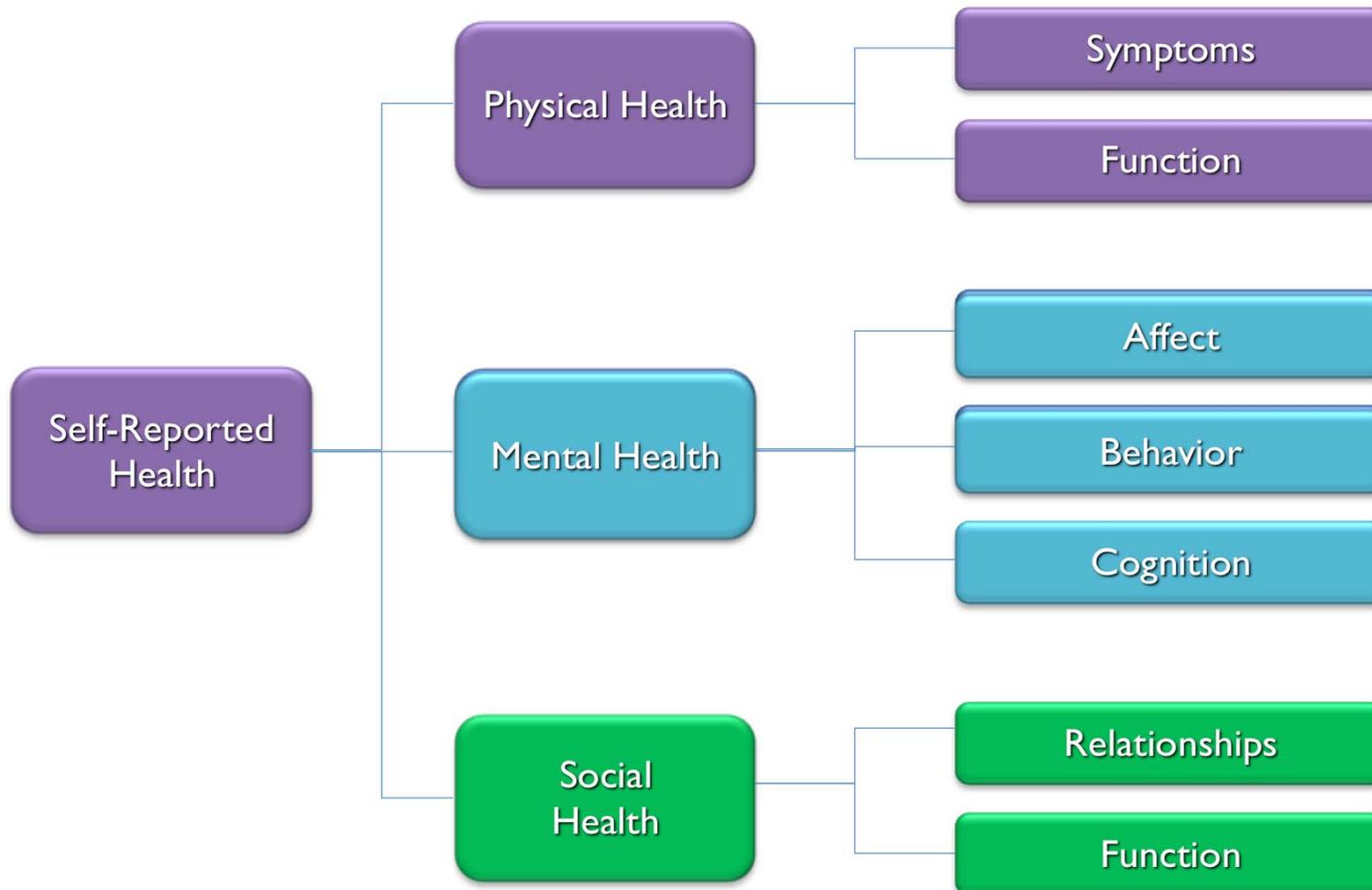
# NIH Patient Reported Outcome Measurement System (PROMIS®)

