

Evidence Evaluation & Methods Workgroup: Developing a Decision Analysis Model

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Overview

- Limitations of evidence review
- Brief introduction to decision analysis
- Case study: Newborn screening for MCADD
- Application to hyperbilirubinemia

ACHDNC: Evidence Evaluation Methods Working Group

- Convened in April, 2011
- Charged with evaluating evidence review methods
- Considered modeling to assist in evidence synthesis and generation
- Hyperbilirubinemia case study

Decision Analysis (DA)

- Systematic approach to decision making under conditions of uncertainty
- Requires explicit consideration of each aspect of the decision problem:
 - Defining full set of alternatives
 - Choices regarding timing of implementation
 - Uncertainties involved
 - Assigning relative values to full set of possible outcomes
- Identifies alternative estimated to result in maximum benefit and uncertainty associated with that projection

Advantages of Modeling

- Can evaluate existing & un-tested alternatives
- Can simulate head-to-head comparisons
- Requires explicit definition of assumptions
- Can identify sources of uncertainty/prioritize future research
- Allows for extension of time horizons

Decision Analysis & Child Health Policy

- Decision analytic modeling approach can provide insights into comparative effectiveness and cost-effectiveness
- Especially salient for child health by providing approach for projecting long-term outcomes
- Cost-effectiveness results increasingly considered

Decision Analysis & Newborn Screening

- Incorporation of modeling into the evidence review process:
 - Simple models
 - Health outcomes
 - No cost-effectiveness analysis (yet)
- Initial goal is to project health benefits and potential harms

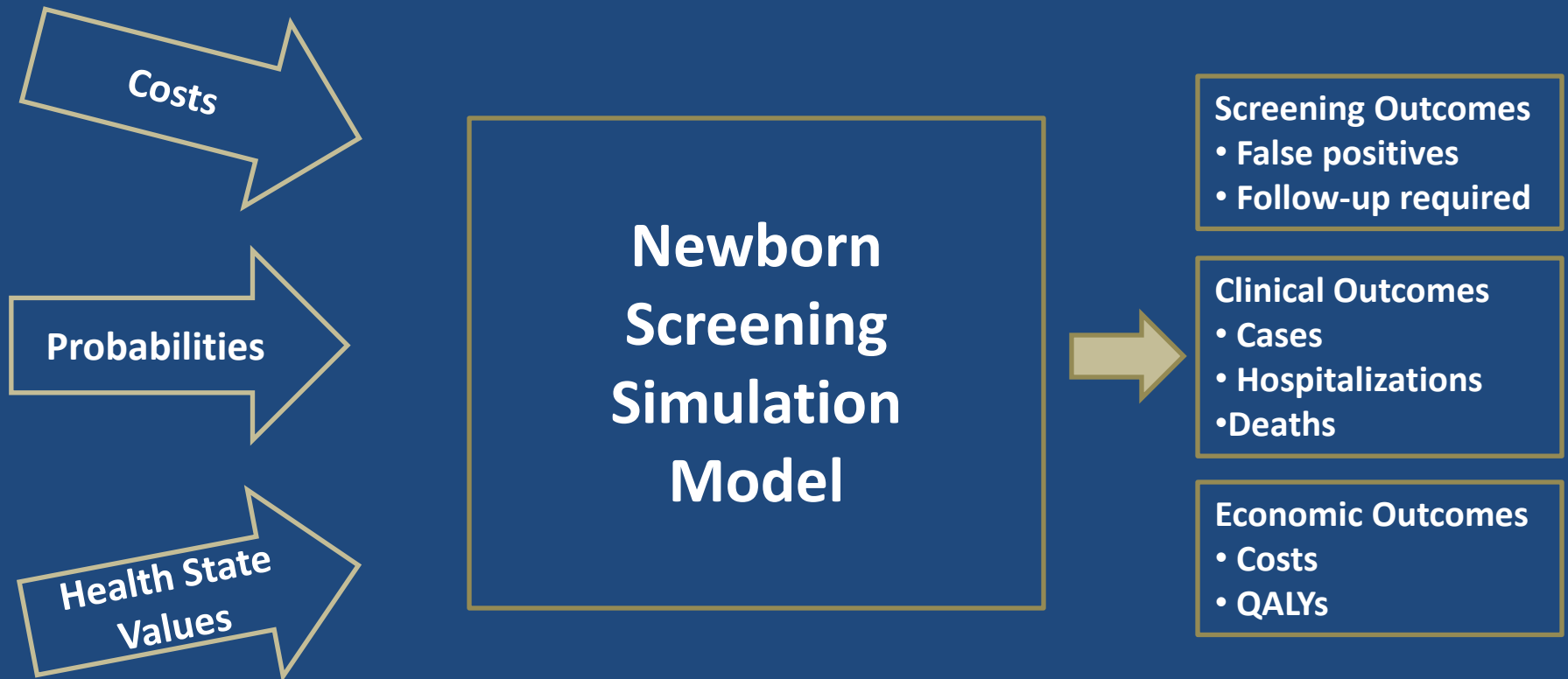
Case Study: Expanded Newborn Screening for MCADD

