



# Newborn Screening Advisory Panel Recommendations: Adding ALD to Newborn Screening Panel

Washington State Board of Health

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Newborn Screening Program

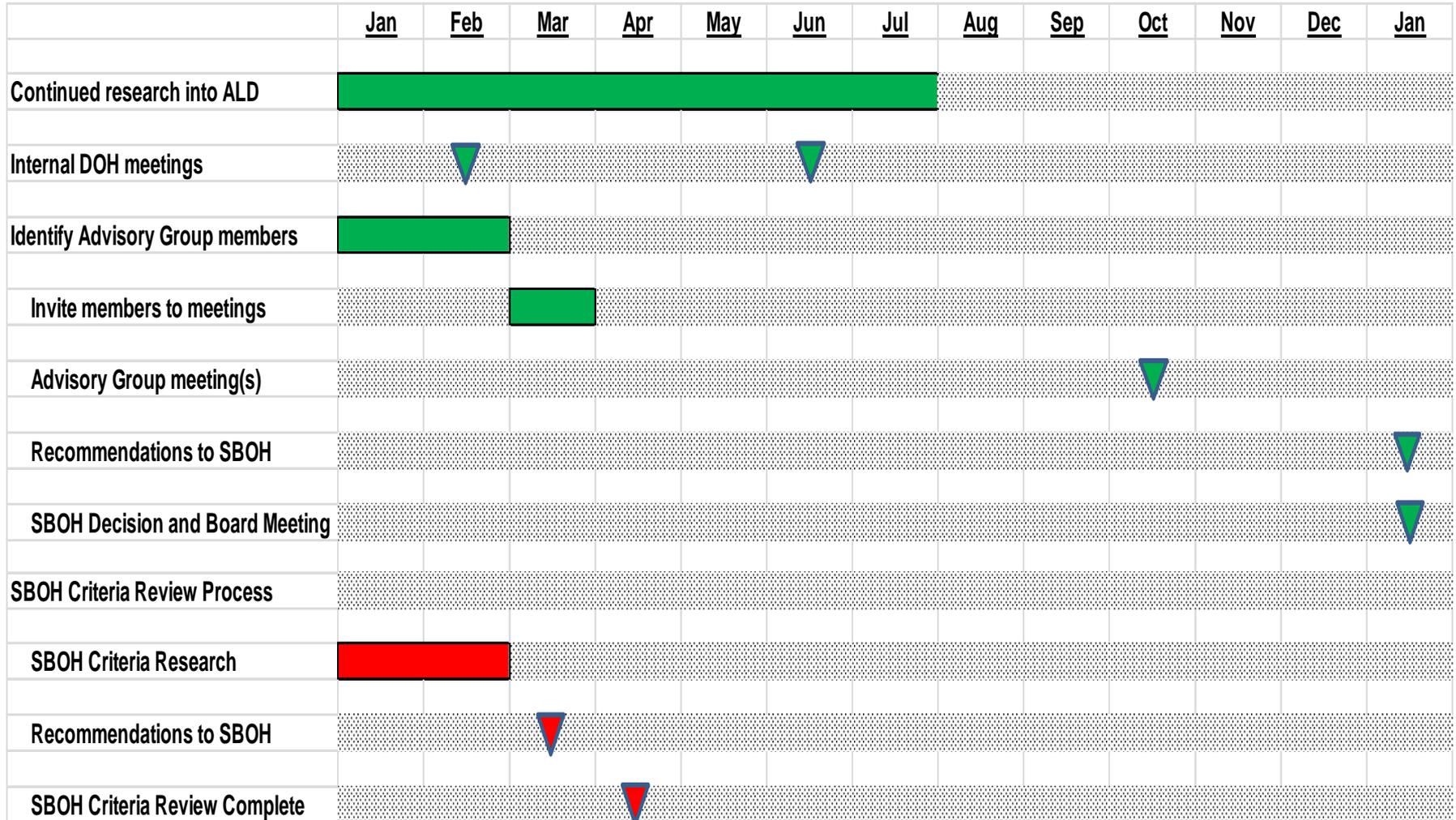
**PUBLIC HEALTH**  
ALWAYS WORKING FOR A SAFER AND  
HEALTHIER COMMUNITY



# Advisory Panel Formation

- SBOH approached in April 2014 to consider adding ALD to newborn screening (NBS) panel
- Internal DOH workgroup formed
- Simultaneously:
  - ✓ SBOH looking at NBS criteria
  - ✓ Federal advisory board also considering ALD
- Advisory Panel requested by SBOH
- Advisory Panel met in October 2015