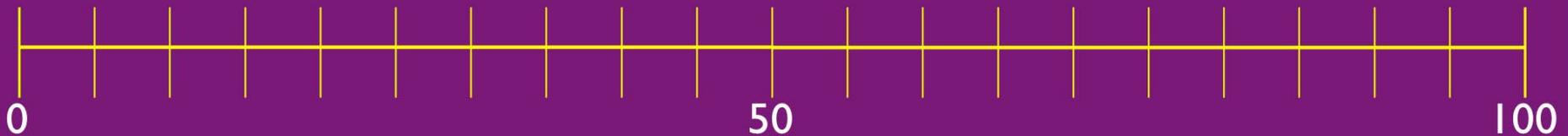
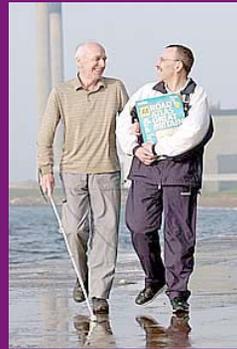


- Goal: to provide clinicians and researchers access to efficient, precise, valid, and responsive *adult- and child-reported* measures of health.
- Originally an NIH Road-Map Initiative; Funding now comes from the NIH Common Fund with representation across most NIH Institutes → \$70M investment since 2004

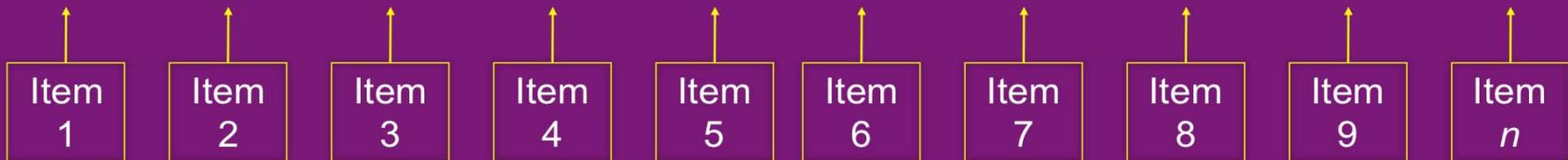


# PROMIS Health Framework





## Physical Functioning Item Bank



Are you able to get in and out of bed?

Are you able to stand without losing your balance for 1 minute?

Are you able to walk from one room to another?

Are you able to walk a block on flat ground?

Are you able to run or jog for two miles?

Are you able to run five miles?



# Pediatric PROMIS Item Banks



## Available Now

1. Physical functioning/mobility
2. Physical functioning/upper extremity
3. Pain interference
4. Fatigue
5. Depressive symptoms
6. Anxiety
7. Anger
8. Peer relationships
9. Asthma impact

## Available 2012

1. Pain behavior
2. Pain quality
3. Physical activity/mobility
4. Physical activity/sedentary
5. Stress experiences
6. Physical distress
7. Life satisfaction
8. Positive affect
9. Meaning and purpose
10. Family belonging
11. Family involvement



# Validation of Self-Reported Maternal Health Measures Study



- Formed an NCS team of maternal health experts
- Rapid cycle development of a working definition
- Leverage major NIH investments in PROMIS and Toolbox
- Selected maternal health measures that operationalize maternal health definition
- Field test measures in the Fall, 2011



# Maternal Health Profile: Selected Measures from PROMIS and Toolbox



- Physical Health
  - Physical function
  - Fatigue
  - Sleep disturbance
  - Sleep-related impairment
- Mental Health
  - Anger
  - Anxiety
  - Depression
  - Positive affect
  - Perceived stress
  - Self efficacy
- Social Health
  - Social support and companionship
  - Social isolation



# Maternal Self-Reported Health Field Test: Fall, 2011



Battery to be field tested in an online sample of 1000 women

- 200 pre-conceptual
- 150 pregnant women (50 per trimester)
- 650 with a child between 0-36 months of age)



# Part 3



## Integration of the NIH Toolbox Initiative into the NCS Health Measurement Project

Richard Gershon, PhD  
Greater Chicago Study Center  
Northwestern University



For more information:  
[www.nihtoolbox.org](http://www.nihtoolbox.org)  
gershon@northwestern.edu

*This study is funded in whole or in part with Federal funds from the Blueprint for Neuroscience Research,  
National Institutes of Health under Contract No. HHS-N-260-2006-00007-C*