- Expansion of newborn screening programs using MS-MS
- Incremental costs very low
- Total costs of screening and follow-up not characterized
- Higher incidence with newborn screening

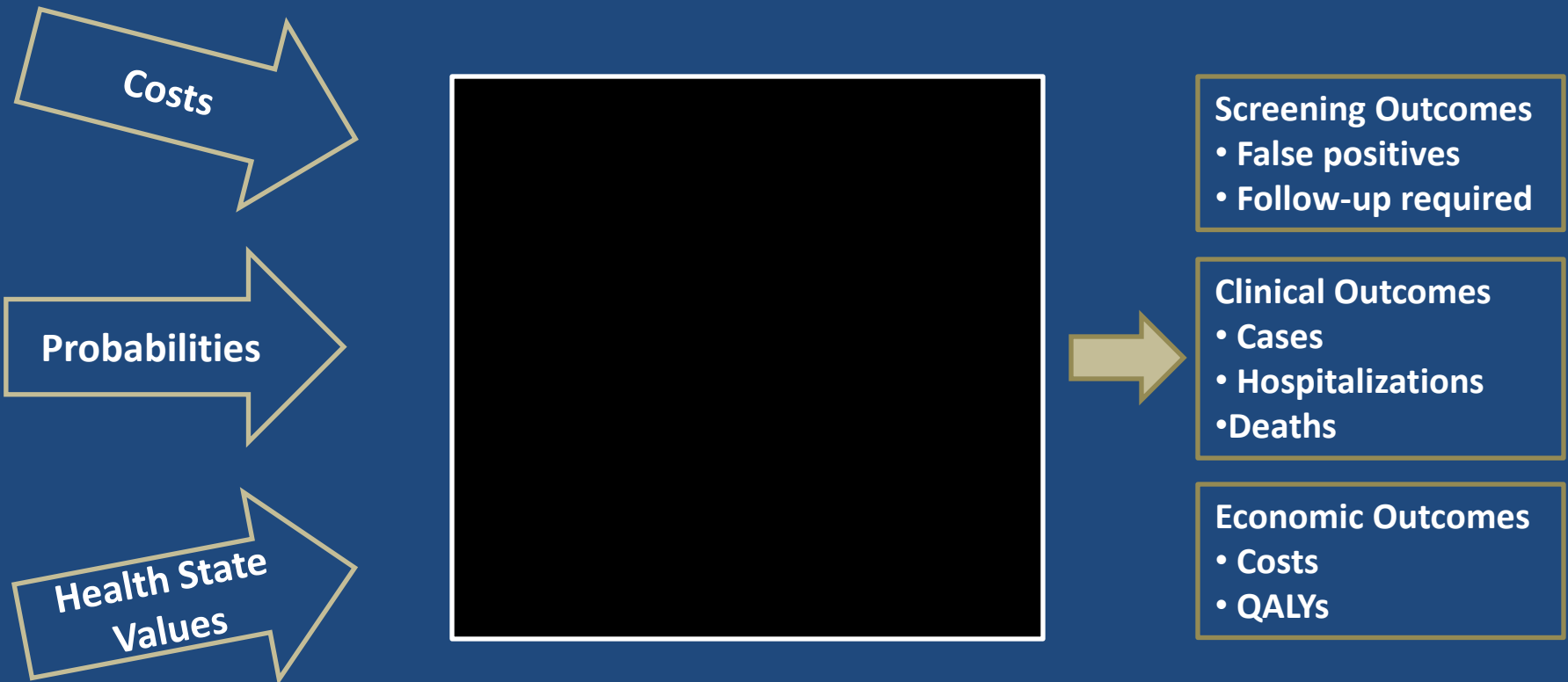
MCADD Example: Methods

- Develop a decision analytic model to project health benefits, health risks, and costs of expanded newborn screening
- Model inputs using primary and published data supplemented by expert opinion:
 - Event probabilities (e.g., outcomes of metabolic disease, test characteristics)
 - Costs of screening and clinical outcomes
 - Effects on health-related quality of life (HRQOL)
- Limited evidence especially for long-term outcomes
- Analysis: Projected long-term clinical outcomes and cost-effectiveness

