Economic Analysis for Adding Newborn Screening for ALD

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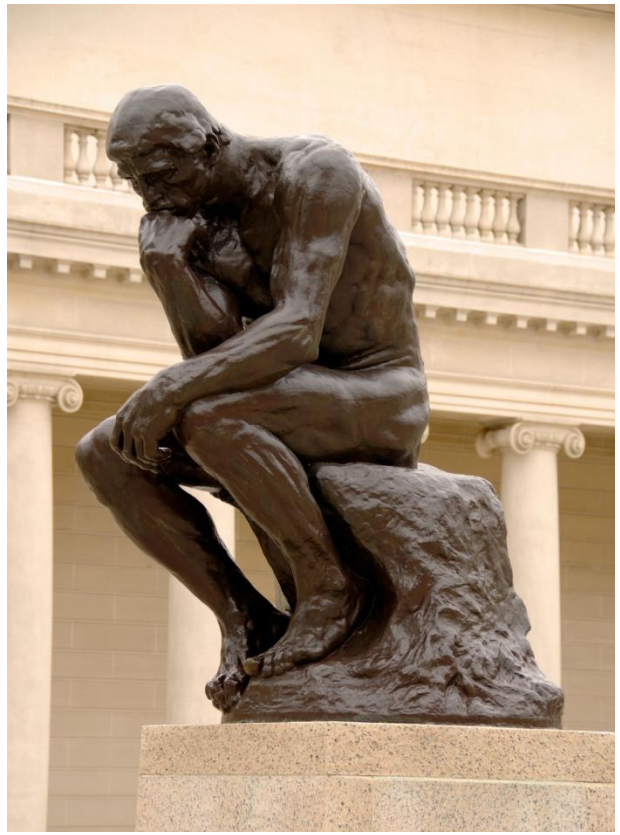
Washington Criteria for NBS

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

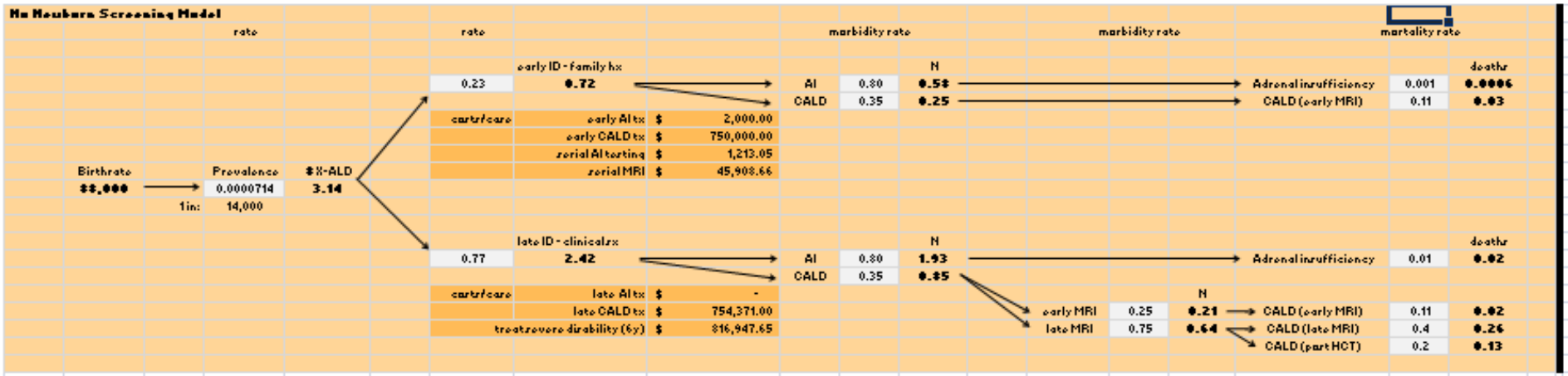
5. Cost-benefit/Cost-effectiveness: The outcomes outweigh the costs of screening. All outcomes, both positive and negative, need to be considered in the analysis. Important considerations to be included in economic analyses include:

- 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.

