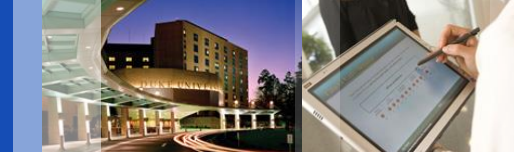


Update from the Cost Analysis Workgroup

Lisa A. Prosser, Ph.D., M.S.
March 14, 2014



COST ANALYSIS WORKGROUP (CAWG)

CRW

Alex Kemper, MD (CHAIR)
Duke University/DCRI

K.K. Lam, PhD
Duke University

Jeffrey P. Brosco MD PhD
University of Miami, CMS South Region - FL Title V

Lisa A. Prosser, Ph.D.
Univ of Michigan Medical School, School of PH

Scott Grosse, PhD
Centers for Disease Control and Prevention

CONSUMERS

Annamarie Saarinen, M.S.
Newborn Foundation |Coalition

NBS/STATE PUBLIC HEALTH

Mei W. Baker, MD, FACMG
Newborn Screening Laboratory/Univ of Wisconsin

Marci Sontag, PhD
NewSTEPS/ 360, CO School of Public Health

Christopher Kus, MD, MPH
ASTHO, Div of Family Health, NY State Dept of Health

Sylvia Mann, M.S., C.G.C.
HI DOH, Western St Gen Services Collaborative

John D. Thompson, PhD
Office of Newborn Screening/WA State DOH

HRSA

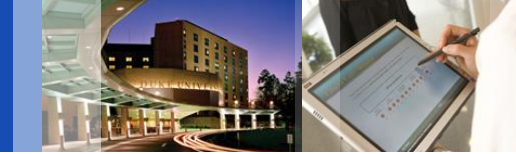
Joan A. Scott, M.S., C.G.C.
Genetic Services Branch, MCHB

Debi Sarkar, M.P.H.
Genetic Services Branch, MCHB



Charge of the CAWG

- To consider methods to assess the “cost of newborn screening expansion” as required by the newly reauthorized legislation
- Deliverable: Report with recommendation(s) to the ACHDNC on how to incorporate cost assessment into the evidence review



Questions to Address:

- 1) What costs of “newborn screening expansion” should be included within a condition review to better inform the Committee?
- 2) What are the critical data elements needed to address the cost of newborn screening expansion?
- 3) What is the availability and feasibility of collecting data?
- 4) What/who are the data sources and who will provide the data? the nominator? The condition review workgroup?
- 5) How will this impact the nomination and review process?



Mission Creep – Many Methods of Economic Evaluation (*from S. Grosse, 2015*)

□ Cost-effectiveness analysis (CEA)

- *Which approach costs less per unit of health gained?*
- *CEA using quality-adjusted life years (QALYs) also called cost-utility analysis (CUA)*

□ Cost-benefit analysis (CBA)

- *Is the monetary value of benefits to society greater than total cost?*

➤ Budget impact analysis (BIA)

- *Expected net change in financial expenditures for a health care system over a given timeframe – budget holder perspective*
- *This type of cost accounting analysis is more feasible and directly useful to states*



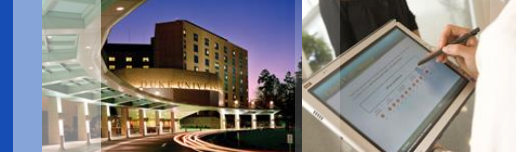
Incremental Costs to Consider in Dried Blood Spot NBS

- ❑ **Costs to public health departments**
 - *Laboratory testing*
 - Staff costs
 - Equipment and reagents
 - Space and utilities
 - *Short-term follow-up and tracking*
- ❑ **Downstream costs to health care systems and families**
 - *Clinical follow-up from screening through diagnosis*
 - *Long-term management, including treatment and monitoring*
 - Target conditions – difference in treatment following early diagnosis
 - Secondary conditions or ambiguous diagnoses
- ❑ **Cost of NBS expansion is more than laboratory costs**



Cost to States to Add a Condition Varies

- ❑ **Average variable cost of laboratory testing may be higher with lower testing volume**
- ❑ **States vary in use of 2nd screens, outsource labs, shared resourcing with regional collaboratives, cost payments for confirmatory and diagnostic testing**
- ❑ **States may offer contracts to specialty centers**



Considerations and Challenges

- **Mission Creep** – Many approaches to assessing “cost of NBS expansion”
- **One Size Does Not Fit All** – Variability across states in costs incurred and paid
- **Feasibility** – Condition Reviews to add Cost Analysis... while Condition Review timeframe limited to 9 months
- **Resources** – Who will conduct the cost analysis?
- **Utility of Cost Information** – How cost information will be considered by Committee in Decision Matrix still to be defined



Proposed Approach

- ❑ ***Budget Impact/Cost Analysis most feasible***
- ❑ ***Focus on Common Cost Categories of NBS Expansion***
 - *Make assumptions clear*
 - *Identify variability or ranges for cost inputs (eg, 1 v 2 screen states)*
 - *Determine scope: specify cost categories, time horizon, perspective*



