HRSA
Health Resources & Services Administration

Blueprint for Change
Implications for Newborn Screening

ACHDNC Meeting
May 4, 2023

Jeffrey P. Brosco, MD PhD
Director, Division of Services for Children with Special Health Needs (DSCSHN)
Maternal and Child Health Bureau (MCHB)

Vision: Healthy Communities, Healthy People

Outline

- CYSHCN and MCHB/HRSA
  - CYSHCN = Children and Youth with Special Health Care Needs
- What is the *Blueprint for Change?*
  - "Measure What Matters"
- What does *Blueprint* mean for NBS?
  - Fulfilling the public health promise of NBS
  - A vision for how data can support improved outcomes for CYSCHN and their families, especially those identified through state NBS programs

It's (Past) Time to Prioritize Sickle Cell Disease

1. Limited guideline implementation

-TCD screening: 47% of 2-9 y/o and 38% of 10-16 y/o (2019)

-Hydroxyurea: 38% of 2-9 y/o and 53% of 10-16 y/o (2019)

2. Lacking national healthcare infrastructure

-Data on epidemiology, health care use, access to care, outcomes that matter

-National quality improvement measure like TCD screening

-Barriers to prescribing HU and other clinical guidelines

-Unconscious bias, systematic and interpersonal racism

3. Strategies to address inequities

-Specialty provider SCD training (APPs)

-Reimbursement for hospital to recoup costs for providing comprehensive quality care

-Provider workshops on diversity and equity

Hsu LL, Hooper W. C, Schieve LA. Prioritizing Sickle Cell Disease. Pediatrics.2022; 150(6):e2022059491

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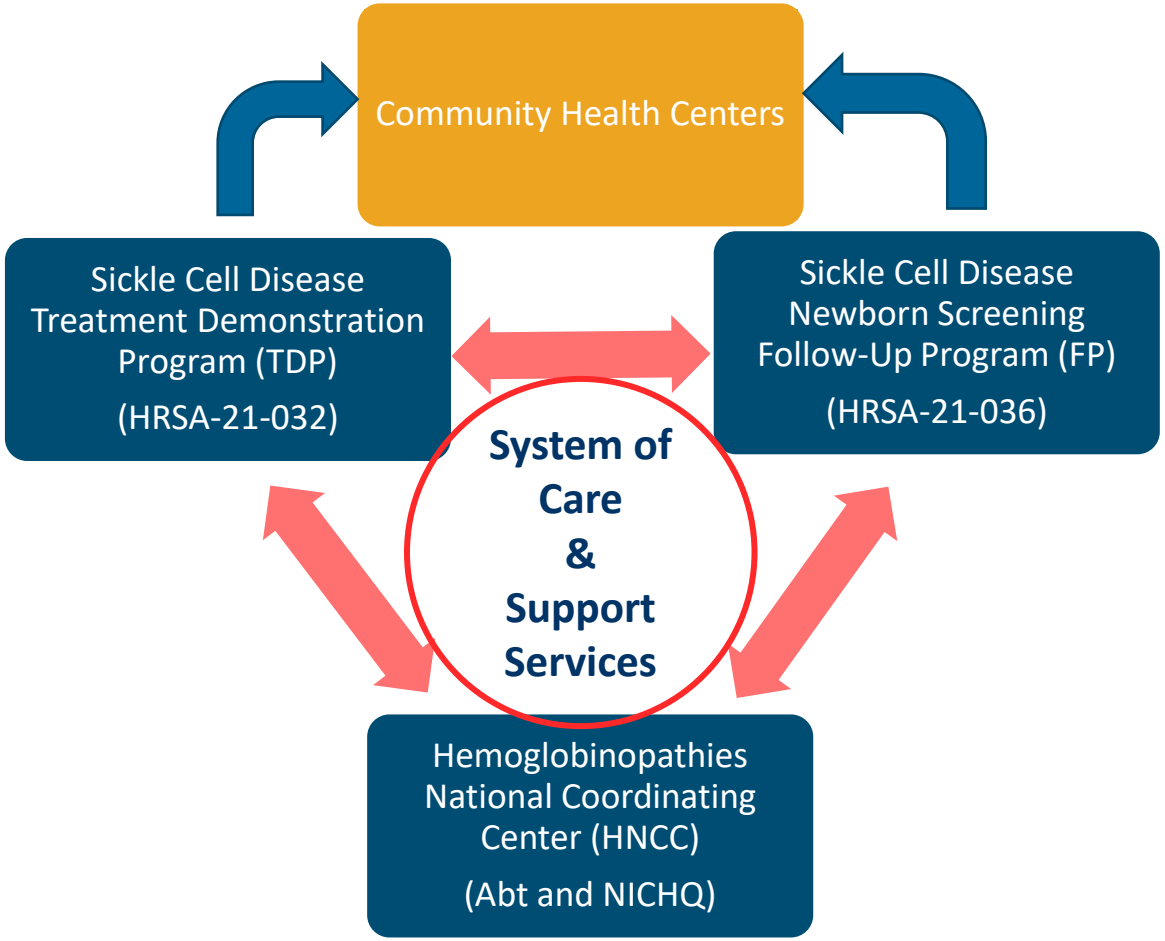
HRSA SCD: Bridging Between Communities and Clinicians



Strengthen the **system of care** and **support services:**

- Educating patients, families, and clinicians (ex: SAPPORT program)
- Linking individuals and families to evidence-based care
- Partnerships between clinicians, community organizations, and other stakeholders

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Children and Youth with Special Health Care Needs

Allergies	9
Asthma	8
Diabetes	0.1
Sickle cell	0.1
Child cancers	0.02
Liver transplant	0.0004

13,000 more (rare) conditions

Learning disability	8.2
ADHD	7.5
Depression	3.3
Autism	2.8
Intellectual disability	1.5
Deaf/Hard of Hearing	0.4
Visual loss	0.4
Cerebral Palsy	0.3
Down Syndrome	0.15

(Chronic conditions per 100)