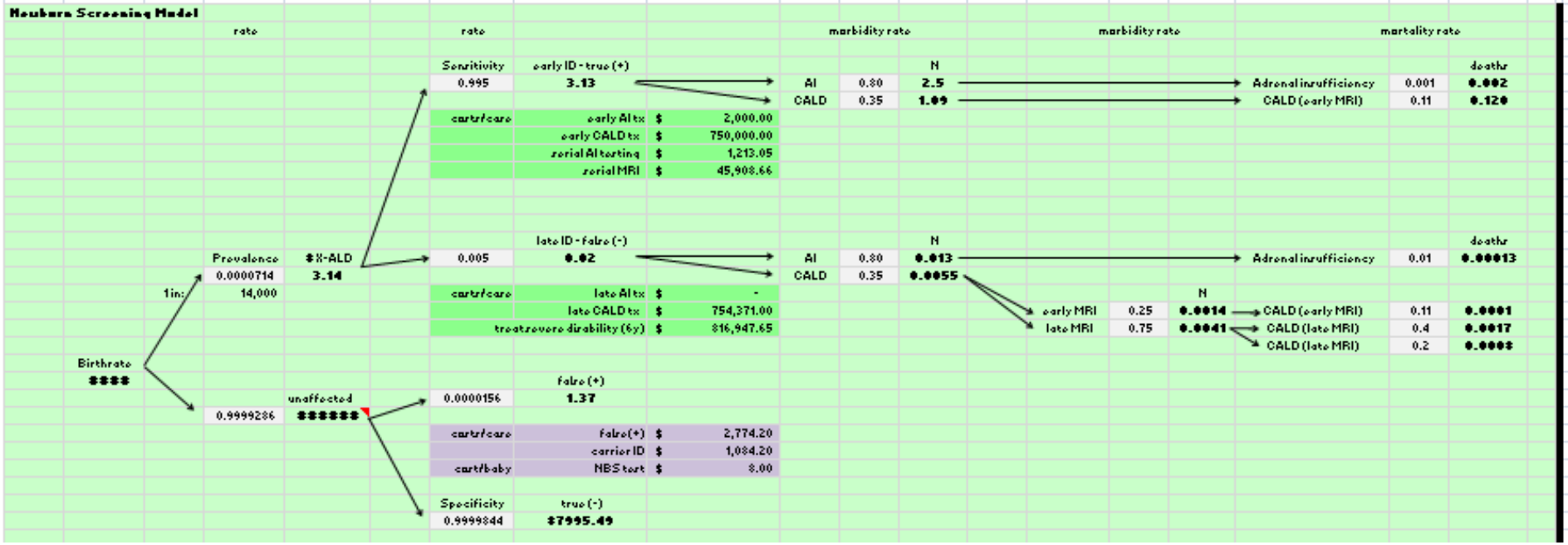


Strategy

- 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



No Screening			
	deaths		0.4536
	surviving		2.6892
	early tx carts	$	190,900.67
	serial monitoring carts	$	43,895.68
	late tx carts	$	585,823.84
	total tx carts	$	820,620.19



NBS Screening			
	deaths		0.1256
	surviving		3.0172
	early tx carts	$	825,852.90
	serial monitoring carts	$	147,551.64
	late tx carts	$	3,804.05
	total tx carts	$	977,208.60