# Project Schedule 2015



# ALD - Adrenoleukodystrophy

- Genetic disorder affecting brain and endocrine system
- X-linked, meaning it primarily affects males and females are carriers
- Prevalence 1:17,000 boys
- Three main forms – most serious is 1:48,500
- Laboratory test exists to screen for ALD

# SBOH Criteria for NBS Conditions

- Available screening technology
- Diagnostic testing and treatment available
- Prevention potential and medical rationale
- Public health rationale
- Cost benefit/cost effectiveness

# Available Screening Technology

- A sensitive, specific and timely screening test on dried bloodspots exists
- X-linked – girls are carriers
  - ✓ Committee decided to vote separately for boys and girls
- Four states are currently or are preparing to screen (NY, CT, CA, and NJ)

# Diagnostic Testing & Treatment Available

- Testing and treatment is available for affected individuals
  - ✓ Biochemical genetics – diagnosis
  - ✓ Endocrinology – prevent life-threatening episodes of low blood sugar
  - ✓ Neurology – regular brain imaging studies, bone-marrow transplant if changes are detected during MRIs

# Prevention Potential & Medical Rationale

- Window of time  $\geq 1$  month of age before symptoms
- Opportunity to prevent adrenal crisis for boys
- Carrier females
  - ✓ No medical rationale for detecting carriers
  - ✓ Some families may benefit from carrier identification

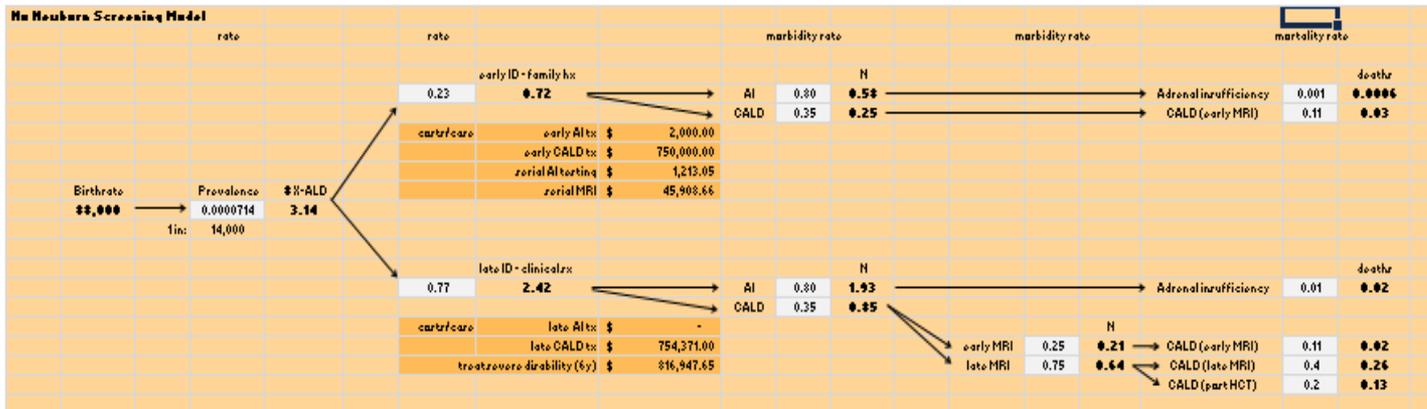
# Public Health Rationale

- Clearly met for boys - many cases have no positive family history
- Carrier females
  - ✓ Can help other males in family with ALD
  - ✓ Information to families and for future decision making