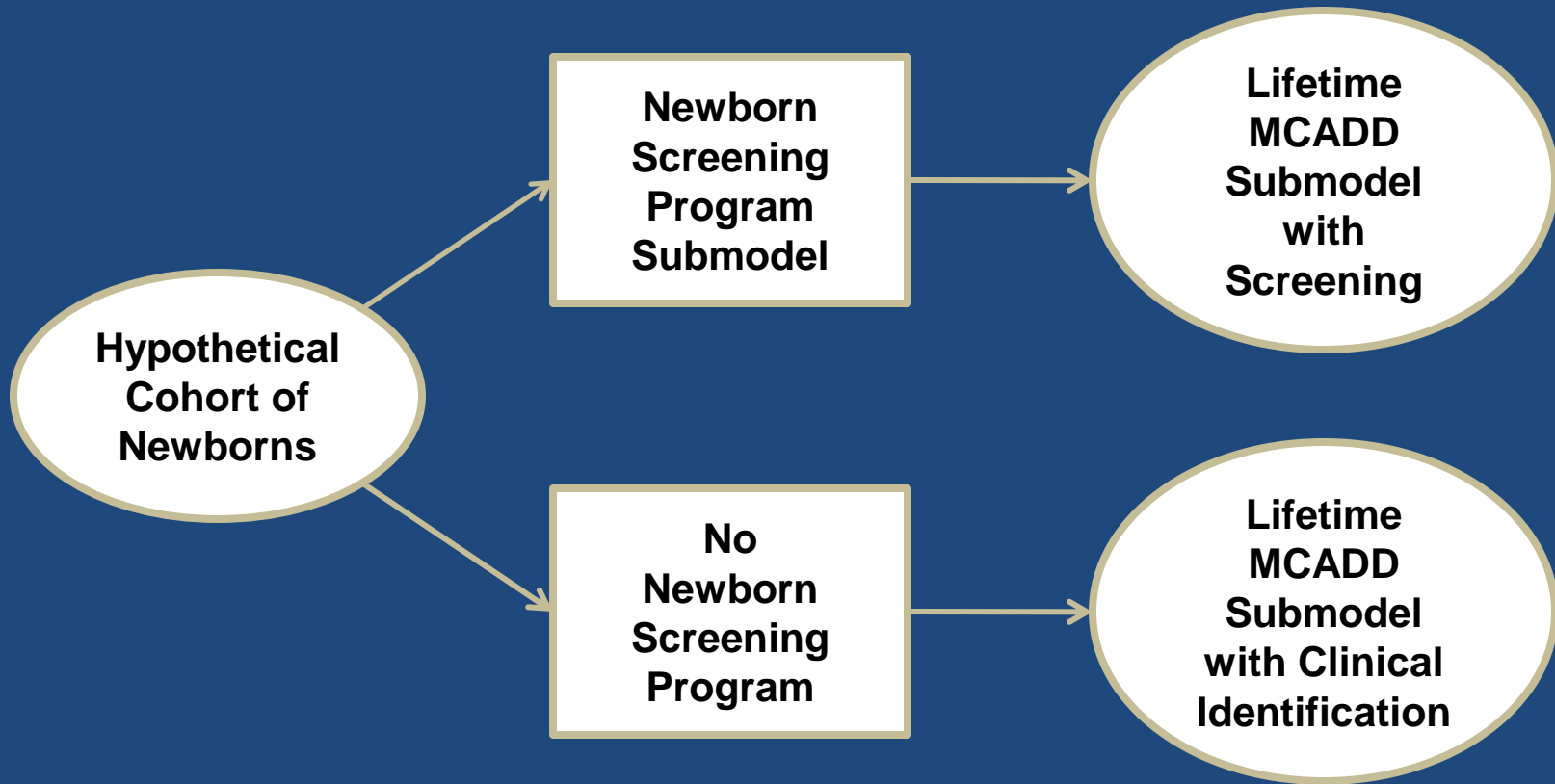
MCADD Example: Decision Analytic Model