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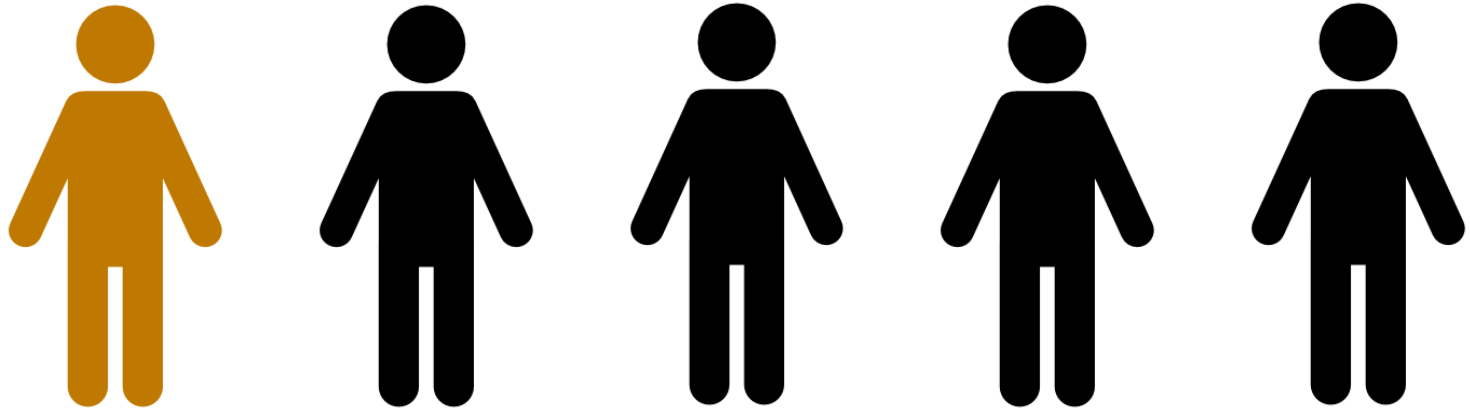
Director, Children and Youth with Special Health Care Needs

Who are CYSHCN?

Children or youth ***who have or are at increased risk for*** chronic physical, developmental, behavioral, or emotional conditions and who also require health and related services of a type or amount beyond that required for children generally.



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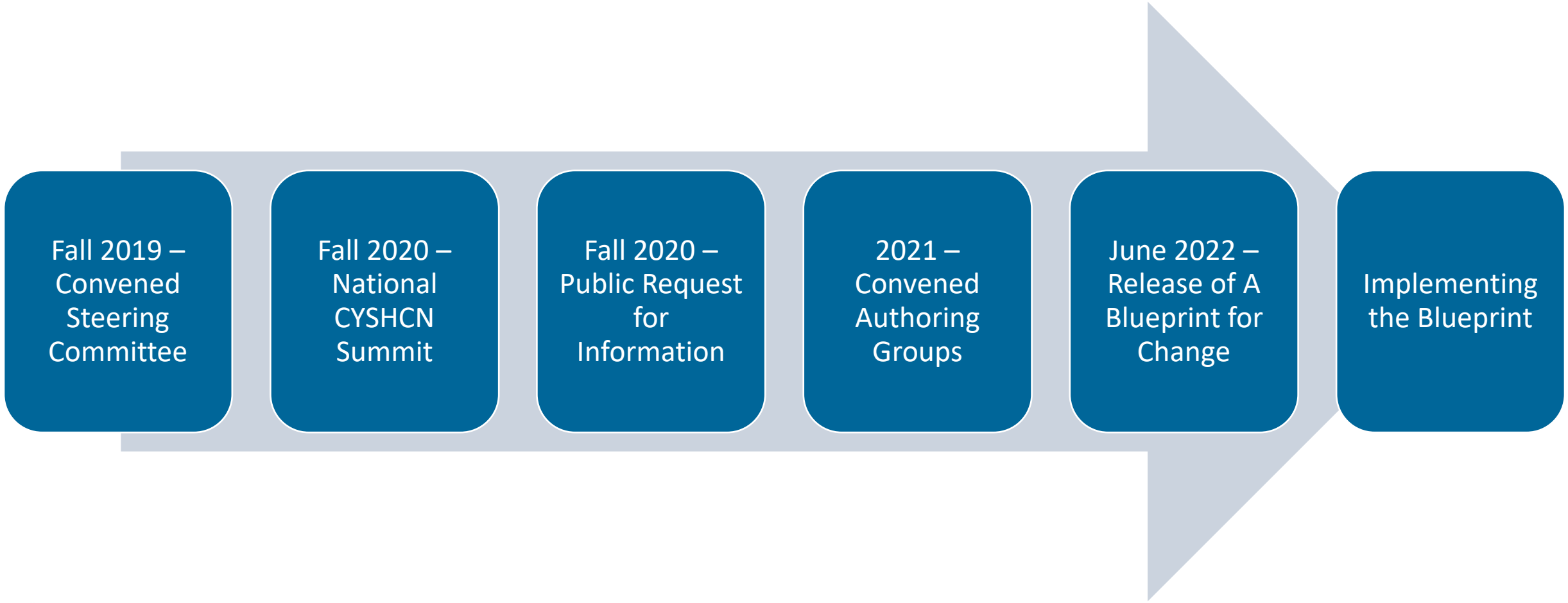
Development of the *Blueprint for Change*