# Cost Benefit Analysis

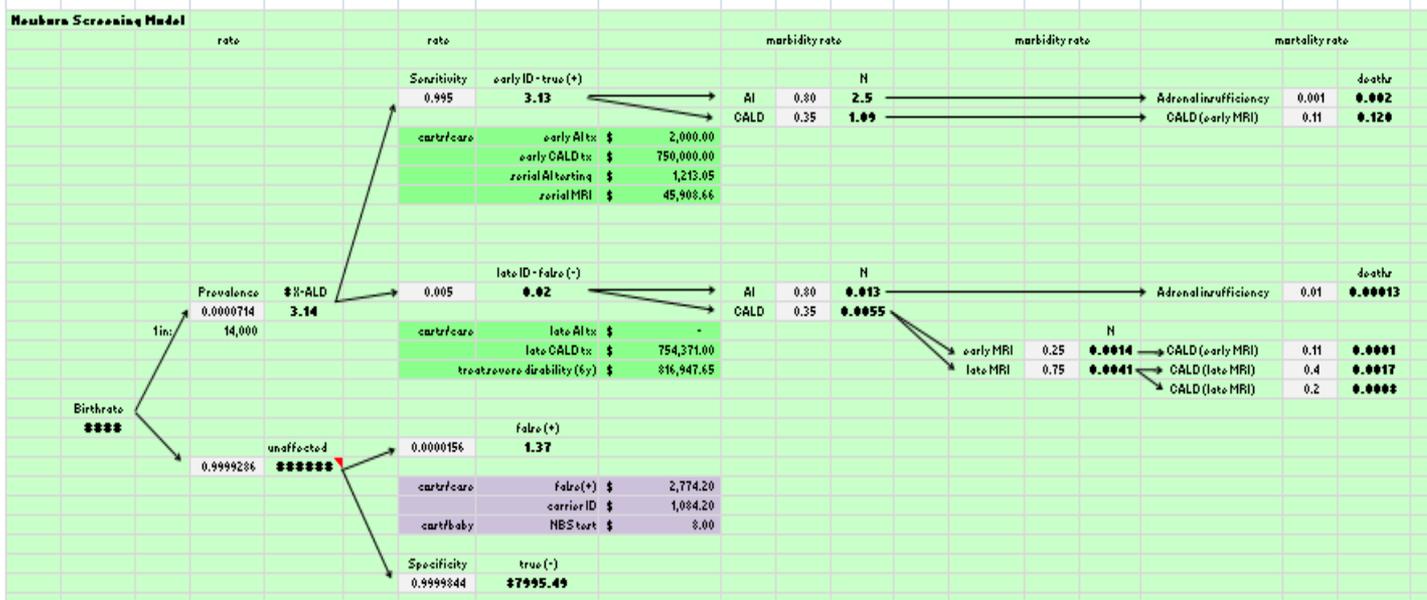
- Decision tree
  - ✓ Compares status quo v. screening model
- Data from
  - ✓ Primary literature (including pilot studies)
  - ✓ Reports from NY NBS program
  - ✓ Expert opinion
- Sensitivity analysis – vary assumptions
  - ✓ High and low estimates for parameters

# Decision Tree



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

AI - adrenalinufficiency ALD  
 CALD - (childhood) cerebral ALD



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

Benefit	death averted	0.3280
value of life saved	\$ 9,000,000.00	
value of life saved	\$ 2,951,709.78	
less treatment care	\$ (156,538.41)	
total benefit	\$ 2,795,171.38	

Cost	care of screening	\$ 704,000.00
care of false (+)	\$ 3,802.70	
total care	\$ 707,802.70	

benefit/care ratio: 3.95  
 net benefit: \$ 1,817,912.78  
 ICER: \$ 88,147.09

# Costs & Benefit Results

- Deaths averted = 0.32 (annual)
    - ✓ Value of lives saved = \$2.9 million
  - Shift in costs of treatment = \$156,000 (more)
  - Cost of screening = \$700,000
  - Costs of false(+) = \$4,000
- 
- » Benefit/cost ratio = 3.95
  - » Net benefit = \$1.8 million
  - » ICER = \$88,000

# Advisory Panel Recommendation

Criteria met?	Males		Females	
	Yes	No	Yes	No
Screening available	18	0	18	0
Diagnostic testing and treatment available	18	0	9	4
Prevention potential and medical rationale	18	0	10	5
Public health rationale	18	0	14	1
Cost-benefit	18	0	10	2

# Universal Screening?

	Vote Count
Universal (males and females)	15
Males only	3
No screening	0

# Summary Recommendations

- Add to panel – meets criteria
- Universal screening (males and females)
  - ✓ No room for error – no missed cases
  - ✓ Cost-benefit analysis based on universal
  - ✓ Performed by other states adopting
  - ✓ More efficient in laboratory – reduced costs

# Advisory Panel

Patricia Fechner, MD, Seattle Children's

Rachel Fordham, Parent & Community Member

Jim Freeburg, WA OSPI

Peggy Harris, Save Babies Through Screening Foundation

Nancy Hite, HCA

Verni Jogaratnam, MD, United Healthcare

Gina Legaz, MPH, March of Dimes

Kathy Leppig, MD, Group Health

Kathy Lofy, MD, DOH

Lawrence Merritt, MD, Seattle Children's

Kathy Ormsby, MSN, ARNP, FNP, WA Nurses Association

Tom Pendergrass, MD, Seattle Children's & WSBOH

Lani Spencer, Amerigroup

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Hannah Tully, MD, Seattle Children's

Ben Wilfond, MD, Seattle Children's

Diana Yu, MD, Mason County Local Health Officer & WSBOH

Bradford Zakes, Zakes Foundation