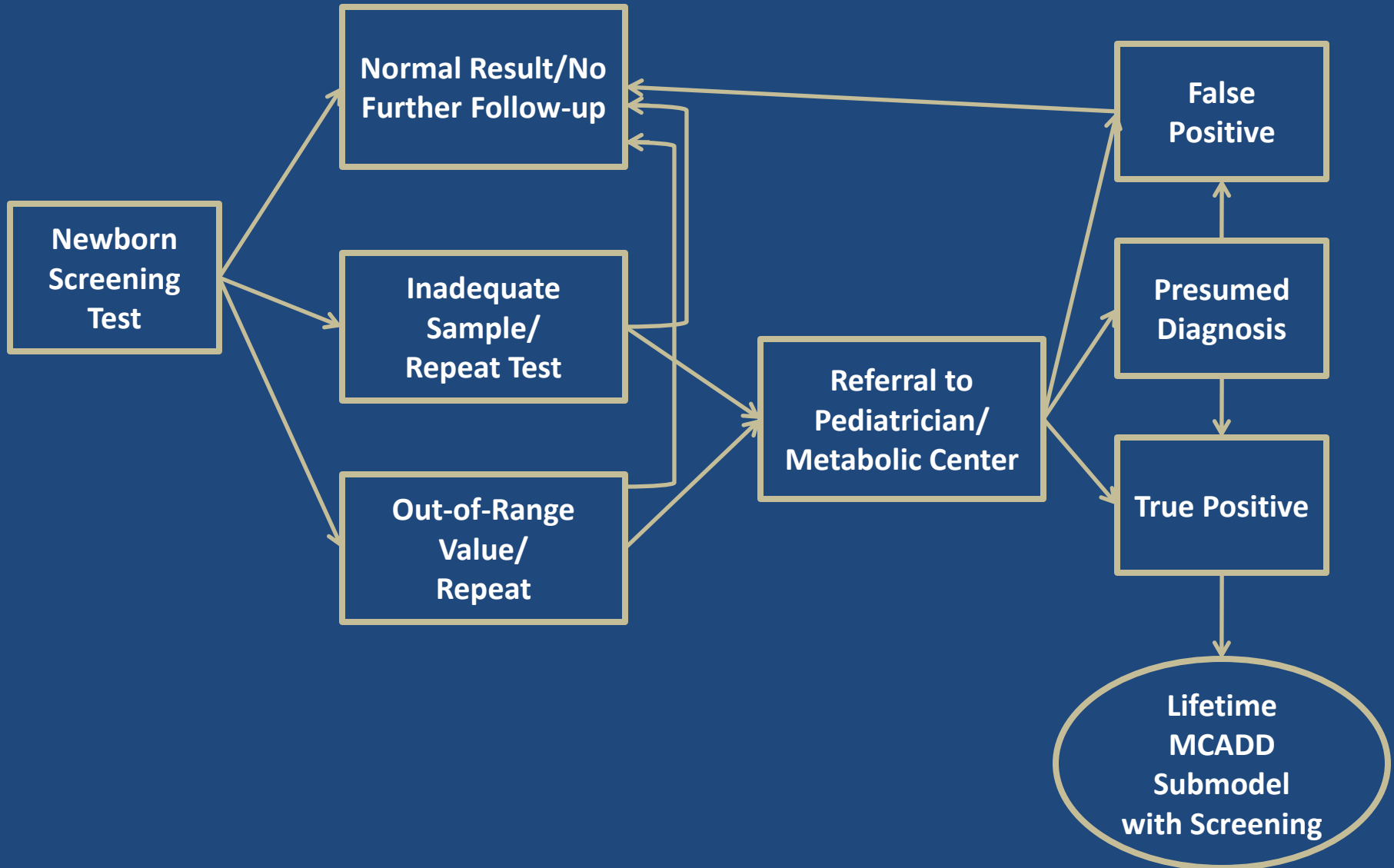
MCADD Example: Decision Analytic Model