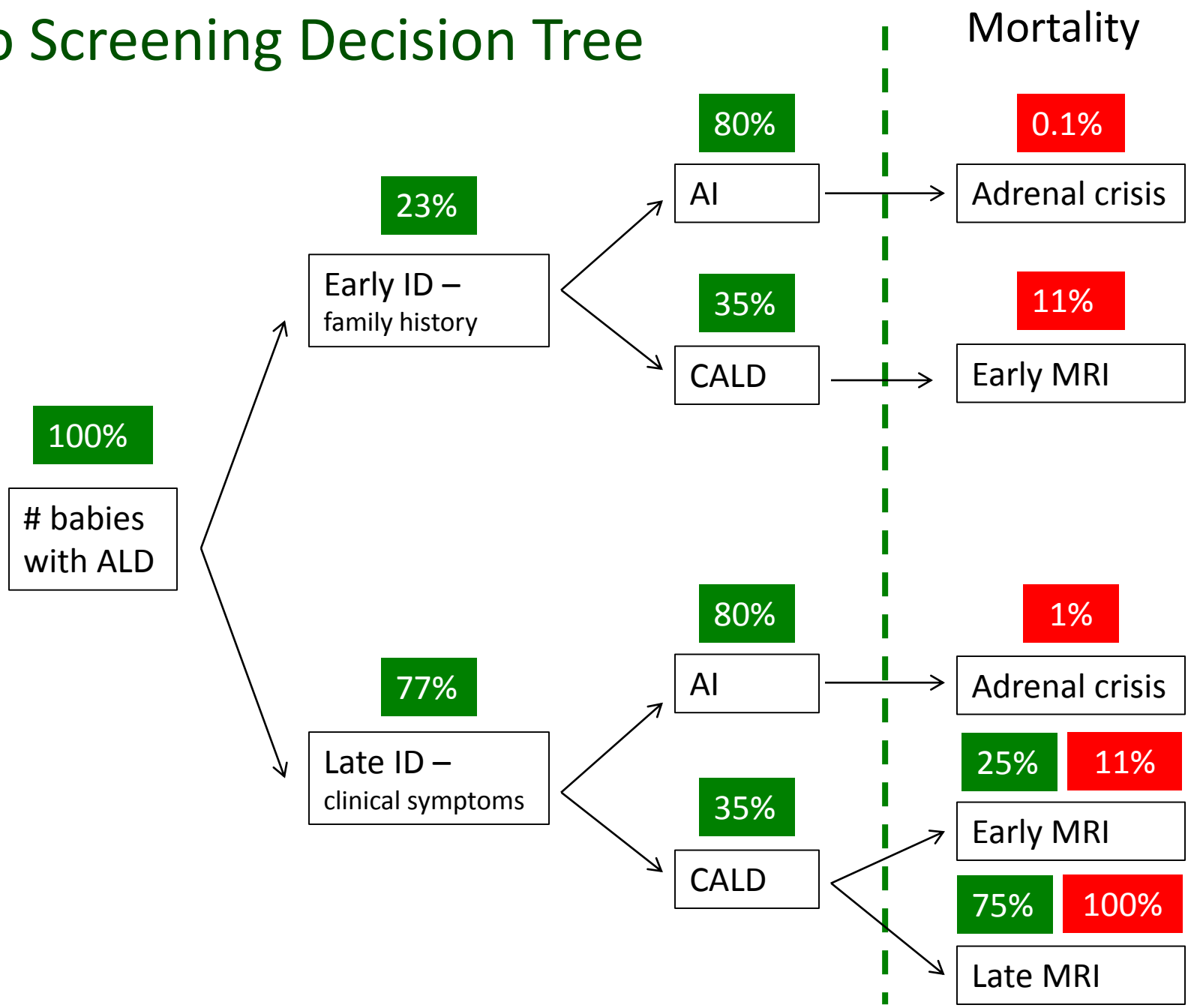
SHIFT

Benefits			
	deaths averted		0.3280
	value of a life	$	9,000,000.00
	value of lives saved	$	2,951,709.78
	less treatment carts	$	(156,588.41)
	total benefits	$	2,795,121.38

