

Newborn Screening Advisory Committee

Thursday, April 6, 2023
11:00 am - 1:30 pm



Role Call / Introductions

1. Name
2. Organization
3. Role
4. Physical Description (e.g. age, skin color, gender, hairstyle and hair color, clothes description, any distinctive accessories)*

*Please include a physical description of yourself for meeting participants who may be visually impaired. Share only those attributes you feel comfortable sharing. Thank you!

Voting Members

- Dr. Abe Elias
- Dr. Allison Young
- Amanda Osborne
- Jennifer Banna
- Kotie Dunmire
- Marion Rudek
- Miranda Prevel
- Sarah Sullivan
- Shelly Eagen

Non-Voting Members

- Amber Bell
- Crystal Fortune
- Margaret Cook-Shimanek

Agenda

11:00a - 11:10a

Welcome & Roll Call

- Voting & Non-Voting Members

11:10a - 11:20a

Unfinished Business

- Internal Committee Updates
- Krabbe follow up

11:20a - 11:35a

x-ALD Nomination Packet Review

11:35a - 11:55a

x-ALD Nomination Packet Discussion

11:55a - 12:15p

Clinical Background from SME

Agenda

12:15p - 12:25p

x-ALD Family Story

12:25p - 12:35p

Laboratory Background

- Montana Public Health Laboratory Screening Methods
-

12:35p - 1:15p

Discussion Period

1:15p - 1:25p

Public Comment Period

1:25p - 1:30p

Newborn Screening Advisory Committee Next Steps & Wrap Up

- Fall meeting: vote to recommend x-ALD
-

Public Comment Period (10 minutes)

- Moderator will announce comment period
- Use “raise hand” feature
- Moderator will call your name
- Unmute yourself
- 2 minute max per comment
- Please email additional comments up to 1 hour after meeting ends to:
HHSNewbornAdvisoryCommittee@mt.gov

Ground Rules

- Mute
- Video
- Clarifying questions
- Avoid interrupting
- Avoid acronyms
- Use specific examples
- Focus on the collective interests and goals
- Additional meetings or communications may be scheduled
- Next steps assigned to ensure accountability
- Facilitators may call on attendees for input
- Safe space



Unfinished Business

Internal Committee Updates

- All Statement by Members received
- Chairperson Shelly Eagen will maintain Committee positions after job change
- Nomination packet available to the public
- Meeting time concerns for clinicians

Krabbe Disease Follow Up

- Decision memo was drafted and sent to the DPHHS Director
- Director Brereton agreed with the Committee's recommendation to not include Krabbe on MT NBS panel
- This decision has been made publicly available via the NBS Advisory Committee website, condition nomination status table

x-ALD Nomination Packet Review

Packet Response Overview

Symptoms and age of onset

- 4-10 years old
- Health regression due to brain damage (demyelination)
- Addison's Disease

How is this disorder currently identified?

- Blood test
- MRI

Why should it be screened at birth

- 1/15,000 births with RUSP inclusion – screening & treatment available

Packet Response Overview (continued)

How is this disorder treated?

- Is there a treatment available?
 - Yes – various (steroids, HSCT, gene therapy)
- Is the treatment in the experimental phase?
 - No – gene therapy treatment is FDA approved

Proposed screening test method

- C26:0-LPC test – 3 tier method

Packet Response Overview (continued, final)

Status of condition in the United States

- States currently screening for the condition - 33
- Registries or databases currently established for the condition - 2

Selection Criteria

Selection Criteria – As Shown on Nomination Packet

	True	Unsure	No
1. It can be identified at a period of time (24 to 48 hours after birth) at which it would not ordinarily be clinically detected.	X		
2. A test with appropriate sensitivity and specificity is available.	X		
3. There is a significant risk of illness, disability, or death if babies are not treated promptly (within the recommended time frame for the condition).	X		
4. Effective treatment is available and access to follow-up care and counseling is generally available.	X		
5. There are demonstrated benefits of early detection, timely intervention, and efficacious treatment.	X		
6. The benefits to babies and to society outweigh the risks and burdens of screening and treatment.	X		

Selection Criteria (continued)

Selection Criteria - As Shown on Nomination Packet			
	True	Unsure	No
7. There are minimal financial impacts on the family.	X		
8. There is a public health benefit to conducting the test.	X		
9. There exist responsible parties who will follow up with families and implement necessary interventions.	X		
10. The condition's case definition and spectrum are well described.	X		
11. FOR LAB USE ONLY - The public health laboratory can support the testing resources and expertise necessary to provide accurate and timely results.		X	



**x-ALD Nomination Packet Discussion
(15 minutes)**



**Clinical Background from Subject
Matter Expert
Dr. Van Haren**





x-ALD Family Story

Kirsten Finn



Laboratory Background

X-ALD Cost Analysis

MTPHL Up-front	Estimate	MTPHL annual	Estimate	Ongoing	Estimate
Additional clinical laboratory scientist (CLS) salary and benefits	~\$100,000*	MS/MS service agreement (charge per year)	\$28,500	Screening fee increase	Approximately \$15.00/sample
Training and travel expense for two CLSs	\$10,000	\$6.40/test/1,300 samples per month (15,600/year)	\$99,840	Sample referral to reference lab (if not tested at MTPHL)	Currently \$36.72; \$41.72*/15,600 screens /year= \$650,832
Tandem mass spectrometry (MS/MS) instrument	\$285,000	Consumable laboratory items	~\$2,000*		
Validation cost ~1,300 samples per month for six months at \$5.00*/sample	\$39,000 (6 month time frame)	Total	\$129,840		
Total	\$434,000*				

*Estimated cost, subject to annual increase

Discussion

Public Comment Period (10 minutes)

- Moderator will announce comment period
- Use “raise hand” feature
- Moderator will call your name
- Unmute yourself
- 2 minute max per comment
- Please email additional comments up to 1 hour after meeting ends to:
HHSNewbornAdvisoryCommittee@mt.gov

Next Steps

- Follow Up from this Meeting
 - Meeting materials will be shared
 - Public website will be updated
- Next Meeting
 - Date to be determined: x-ALD Discussion & Vote
 - Doodle Poll coming

Follow Up & Thank You

Please email if you have any questions, comments, or need anything

HHSNewbornAdvisoryCommittee@mt.gov