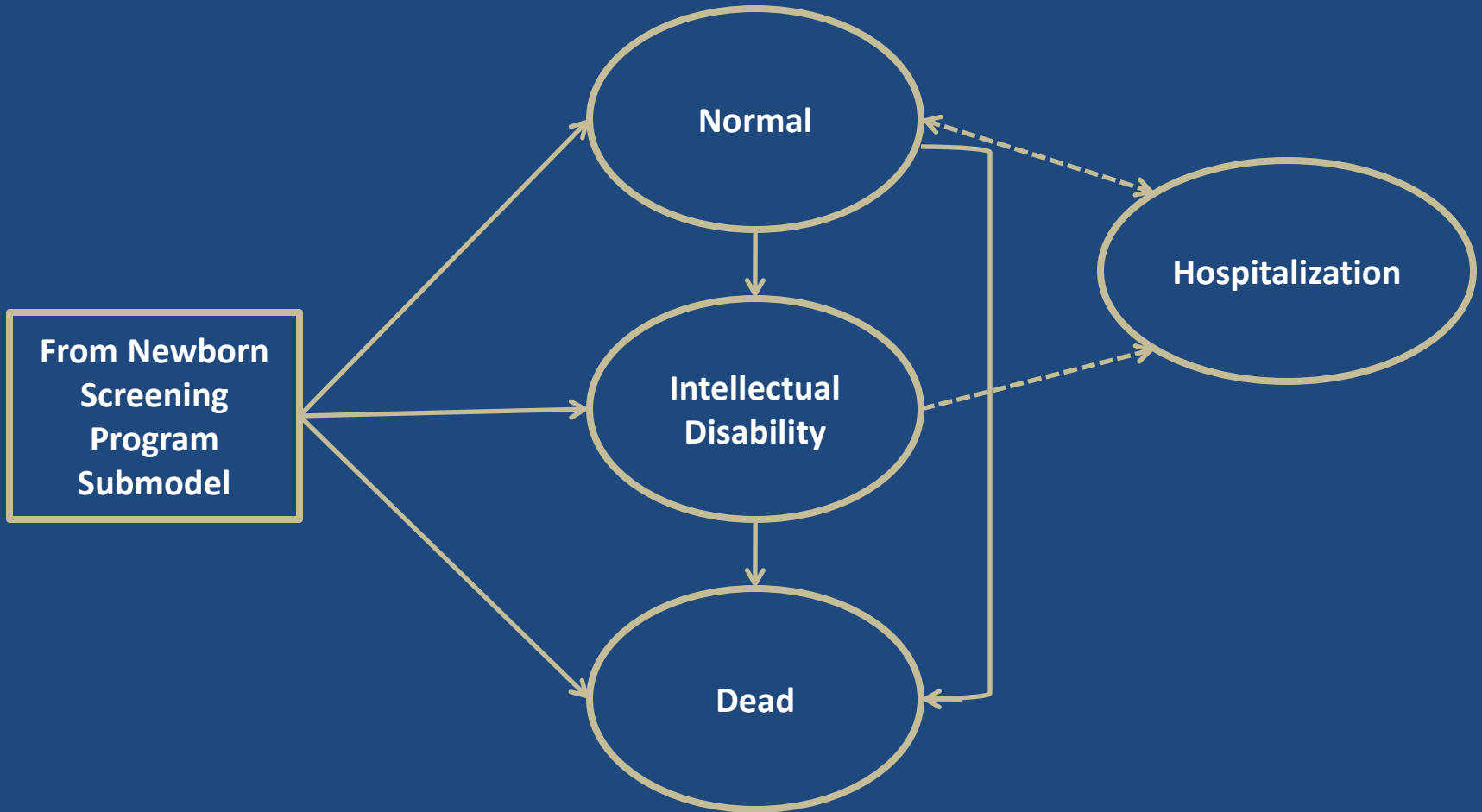
MCADD Newborn Screening Simulation Model



