Economic Analysis for Adding Newborn Screening for ALD

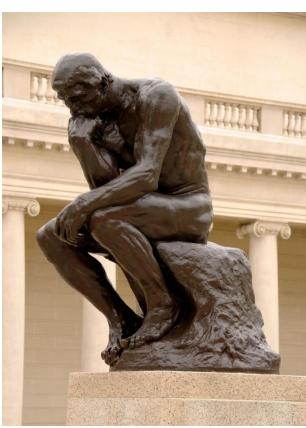
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Washington Criteria for NBS

- 1. Early identification benefits the newborn
- 2. Treatment is available
- 3. Nature of the condition justifies population-based screening
- 4. A good screening test exists
- 5. The benefits justify the costs of screening



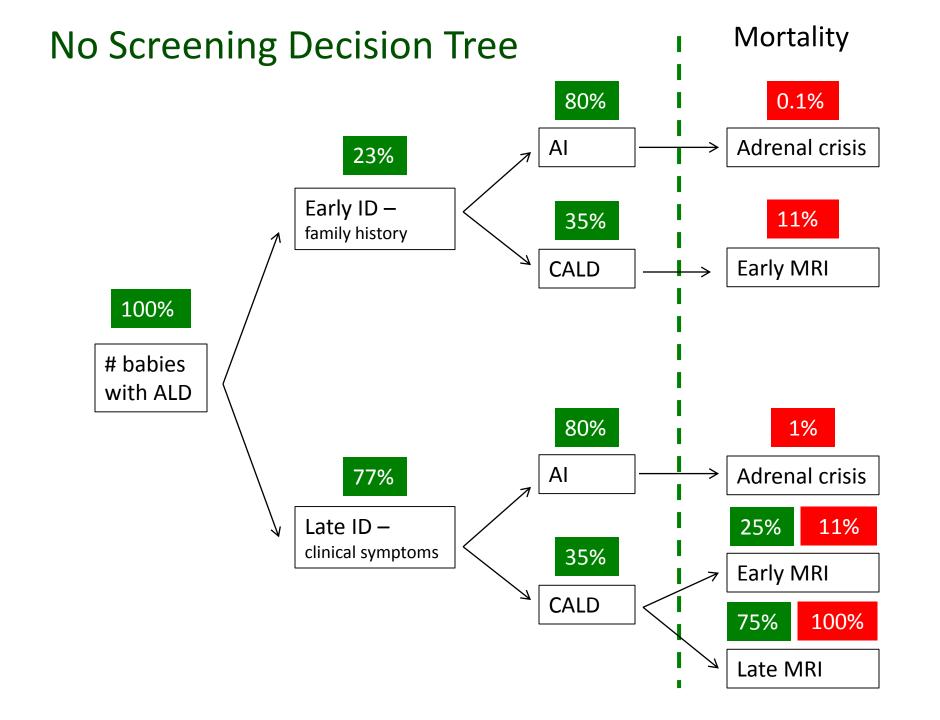
- **5. Cost-benefit/Cost-effectiveness:** The outcomes outweigh the costs of screening. All outcomes, both positive and negative, need to be considered in the analysis. Important considerations to be included in economic analyses include:
 - The prevalence of the condition among newborns.
 - The positive and negative predictive values of the screening and diagnostic tests.
 - Variability of clinical presentation by those who have the condition.
 - The impact of ambiguous results. For example the emotional and economic impact on the family and medical system.
 - Adverse effects or unintended consequences of screening.

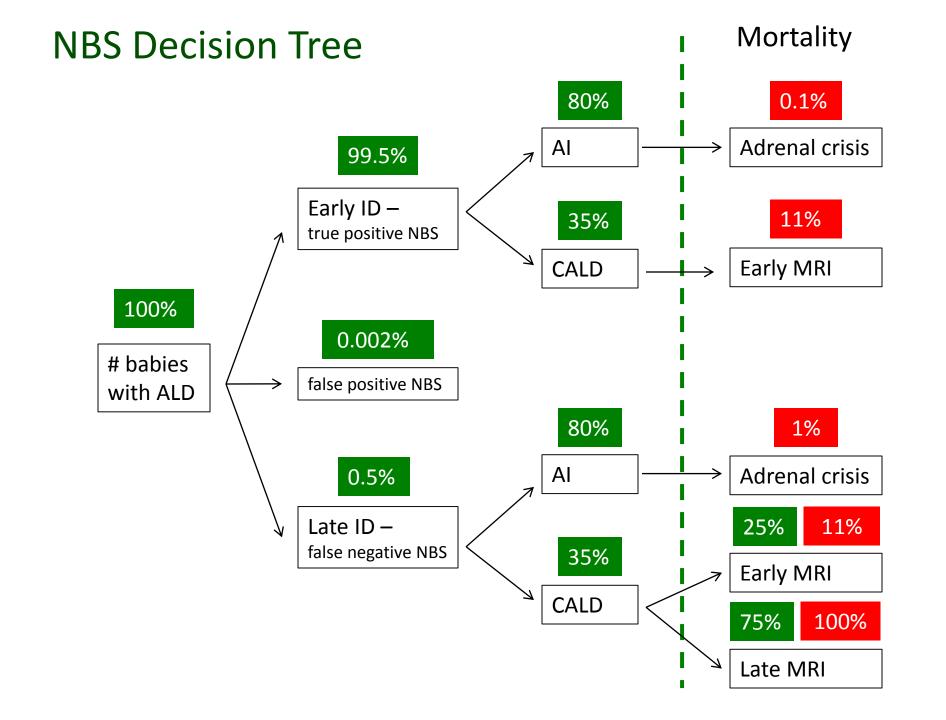


Strategy

- Decision Tree
 - Compares status quo v. screening model
- Data from
 - Primary literature (including pilot studies)
 - Reports from NY NBS program
 - Expert opinion
- Sensitivity analysis vary assumptions
 - High and low estimates for parameters