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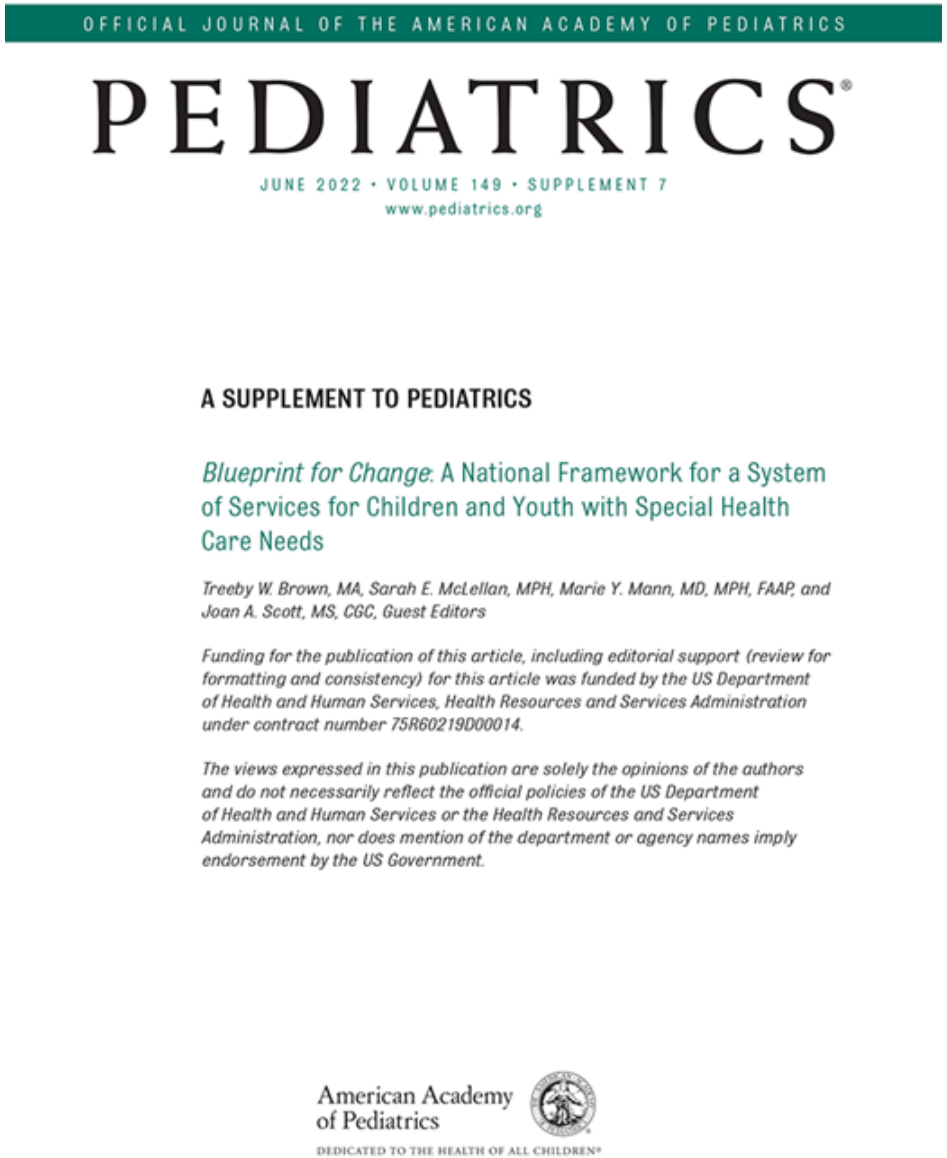
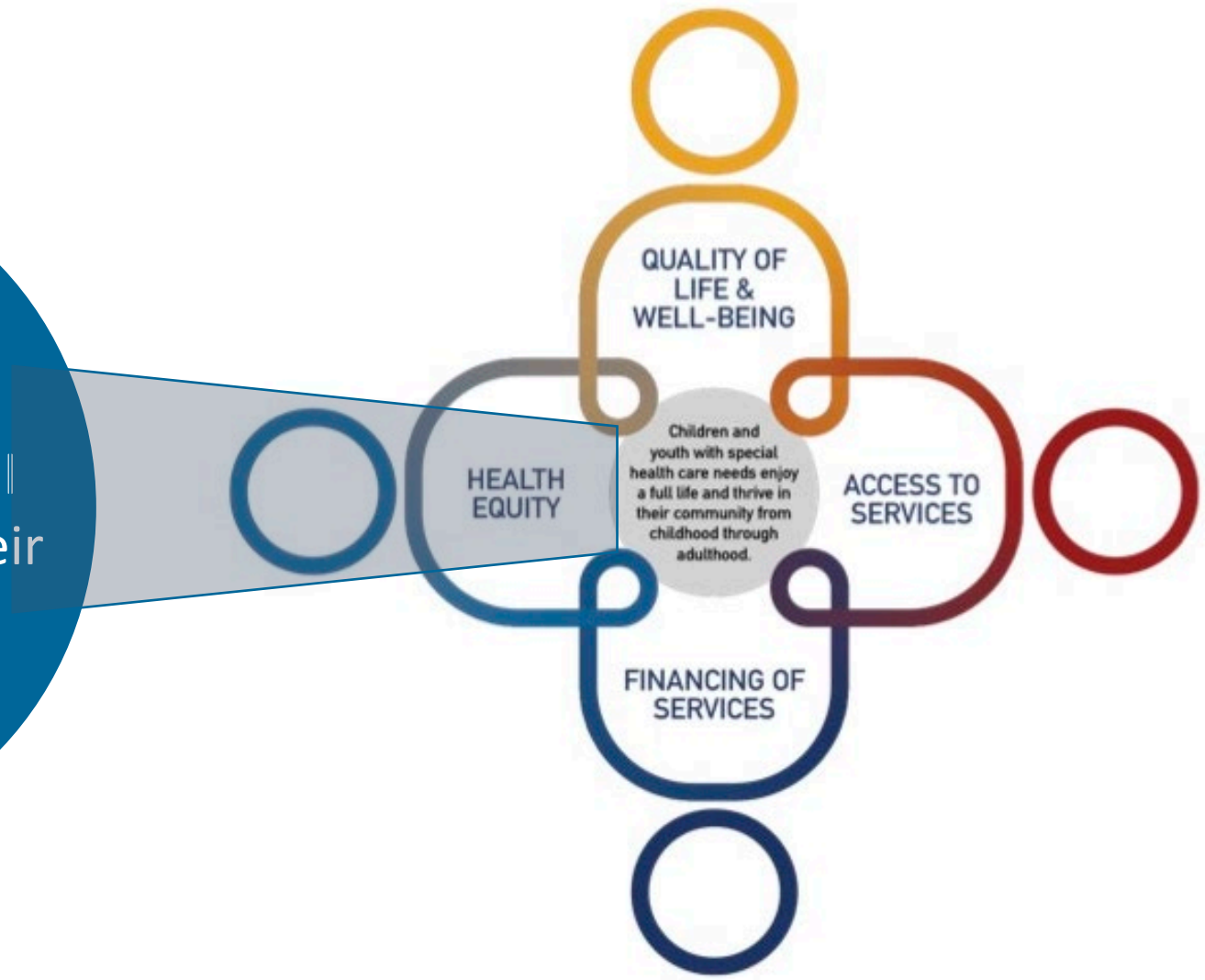
ARTICLES