Next Steps

- Review methods used for MPS I Cost estimates
- Develop draft template to estimate incremental costs of adding a NBS condition
- Coordinate efforts with CRW, Pilot Study Workgroup, AC, HRSA, and others
- Prepare range of cost estimates for X-ALD for AC
- Develop proposal for development of software tool that could be shared with states to project costs
 - Requires collection of data to develop a cost function
 - Requires programming skills



EXTRA



Time and Resource Constraints for Reviews

- ❑ **Legislation *restricting* Condition Reviews to 9 months**
- ❑ **Modeling cost-effectiveness or cost-benefit of expanding NBS is resource intensive**
 - *CDC CEA of screening for CCHD took two years*
 - *APHL CEA of screening for SCID has taken 9 months to adapt an existing model*
 - *SCID and CCHD models were conducted after conditions had been added to the RUSP*
 - *Previously published systematic reviews were available*
 - *Other costing or cost-effectiveness analyses had been published*
- ❑ **Economic evaluations of screening for candidate disorders may be even more challenging**



How Can Decision Makers Use Economic Evaluations?

- ❑ Consider health outcomes and costs as *separate* criteria, i.e., traditional approach
- ❑ Assess balance of costs and outcomes, e.g., net benefit or cost-effectiveness ratio
- ❑ Use economic findings to inform decision to approve an intervention
 - *Decision rule – yes/no decision or deferral of final decision*
 - *Cost-effectiveness or net benefit as one among many decision criteria*
- ❑ Use findings to identify gaps in knowledge and prioritize research
- ❑ Use economic findings to guide prioritization or implementation by providers (states)



Economic Cost of Screening for a Disorder

- ❑ Incremental cost of screening
- ❑ Incremental costs of confirmatory and diagnostic testing
 - *Cost per test multiplied by number of infants tested with NBS minus number of infants tested without NBS*
- ❑ Incremental costs of treatment

TABLE 2 Projected Costs and Health Benefits for Newborn-Screened and Clinically Identified Newborn Cohorts

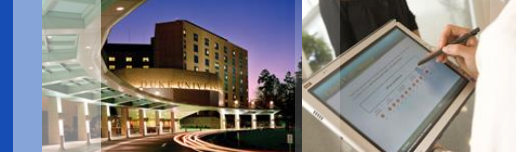
	Clinical Identification (SE)	Newborn Screening Program (SE)	Difference With Screening
Population, n			
Size of population	100 000	100 000	
Children diagnosed with MCADD	5.88 (0.01)	8.40 (0.01)	2.52
False-positive screen results	NA	20 (0.02)	20
Costs, \$^a			
Screening	NA	710 251	710 251
Treatment ^b	630 704 (10 639)	919 231 (12 243)	288 527
Total	630 704 (10 639)	1 629 482 (12 250)	998 778

Prosser LA, Kong CY, Rusinak D, Waisbren SL. Projected costs, risks, and benefits of expanded newborn screening for MCADD. *Pediatrics*. 2010;125(2):e286-294.



Value is in the Eyes of the Stakeholder

- ❑ **For some, only health outcomes matter**
 - *Medicare coverage decisions based on “medical necessity”*
- ❑ **Others are interested in budget impact**
 - *Affordability – direct outlays*
 - *Net cost savings and return on investment (ROI)*
- ❑ **Affordability or value?**
 - *If an intervention is “affordable” in terms of overall costs and no major change in infrastructure is required, decision may be driven by perceived benefits alone*
 - *If intervention is perceived as difficult or expensive, consideration of cost-effectiveness or cost-benefit may play a role*



Costs of Diagnostic Testing for MPS I

MPS I Condition Review: Public Health System Impact Assessment: Fact Sheet for MPS I Screening

- ❑ **Between 8 and 45 per 100,000 infants screen positive for MPS I and referred for diagnostic testing**
- ❑ **Confirm low or undetectable enzyme activity**
 - *Alpha-L-iduronidase enzyme activity assay in white blood cells*
 - *Urinary excretion of glycosaminoglycan (GAG)*
 - *Cost between \$200 and \$600 per specimen tested*
 - *Total cost of \$2,400 to \$27,000 for 100,000 infants screened*
- ❑ **Diagnostic molecular testing**
 - *Cost between \$1,000 and \$2,800 per IDUA gene sequencing test*
 - *Total expected cost between \$2,000 and \$8,000.*
- ❑ **Total cost \$4,500 to \$36,000, or \$0.05-0.35 per infant**



How Do Other Federal Advisory Committees Use Economic Information?

❑ US Preventive Services Task Force

- *No explicit use*

❑ Community Guide

- *Existing economic estimates reviewed by CDC economists AFTER a decision is made to recommend a service*
- *Intended to help stakeholders with prioritization of implementation*

❑ Advisory Committee on Immunization Practices (ACIP)

- *Required input for decisions on adding vaccines to schedules*
- *Nominators for vaccines must provide economic analysis*
- *Reviewed by CDC economists and Committee members*



Potential Cost Target Deliverables:

- a) **Comparative across Conditions:** Provide a range of per-child cost estimates to the AC and DHHS –
- b) **Inform States in future adoption and implementation:** Provide a spreadsheet tool that states can use to project their costs to add the disorder based on various perspectives, time horizons, and so on
 - desirable but will need to determine feasibility with existing resources*