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	/				bess	strovoro dirability (6y)						* late MRI	0.75		CALD (late MRI)	0.4	0.0017			totalbonofitr	i	2,795,121.38
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Parameters

- Birthrate
- Prevalence of ALD
- % of cases with family history of ALD
- Morbidity rates (adrenal, CALD)
- Mortality rates (adrenal, CALD)
- Screening test performance
- Costs of newborn screening
- Costs of early v. late treatment
- Costs of serial testing
- Value of a life

Base-Case: Morbidity & Mortality

No Screening	deaths		0.7089
	surviving		2.4339
	early tx costs	\$	190,900.67
	serial monitoring costs	\$	43,642.54
	late tx costs	\$	682,748.34
	total tx costs	\$	917,291.55
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NBS Screening	deaths		0.1273
NBS Screening	deaths surviving		0.1273 3.0156
NBS Screening		\$	
NBS Screening	surviving	\$ \$	3.0156
NBS Screening	surviving early tx costs		3.0156 825,852.90
NBS Screening	surviving early tx costs serial monitoring costs	\$	3.0156 825,852.90 147,418.54

Base-Case: Shift

SHIFT		
Benefits	deaths averted	0.5816
	value of a life	\$ 9,000,000.00
	value of lives saved	\$ 5,234,735.87
	less treatment costs	\$ (60,413.32)
	total benefits	\$ 5,174,322.55

Base-Case: Shift

SHIFT			
Benefits	deaths averted value of a life value of lives saved less treatment costs total benefits	\$ \$ \$ \$	0.5816 9,000,000.00 5,234,735.87 (60,413.32) 5,174,322.55
Costs	costs of screening costs of false(+) costs of carrier ID total costs	\$ \$ \$ \$	880,000.00 3,802.70 2,972.31 886,775.01

Benefit/Cost Ratio

SHIFT		
Benefits	deaths averted	0.5816
	value of a life	\$ 9,000,000.00
	value of lives saved	\$ 5,234,735.87
	less treatment costs	\$ (60,413.32)
	total benefits	\$ 5,174,322.55
Costs	costs of screening	\$ 880,000.00
	costs of false(+)	\$ 3,802.70
	costs of carrier ID	\$ 2,972.31
	total costs	\$ 886,775.01
	benefit/cost ratio	5.83

Net Benefit

SHIFT		
Benefits	deaths averted	0.5816
	value of a life	\$ 9,000,000.00
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	total benefits	\$ 5,174,322.55
Costs	costs of screening	\$ 880,000.00
	costs of false(+)	\$ 3,802.70
	costs of carrier ID	\$ 2,972.31
	total costs	\$ 886,775.01
	benefit/cost ratio	5.83
	net benefit	\$ 4,287,547.54

Parameters

Range

- Birthrate
- % of cases with family history of ALD
- Morbidity rates (adrenal, CALD)
- Mortality rates (adrenal, CALD) → 30% to 90% (late)
- Screening test performance
- Costs of early v. late treatment
- Costs of serial testing

Model built in MS Excel

Mortality						
	early ID	low	mid	high	ref	
	death from adrenal insufficiency	0	0.001	0.005	expert opinion - Dr. Gerald F	Raymond
	death rate after HCT (Loes score < 10)	0.05	0.11	0.17	Miller et al. 2011	
	death rate after HCT (Loes score < 9)		0.05		Mahmood et al. 2007	
			0.08		Peters et al. 2004	
	late ID					
	death from adrenal insufficiency	0	0.01	0.05	expert opinion - Dr. Gerald F	Raymond
	death rate after HCT (Loes score ≥ 10)	0.15	0.4	0.6	Miller et al. 2011	
	death rate after HCT (Loes score ≥ 9)		0.46		Mahmood et al. 2007	
			0.55		Peters et al. 2004	
	death rate from late diagnosis and Loes score ≥ 10 (within 5y)		1		expert opinion - Dr. Gerald F	Raymond
	death rate from late diagnosis and Loes score ≥ 10 (by 18y)	0.7	0.8	0.9	expert opinion - Dr. Jennife	r Kwon - 2

Formula driven – assumptions can be tailored by individual programs

Recent Models Available

X-ALD (2015) - b/c ratio = 5.83

MPS-I (2017) – b/c ratio = 0.88

Pompe disease (2017) - b/c ratio = -18.02

Acknowledgements

Economics

Scott Grosse (CDC)

Neurology

- Gerald Raymond (U of Minnesota)
- Jennifer Kwon (U of Rochester)

Newborn Screening

- Joe Orsini (NY NBS)
- Beth Vogel (NY NBS)

Parent Advocates

Brad and Nancy Zakes



Lesson Learned

2017 update – significant analysis

- Prompted further conversations with neurologists
- Current clinical practice differs from published literature
 - Clinically identified cases with Loes scores ≥ 10 no longer transplanted
- b/c ratio improved from 3.95 to 5.83

Washington State Newborn Screening