# NIH Toolbox Goals



- Develop unified/integrated measures (assessment tool) of multiple indicators (cognitive, emotional, motor, sensory) of neural and behavioral health functioning for use in large cohort studies and clinical trials
- Could be used as a form of “common currency” across diverse study designs and populations
- Would maximize yield from large, expensive studies with minimal increment in subject burden and cost



# Sponsorship



- Funding is provided by the 15 NIH agencies which make up the NIH Neuroscience Blueprint



# Toolbox Development



- Established a rich collaborative network
  - 242 named personnel
  - 126 consultants
  - 19 subcontracts
- Released Request for Information (152 responses) and conducted Expert Interviews (44 interviews)
  - Nominated and rank sub-domains and constructs
  - Determined instrument cost and time constraints
- Conducted literature review
  - Developed library of 1,391 instruments based on review and expert interviews



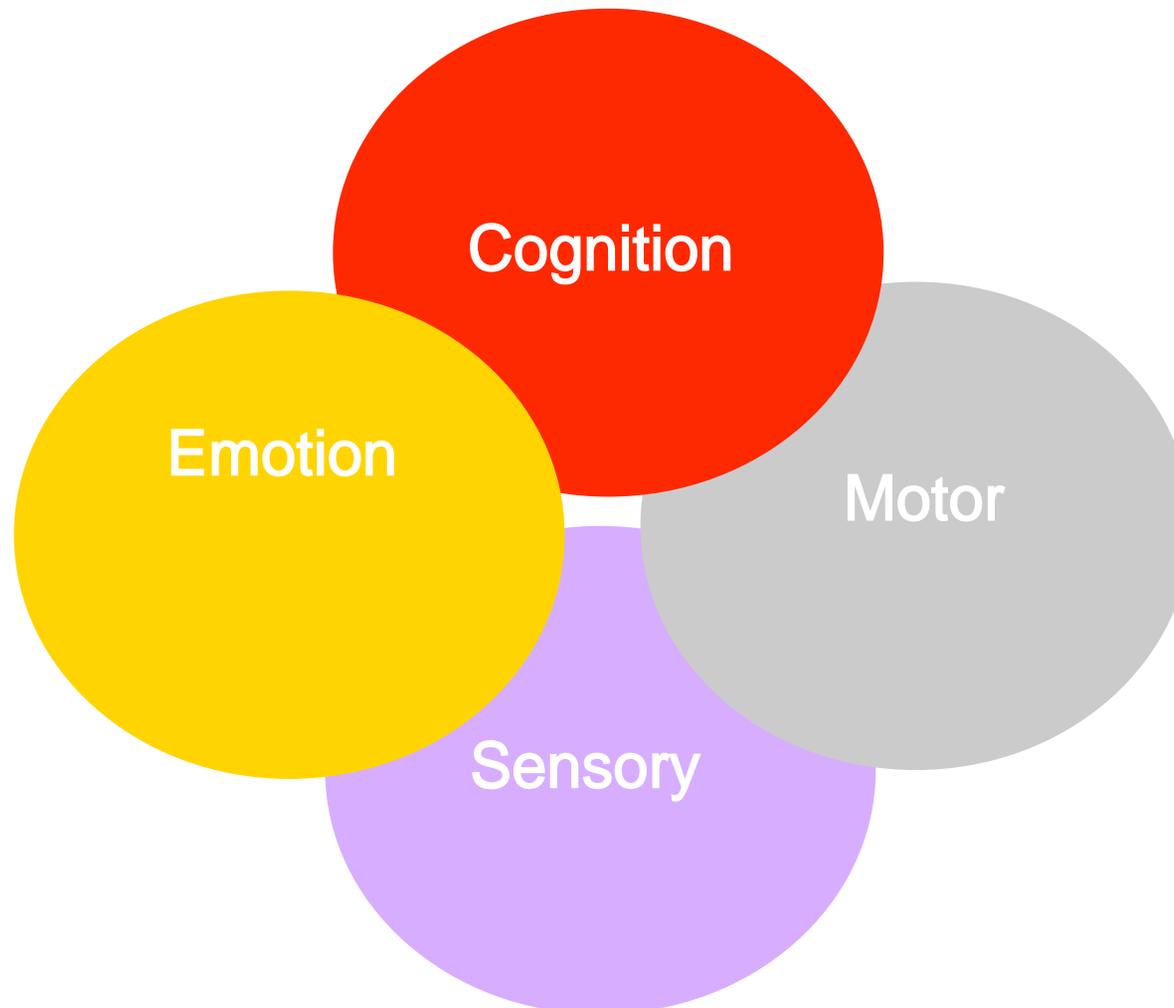
# Selection Criteria



- Applicable across the age span
- No intellectual property concerns
- Psychometrically sound
- Brief, easy to use
- Applicable in a variety of settings and with different subgroups
- Preference for instruments already validated and normed for ages 3 – 85 years



# Toolbox Domains



# Cognition Domain Components



**Executive  
Function**  
*Inhibitory Control  
Cognitive Flexibility*

**Episodic  
Memory**

**Language**  
*Vocabulary Comprehension  
Reading Decoding*

**Attention**

**Working Memory**

**Processing Speed**



# Language



*A set of mental processes that serve to translate thought into symbols (words, gestures) that can be shared among individuals for purposes of communication*

## Toolbox Picture Vocabulary Test

- ✗ The respondent hears the word, is presented with four images on screen, and must select the image that most closely represents the meaning of the word.
- ✗ Administered in computer-adaptive format.