MCADD Newborn Screening Submodel



Lifetime MCADD Submodel

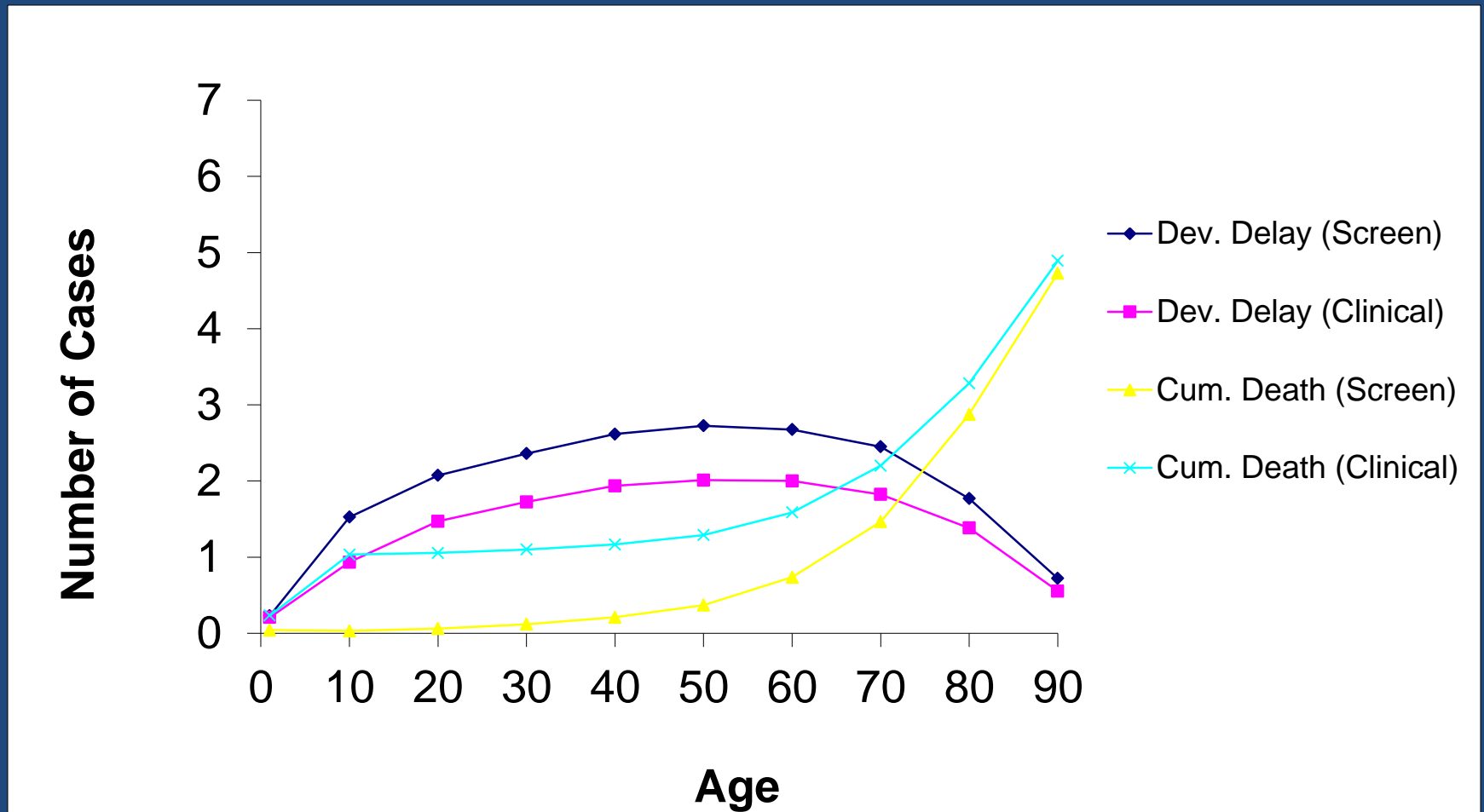


MCADD Example: Projected Outcomes

	Clinical Ident.	Screening	Δ
Population	100,000	100,000	-
Children w/MCADD	5.88 (0.01)	8.4 (0.01)	2.52
FP screen	N/A	20 (0.02)	20
Costs (lifetime)	\$630,710	\$1,629,482	\$998,778
QALYs	2,976,780.08	2,976,827.03	46.95
C/E ratio		\$21,273 (395)	

Source: Prosser et al., *Pediatrics*, 2010

MCADD Example: Projected Long-term Outcomes



Source: Prosser et al., Pediatrics, 2010

MCADD Example: Policy Implications

- Model predicts short- and long-term outcomes
 - Screening test and follow-up results
 - Projected number of children with condition, cases of developmental delay, hospitalizations, deaths
 - Costs
 - Quality-adjusted life years
- Results robust to changes in underlying assumptions
- Results sensitive to test costs, but not false positive rate

Application to Hyperbilirubinemia

- Create decision analysis model
- Expert panel input
- Project short- and long-term health outcomes

Projected Health Outcomes

Selected Outcomes	Clinical Assessment	Universal Screening
Screening outcomes		
Short-term:		
Hospitalizations		
Long-term:		
Acute bilirubin encephalopathy		
Chronic bilirubin encephalopathy		
Unaffected		

Anticipated Findings

- Projected health outcomes and associated uncertainty
- Identification of key parameters
- Process for specifying assumptions on health benefits and potential risks

Moving forward

- Adding decision analysis to the evidence review can provide:
 - Approach for evidence synthesis
 - Method for specifying assumptions
- Incorporation of cost/cost-effectiveness analysis

Questions