https://publications.aap.org/pediatrics/issue/149/Supplement%207



MCHB *Blueprint for Change for CYSHCN*

Children and youth with special health care needs enjoy full lives and thrive in their communities from childhood through adulthood



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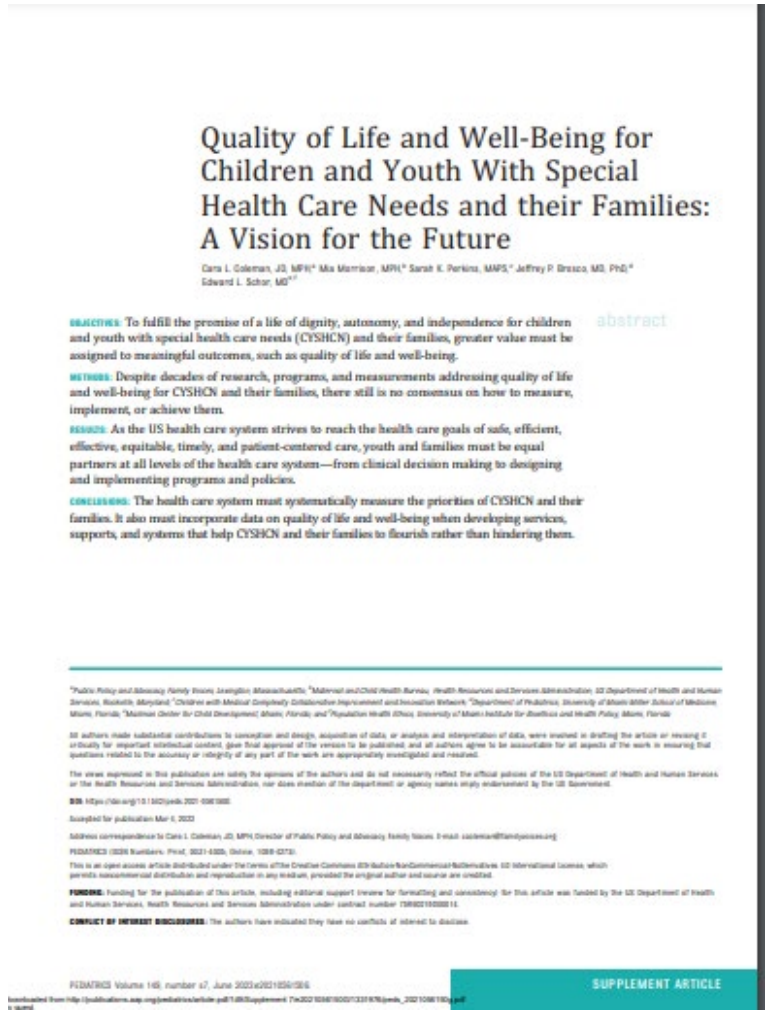
What's New in the Blueprint? Quality and Equity

- QOL: Child and caregiver well-being
  - What families tell us really matters
  - Children thrive when caregivers are healthy
  - Appropriate measures/outcomes point the system in the right direction, even if imperfect
- EQUITY: Every child is thriving
  - Fair and equitable outcomes
  - One approach: "targeted universalism"
  - Ensure that historically underserved and/or marginalized populations have equitable outcomes



Quality of Life and Well-Being for Children and Youth With Special Health Care Needs and their Families: A Vision for the Future

Cara L. Coleman, JD, MPH,[a] Mia Morrison, MPH,[b] Sarah K. Perkins, MAPS,[c] Jeffrey P. Brosco, MD, PhD,[d] Edward L. Schor, MD[e,f]

abstract

OBJECTIVES: To fulfill the promise of a life of dignity, autonomy, and independence for children and youth with special health care needs (CYSHCN) and their families, greater value must be assigned to meaningful outcomes, such as quality of life and well-being.

METHODS: Despite decades of research, programs, and measurements addressing quality of life and well-being for CYSHCN and their families, there still is no consensus on how to measure, implement, or achieve them.

RESULTS: As the US health care system strives to reach the health care goals of safe, efficient, effective, equitable, timely, and patient-centered care, youth and families must be equal partners at all levels of the health care system—from clinical decision making to designing and implementing programs and policies.

CONCLUSIONS: The health care system must systematically measure the priorities of CYSHCN and their families. It also must incorporate data on quality of life and well-being when developing services, supports, and systems that help CYSHCN and their families to flourish rather than hindering them.

SUPPLEMENT ARTICLE

Coleman et al, "Quality of Life and Well-Being for CYSHCN and their Families" *Pediatrics* June 2022; Houtrow et al., "Health Equity for CYSHCN" *Pediatrics* June 2022.

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Blueprint GOAL Plain Language Version

Every child gets the services they need,

so that they can play, go to school,

and grow up to become a healthy adult.

(And so grown-ups and siblings can thrive too.)

Original language: “Children and youth with special health care needs enjoy full lives and thrive in their communities from childhood through adulthood.”

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What do we do? “Measure What Matters”

1. **QOL**
   - Universal measures: child thriving, kindergarten readiness, healthy weight, successful transition to adulthood, caregiver well-being
   - At least one condition-specific measure
2. **Populations**
   - Systems-level
     - What % of children/caregivers achieve the measures?
   - Equity
     - Do the demographics of numerator match those of the denominator?
3. **Accountable**
   - All organizations plan, track, explain (some SDOH/HRSN in their control)
     - Some rewarded for increased % of people achieving measures?
   - Universal measures in NOFOs, Title V, Medicaid, NSCH, CDC, etc.