- ✗ Initial item difficulty based on age/education.
- ✗ IRT-based ability score provided.



# Toolbox Picture Vocabulary Test



Motor  
Subdomains  
Components



Endurance

Locomotion

Strength

Dexterity

Balance

*Upper Extremity*  
*Lower Extremity*

*(Non-vestibular)*



# Sensation

Domains  
Components



## Olfaction

*Odor Identification*

## Taste

*Preference  
Intensity  
Bitter Perception  
PROP Sensitivity*

## Vestibular Balance

*Vestibular-ocular Reflex  
Vestibulospinal Function*

## Audition

*Hearing Threshold  
Speech Processing  
Hearing Loss  
Middle Ear Function*

## Somatosensation

*Texture Discrimination  
Proprioception  
Pain  
Touch Discrimination  
Touch Perception*

## Vision

*Visual Acuity  
Visual Function HRQOL  
Peripheral Vision*



# Emotional Health Subdomains Components



## Positive Affect

*Happiness  
Life Satisfaction  
Well-Being*

## Negative Affect

*Sadness  
Fear  
Anger  
General Distress  
Apathy*

## Stress and Coping

*Perceived Stress  
Coping Strategy  
Coping Self-Efficacy*

## Social Relationships

*Social Support  
Social Network  
Integration  
Loneliness*

Self Report: Ages 8-85; Parent Proxy Version 3-12



# The Process Continues...



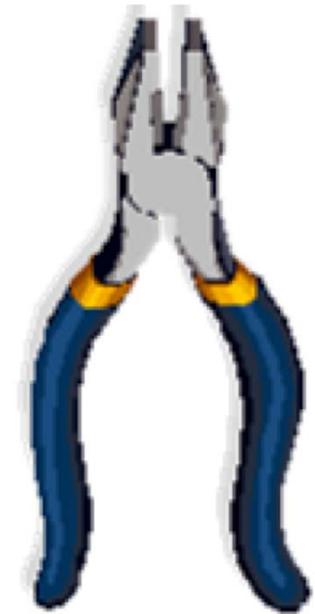
- Instrument development
- Pre-testing and piloting of new items and instruments
- Concurrent validation of new instruments with industry “Gold Standards” (N=17,000)
- Validation in clinical populations
- Norming in national general population sample



# 47 Assessments Being Tested



- Technician assisted measures
- Equipment based measures
- Computerized Adaptive Testing (CAT)
- Short Forms



# Toolbox Norming Aug-Nov 2011



- 3,500 Children in single year age bands (3-17 years)
- 1,000 adults in 7 age-bands, including mothers of children also being tested
- 100 pregnant women
- Norming has been jointly sponsored by the NCS.
  - The sampling plan accounts for appropriate race/ethnicity and level of education for each age band.