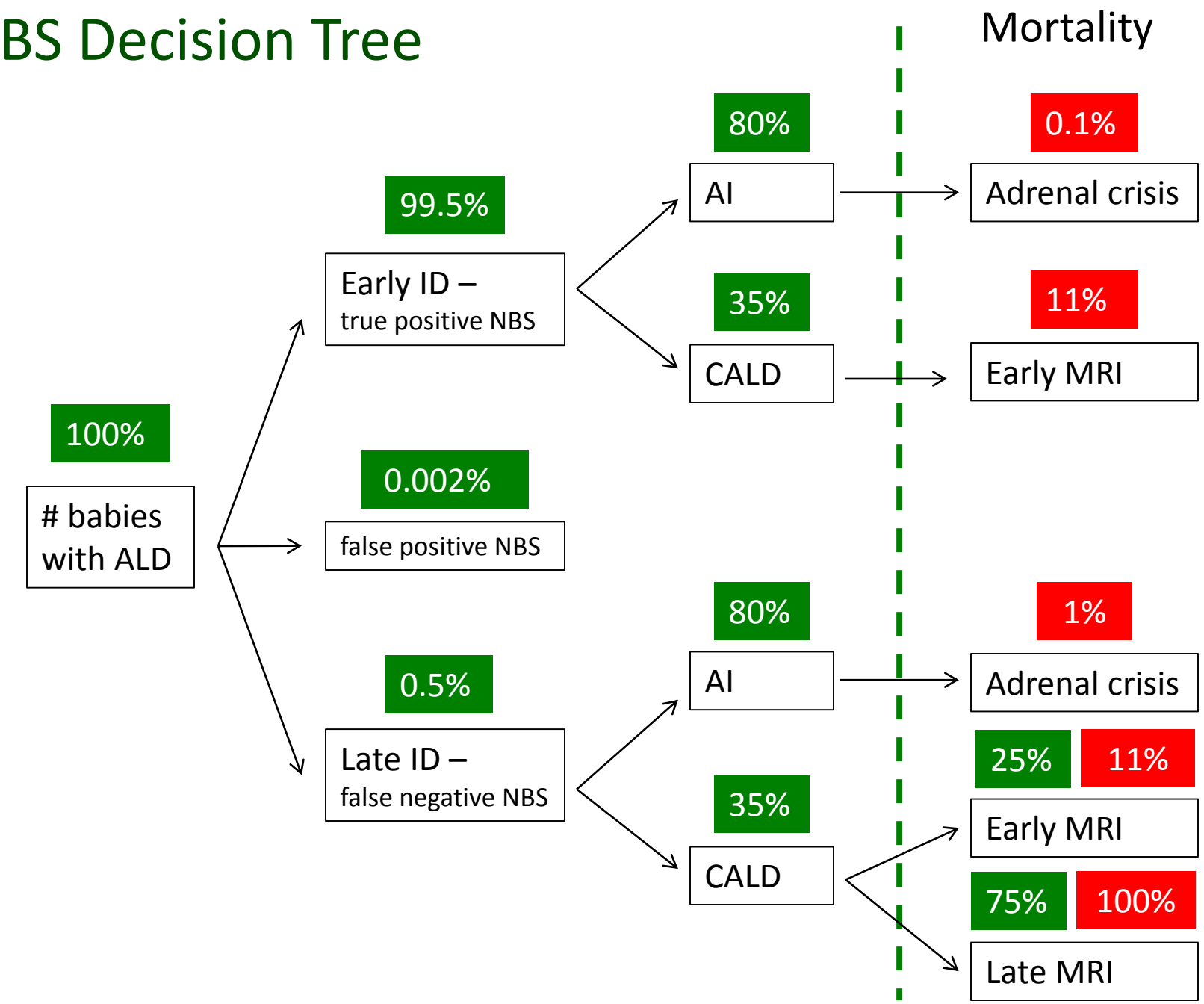
Carts			
	carts of screening	$	704,000.00
	carts of false(+)	$	3,802.70
	total carts	$	707,802.70

	benefit/cost ratio		3.95
	net benefit	$	1,817,912.78
	ICER	$	88,147.09

No Screening Decision Tree



NBS Decision Tree



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
NBS Screening	deaths		0.1273
	surviving		3.0156
	early tx costs	$	825,852.90
	serial monitoring costs	$	147,418.54
	late tx costs	$	4,433.43
	total tx costs	$	977,704.87

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		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

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
	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	$	4,287,547.54

Parameters Range

- Birthrate
- Prevalence of ALD → 1:11,000 – 1:17,000
- % of cases with family history of ALD
- Morbidity rates (adrenal, CALD)
- Mortality rates (adrenal, CALD) → 30% to 90% (late)
- Screening test performance
- Costs of newborn screening → $5 - $11 per baby
- Costs of early v. late treatment
- Costs of serial testing
- Value of a life → $7 - $11 million

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 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 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 Raymond
	death rate from late diagnosis and Loes score ≥ 10 (by 18y)	0.7	0.8	0.9		expert opinion - Dr. Jennifer 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 $\geq$ 10 no longer transplanted
- b/c ratio improved from 3.95 to 5.83

Washington State Newborn Screening

www.doh.wa.gov/nbs

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Come visit our lab!