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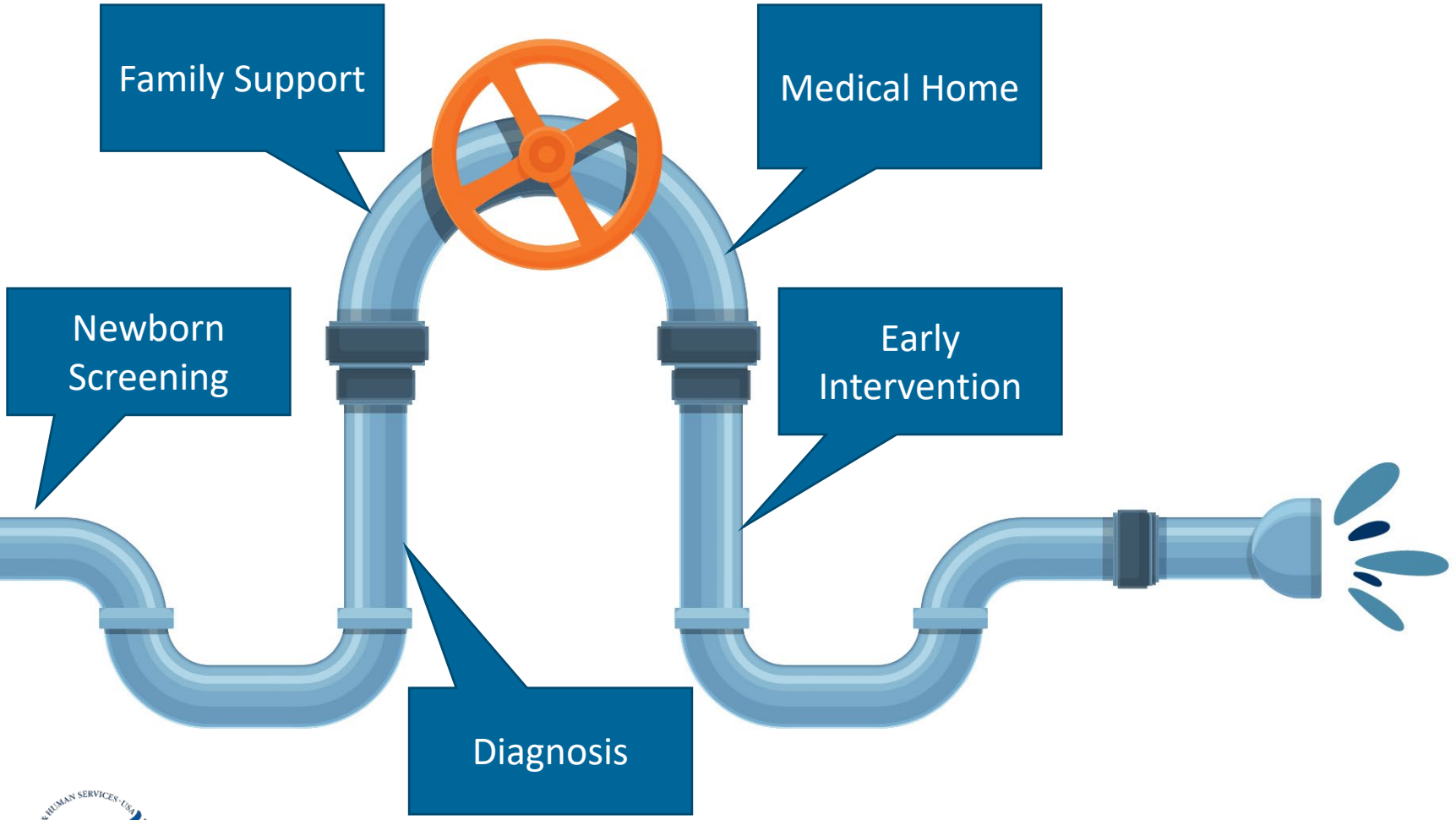
EXAMPLE: NBS for Deaf/Hard-of-Hearing Infants



Children achieving language acquisition milestones

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ACHDNC – Genetics in Medicine (2008)

Long-term follow-up after diagnosis resulting from newborn screening: Statement of the US Secretary of Health and Human Services' Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children

Alex R. Kemper, MD, MPH[1], Coleen A. Boyle, PhD[2], Javier Aceves, MD[3], Denise Dougherty, PhD[4], James Figge, MD, MBA[5], Jill L. Fisch[6], Alan R. Hinman, MD, MPH[7], Carol L. Greene, MD[8], Christopher A. Kus, MD, MPH[9], Julie Miller, BS[10], Derek Robertson, MBA, JD[11], Brad Therrell, PhD[12], Michele Lloyd-Puryear, MD, PhD[13], Peter C. van Dyck, MD, MPH[13], and R. Rodney Howell, MD[14]

- Central components
  - Care coordination
  - Evidence-based treatment
  - Quality improvement
- Features
  - Quality chronic disease management
  - Condition-specific treatment
  - Care throughout lifespan

ACHDNC – Genetics in Medicine (2011)

What questions should newborn screening long-term follow-up be able to answer? A statement of the US Secretary for Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children

Cynthia F. Hinton, PhD, MPH[1], Lisa Feuchtbaum, DrPH, MPH[2], Christopher A. Kus, MD, MPH[3], Alex R. Kemper, MD, MPH[4], Susan A. Berry, MD[5], Jill Levy-Fisch, BA[6], Julie Luedtke, BS[7], Celia Kaye, MD, PhD[8], and Coleen A. Boyle, PhD, MS[1]