# Interim Accrual Report



- Data collection started August 11, 2011
- Appointments have been set for 3,000 subjects
- Data collection has been completed for 450 subjects



# Vision for Final Toolbox



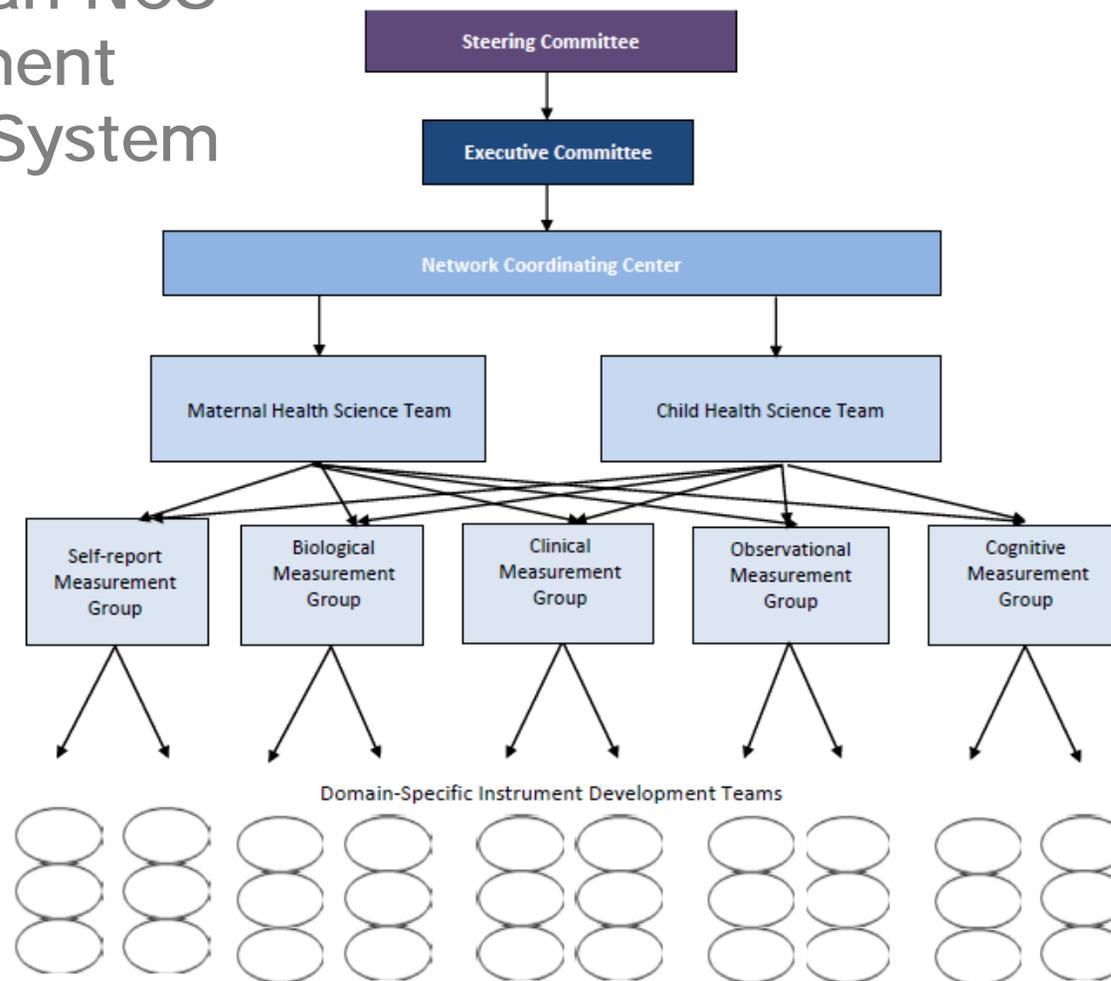
- Four domain-level batteries
- Domain batteries to take no more than 30 min to administer; entire Toolbox administration max of 2 hours (40 minutes for 3-7 year olds)
- English and Spanish versions
- Supplemental set of additional instruments



# Next Steps : NCS HMN



- Establish an NCS Measurement Learning System



# How To Get Involved?



- Attend breakout session on Friday, August 26<sup>th</sup>
- Keep a lookout for announcement in NCS Newsletter or a webpage;
- Send comments, suggestions, recommendation to:

[wnicholas@mednet.ucla.edu](mailto:wnicholas@mednet.ucla.edu)

