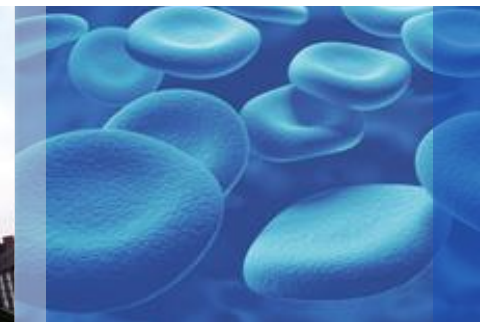


Cost Analysis Workgroup: Update

Alex R. Kemper, MD, MPH, MS

February 12, 2016





Charge

- To consider methods to assess the “cost of newborn screening expansion” as required by the newly reauthorized legislation
- Deliverable: Report with recommendation to the ACHDNC on how to incorporate cost assessment into the decision-making process



Cost Assessment Plan - Recap

- Objective: Budget Impact on States
- Methods
 - *Interviews, surveys with programs screening or considering screening (preferably states)*
 - *Vendors*
 - *Other sources: Literature, Technical Experts*
- Data
 - *Primary (critical, costs incurred by state to add NBS for a condition)*
 - *Screening, laboratory costs, through STFU*
 - *2 year time horizon, annualized*
 - *Secondary (optional, depending on resources)*



Pretesting the Draft Approach - Planning

- Aim: To assess feasibility and effectiveness of proposed cost assessment methods
- Target Condition Selection for Pretest:



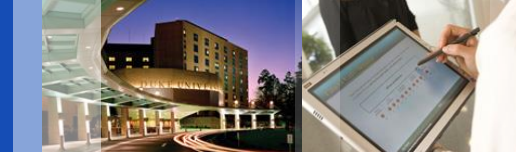
Pretesting Target Condition: MPS I or Pompe?

- Characteristics
 - *Single or multiplex screening*
 - *Dual platforms in use (MS/MS, DMF)*
 - *Laboratory-developed vs. commercially available*
 - *Comparison with initial cost estimates from MPS I condition review*
 - *Which Condition to Pretest? Both MPS I and Pompe offer numerous complexities to inform cost assessment methodology*



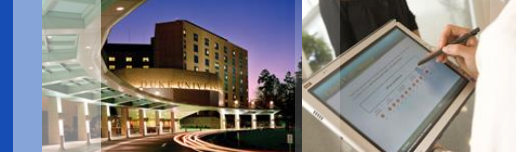
Cost Assessment Pretest - Aim

- Cost estimates of adding MPS I, Pompe NBS for single- and multiplex scenarios
- NOT to estimate costs for every variation
- *Complexities should lead us down paths that will **inform** the range of variation in screening across states and other conditions*



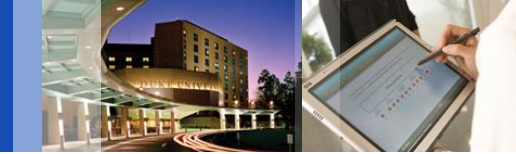
High Variability in Costs...

- Birth rate
- Geographic/Regional Locale
- Existing laboratory facilities and personnel
- Laboratory Information Systems
- Use of outside labs
- Shared resources with other states
- Availability of and contracts with specialty centers
- Service contract specifics
- NBS funding structure
- *And so on, and so on.....*



Assumptions and Starting Points

- ***Start somewhere, and be clear about Base and Starting Assumptions...***
 - *Assume a hypothetical state with 100,000 births*
 - *Single specimen screening per infant (i.e., no routine second screens)*
 - *Purchase of equipment and supplies (vs. service contracts, existing infrastructure)*
 - *In-house laboratory screening*
 - *2-year cost projections, annualized*
- ***...and Estimate “Conceptual Confidence Ranges”***



State Public Health Lab Costs

PRIMARY COST CATEGORIES

Laboratory

- Equipment
- Supplies (disposables, reagents)
- Installation and maintenance
- Space and utilities
- Staffing
- Laboratory information systems

Staff Development & Services

- Training, education
- Outreach and referral for confirmatory testing & STFU



State Public Health Labs

SECONDARY COST CATEGORIES

State Public Health Budget

- Long-term tracking and monitoring
- Educational outreach
- Reporting & LT Surveillance

Families and Health Care Systems

- Treatment and long-term care



Key Questions for Pretest

- **How best to get cost estimates from states with screening mandates with least burden?**
 - *No standard approach to estimating*
 - *Confidential/protected vendor pricing, estimates &*
 - *Estimates specific to states*
 - *Cost components and categories vary*
- **Will need to pretest flexible approaches to gathering costs from states and vendors**
 - *spreadsheets,*
 - *total cost estimates with checklist of components*



Pretest Plan (*Albeit Very Ambitious*)

Cost Assessment	Pretest Activities	Timeline
Stage 1: <i>Protocol Review and Screening Implementation</i>	<ul style="list-style-type: none"> • Review protocol, identify screening methods and platforms • Finalize cost questions • Identify states & contact 	FEB 2016
Stage 2: <i>Information Gathering</i>	<ul style="list-style-type: none"> • Interview/Email states screening or near screening for cost estimates • Contact Vendors for estimates • Follow up re: questions and methods 	MAR 2016
Stage 3: <i>Synthesis</i>	<ul style="list-style-type: none"> • Categorize cost information • Obtain mid-point and ranges • Outline assumptions and context • Review methods, feedback, and cost estimates with CAWG and CRW 	APR 2016
Stage 4: <i>Reporting</i>	<ul style="list-style-type: none"> • Finalize methods • Report Cost estimates • Report to ACHDNC 	APR/MAY 2016



Next Steps

- Scope out costs from MPSI and Pompe protocols
- Identify states that are preparing to screen
- Gather state costing templates to confirm cost categories
- Gather state costing estimates (Interviews, review of screening cost outlines)
- Present initial pretest findings at next AC meeting



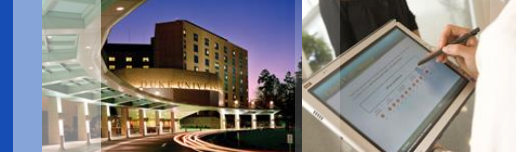
Bigger Questions Looming...

- What are the minimum requirements for a pilot study to adequately inform screening implementation and costs?
- How useful will the cost estimates be (with limited time and resources)?
 - *For states with different situations?*
 - *For the Advisory Committee?*
- How will the Advisory Committee use the cost estimates in decision-making?



Prerequisites for Conducting the Cost Assessment

- Pilot screening
 - *U.S. based vs non-U.S.*
 - *State NBS or Research study*
 - *Evidence from High-throughput screening --
Minimum # screened in pilot? (>5,000 newborns?*)*
 - *Minimum # screened positive and true positive?*



What are the Prerequisites for Conducting the Cost Assessment?

- Pompe - Population-based screening, Non-U.S. (Taiwan)
- MPSI - State NBS Pilot screening (non-live), U.S. research
- X-ALD - State NBS live screening
 - *with positive screens and confirmed cases (X-ALD)*
 - **Minimum # screened positive and true positive?*
 - **note –X-ALD screening study with MD NBS (n=5,000), No detected cases, Italy-regional, population-based LSD screening (<5000), no positive Pompe or MPS I*