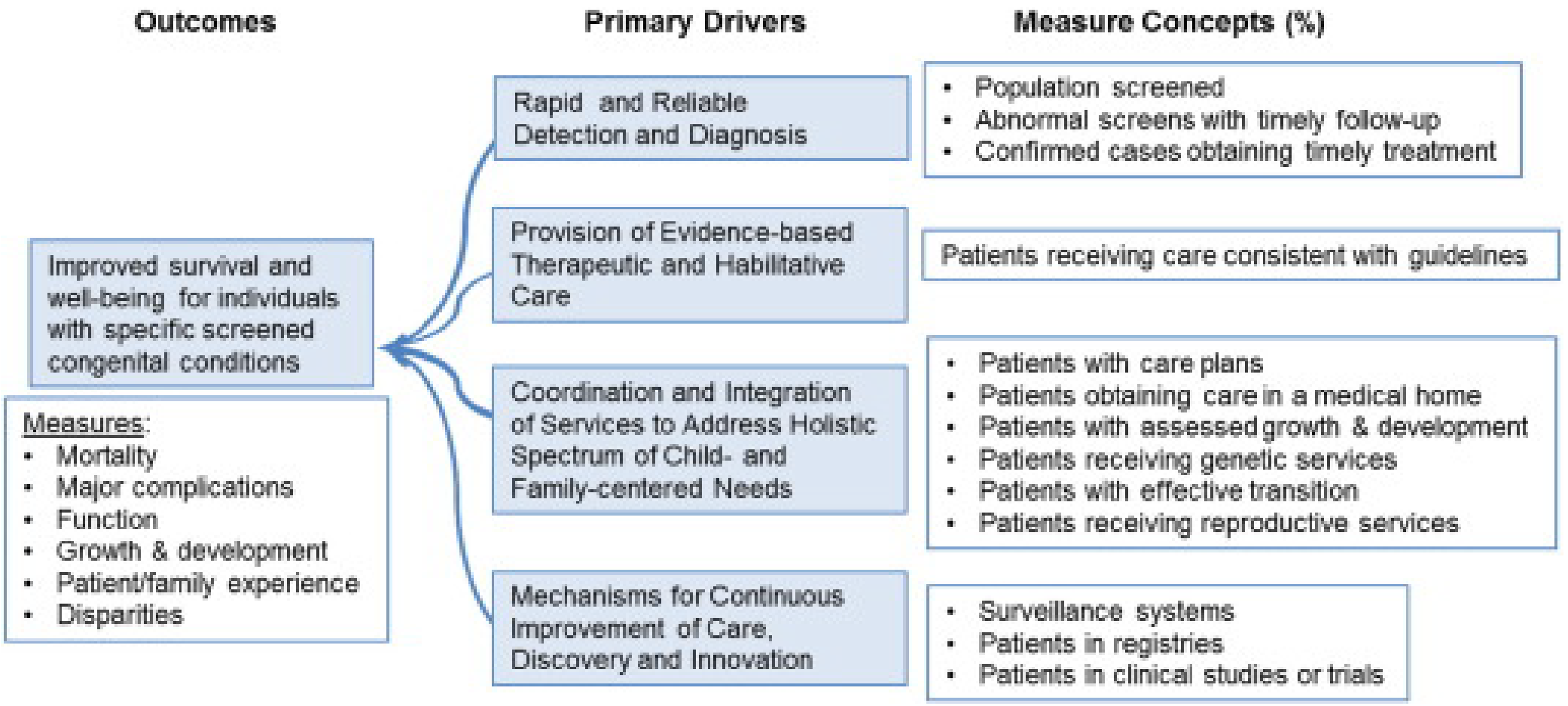
- Central components
  - Care coordination
  - Evidence-based treatment
  - Quality improvement
- Perspectives
  - State and nation
  - Primary/specialty providers
  - Families

ACHDNC – Molecular Gen & Metab (2016)

A framework for assessing outcomes from newborn screening: on the road to measuring its promise☆

Cynthia F. Hinton [a,*], Charles J. Homer [b], Alexis A. Thompson [c], Andrea Williams [d], Kathryn L. Hassell [e], Lisa Feuchtbaum [f], Susan A. Berry [g], Anne Marie Comeau [h], Bradford L. Therrell [i], Amy Brower [j], Katharine B. Harris [k], Christine Brown [l], Jana Monaco [m], Robert J. Ostrander [n], Alan E. Zuckerman [o], Celia Kaye [p], Denise Dougherty [q], Carol Greene [r], Nancy S. Green [s], the Follow-up and Treatment Sub-committee of the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC):

Framework for Assuring Good Outcomes from NBS



Hinton et al, 2016

The Role of Quality Measures to Promote Long-Term Follow-up of Children Identified by Newborn Screening Programs

Presented by the FUTR Workgroup to ACHDNC (February 2018)

- Quality measures are a crucial part of health and health care system
- Many different types of quality measures
- Creating/collecting data for these measures for NBS can be challenging
- Different perspectives needed, esp. patient/family/consumer
- Engage a broad range of stakeholders to
  - Identify a core set of long term follow-up quality measures and data resources
  - Encourage the use of large data collection activities (e.g NSCH) and QI activities (e.g. HEDIS)
  - Health Information Technology (HIT) standards/Clinical Decision Support (CDS) in the EHR

GOAL: Three (Integrated) Buckets of Data

1. Lab result analysis
   - Use data from state NBS to improve risk assessment at the pre-diagnostic level
2. Notification/confirmation families & clinicians
   - Short-term follow-up (quality indicators for timeliness)
   - NewSTEPs
3. Longitudinal clinical care
   - LTFU = “long-term” follow up or “longitudinal” follow-up
   - Public health surveillance

Bucket # 1: Lab Result Analysis

- CDC ED3N
  - Carla Cuthbert and Amy Gaviglio's presentation
- Data from Bucket # 2 is essential to continuous quality improvement for Bucket # 1
  - "Clinical data" - diagnosis to confirm lab result analysis
  - Bucket # 3 also useful for late onset conditions (childhood, adult)

Bucket # 2 – Short-term follow-up: NewSTEPs

HRSA has funded short-term follow-up data activities for two decades, including the Newborn Screening Data Repository and Technical Assistance Program ("NewSTEPS") since 2012

Program Goal: To enhance, improve and expand the newborn screening system by supporting state public health newborn screening programs, public health professionals, families, and primary and specialty care practitioners on the implementation of state-based public health newborn screening

APHL NewSTEPs Center NBS Support Infrastructure:

- Technical Assistance
- Data Repository
- Quality Improvement*

** QI portion of this work is funded under a separate award, activity code: UG8*

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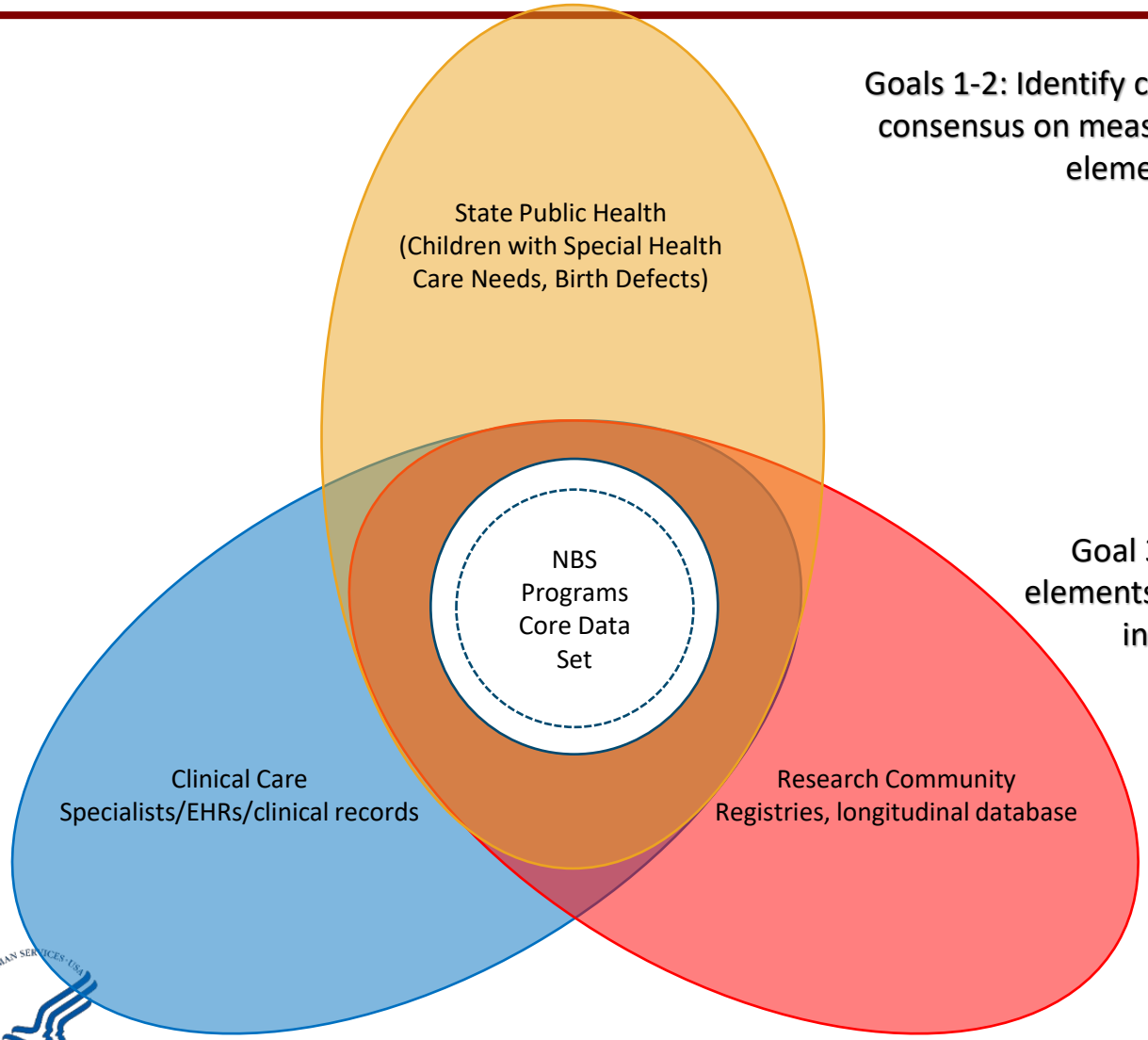
Bucket # 3: LTFU Data Scheme from MCHB grantees



Goals 1-2: Identify core elements, get consensus on measurement of core elements

NBS Core Data Set (examples)

- Diagnosis
- Date of appropriate first intervention
- Are they alive?
- Within the last 12 months did the child receive care and treatment specific to the diagnosis? Type of care provider?

Goal 3: Map data elements that would go into these

State Public Health (examples)

- Treatment? (Yes/No; Reason reported for no treatment in the last 12 months?)
- Alive? (Cause of death: Injury, medical, unknown, unasked, other, other)
- Developmentally appropriate milestones? (Yes/no/ unknown/unasked by category)
- Healthcare utilization: How many ER visits in the last 12 months? How many hospitalizations in the last 12 months? How many clinic visits in the last 12 months?

Clinical Outcomes (examples)

- Clinical trial participation
- Quality of Life
- Patient Reported Outcomes

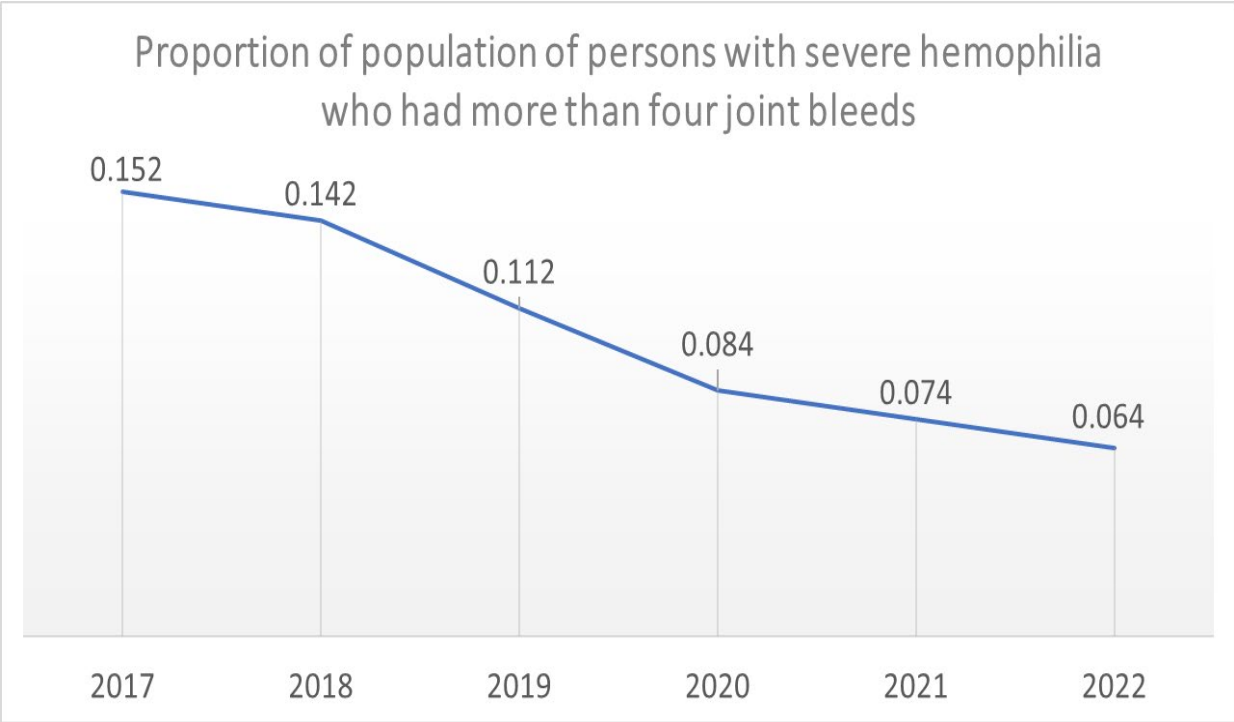
Research (examples)

- Disorder specific outcomes
- Demographics
- Medical home status

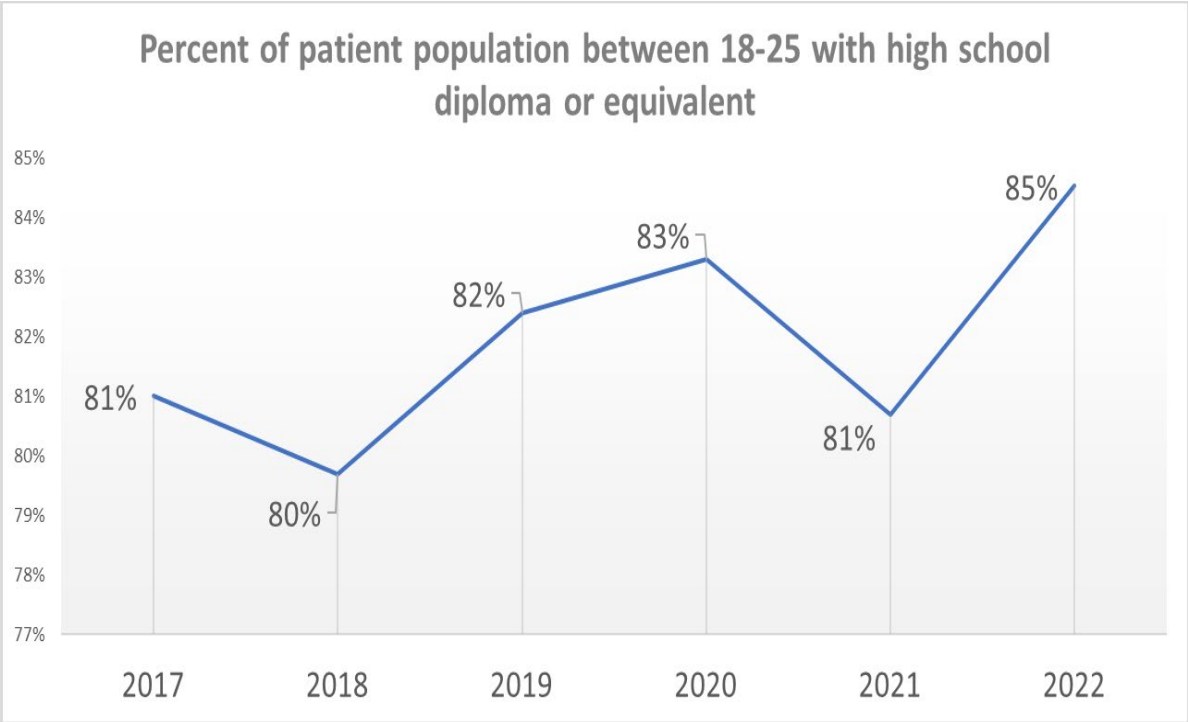
Goal 4: Next steps, including opportunities to collaborate, disseminate

Hemophilia: CDC – HRSA Collaboration

Proportion with >4 joint bleeds



Percent with high school diploma



Community Counts Registry for Bleeding Disorders Surveillance (Community Counts Registry), CDC/NCBDDD and ATHN

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Conclusion: Federal Role in NBS

- HRSA (with federal partners)
  - Support ACHDNC and RUSP process
- Support state NBS programs: “NBS is a system, not a test”
  - Implementing new conditions
  - Short-term follow-up
  - Long-term follow-up
- Public Health Ideal
  - If we are going to screen a baby for a condition, we have some obligation to make sure that the child gets treatment
  - Integrated data approach to reaching that ideal

Contact Information

Jeffrey P. Brosco MD PhD

Director, Division of Services for Children with Special Health Needs (DSCSHN)

Maternal and Child Health Bureau (MCHB)

Health Resources and Services Administration (HRSA)

jbrosco@hrsa.gov

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