



**NBS Advisory Committee Meeting  
MINUTES**

Wednesday, December 21, 2022  
12:00 p.m. – 2:00 p.m.

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## Attendees

### **Voting Advisory Committee Members Present**

<b>Voting Advisory Committee</b>	<b>Position</b>
Abdallah "Abe" Elias	Director of Medical Genetics and Clinical Geneticist, Shodair Children's Hospital
Allison Young	Pediatrician, Western Montana Clinic
Amanda Osborne	Licensed, Certified Professional Midwife, Helena Birth Studio
Jennifer Banna	Center Coordinator, Family to Family Parent of child with rare metabolic disorder
Kotie Dunmire	High School Business and Special Ed Teacher, Butte High School Parent of child with Cystic Fibrosis and PKU
Marion Rudek	Nurse Practitioner, Blackfeet Community Hospital
Shelly Eagen	Nurse Practitioner, Pediatric Pulmonary, Billings Clinic
Sarah Sullivan	RN, Parent to two children with homocystinuria

### **Voting Advisory Committee Members Absent**

<b>Voting Advisory Committee</b>	<b>Position</b>
Miranda Prevel	EPSDT Program Specialist, DPHHS

### **Non-Voting Advisory Committee Members**

<b>Non-Voting Advisory Committee</b>	<b>Position</b>
Amber Bell	Section Supervisor, Children's Special Health Services, DPHHS
Crystal Fortune	NBS Follow Up Coordinator, Montana Public Health Laboratory, DPHHS
Debbie Gibson	Lab Services Bureau Chief, Montana Public Health Laboratory, DPHHS
Jacqueline Isaly (Absent)	Family and Community Health Bureau Chief, DPHHS
Margaret Cook-Shimanek (Absent)	Acting State Medical Officer, DPHHS

### **Facilitators**

<b>Facilitator</b>	<b>Position</b>
Kirsten Krane	Co-founder, Yarrow

Krystal Bosenbark	Public Health Specialist, Yarrow
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**Children's Special Health Services Support Staff**

<b>CSHS support</b>	Position
Leanna Schearer	Program Assistant, CSHS

**Public**

Public	Location
Rep. Dennis Lenz	Billings
Dani Lindeman	

## Welcome & Roll Call

(Yarrow Facilitators, Voting & Non Voting Committee Members, Ground Rules)

- Yarrow welcomed the group and did roll call while leading introductions so each person could introduce themselves, providing their roles, organizations, and a description of themselves.
  - Note: physical description is requested during introductions for those that might be seeing impaired.
- Yarrow provided an overview of Agenda, Ground Rules, and guidelines for the Public Comment Period taking place before the vote on Krabbe.
- Yarrow reviewed the number of members needed to reach quorum and hold a vote for reviewed documents. At the time of roll call, a quorum was established for this meeting.
- Yarrow reviewed the number of conflict of interest forms received, and verbally requested that each voting Committee member share whether they declared or did not declare any conflicts of interest.
  - All voting members reported having no conflicts of interest to declare

## Unfinished Business Review

- **Review of Nomination Process Procedure Voting Results**
  - Yarrow reviewed the nomination process procedures that were introduced during the last Advisory Committee meeting
  - Yarrow read the results of the Nomination Process Procedures voting survey
    - 8 out of 9 Committee members voted to adopt the Nomination Process Procedures
    - **Motion:** Members agreed to adopt the Nomination Process Procedures
- **Overview of Nomination Process Flowchart**
  - Yarrow reviewed the nomination process, from receiving a nomination packet from a citizen or advocacy group, to the condition's placement on the NBS panel and rolled out to birthing facilities.
  - See *Appendix A* at the end of the Meeting Minutes for detailed flowchart

## Krabbe Nomination Packet Review

- **Packet Review**
  - Amber Bell led the review of the Krabbe nomination packet
    - Packet was reviewed for completeness, correctness, and if it meets the criteria.
    - Amanda Osborne asked for additional explanation of how the table outlining the Selection Criteria should be used to make or influence decisions around whether to decide to include Krabbe in the screening panel.
    - Dr. Elias reminded everyone that there is not a specific number of the Selection Criteria that need to be listed as "True" "Unsure" or "No" to influence a vote of support in one way or another.
    - Jenn Banna reminded everyone that this would normally be filled out by a family or interest group rather than the DPHHS, as it was this time.
    - Rep Dennis Lenz shared that a family member was the person who was the

impetus for the bill that established this committee.

- Amber Bell thanked Rep Lenz for putting forward this legislation and highlighted the importance of a Newborn Screening Committee.

### Krabbe Discussion

- Yarrow opened the floor for discussion of any questions or concerns about adding Krabbe to the NBS panel
  - Discussion about psychosine testing cost
    - Each psychosine test costs \$72 for the Mayo Clinic Lab to analyze.
      - This is a discounted price; the undiscounted price was \$290 per test
    - In a 12 month period, if the Montana Lab had 15 tests that needed to be analyzed, it would put the estimated cost at \$1080 for that year.
  - Discussion about the timeline for sending specimens to labs.
    - Most birthing facilities in MT will get samples to the State lab within 24 hours.
    - GALC enzyme test has to be positive to run the psychosine test.
      - If there were enough dried blood spot punch remaining, the State lab could send directly to Wisconsin immediately, and without having to have the family re-submit a sample for the psychosine test.
      - The State lab could then ship the sample to the Wisconsin lab overnight.
      - Results would come back within a couple of days.
        - This would be a total turn around time of 3-5 days.
    - Discussion around considerations of the design of the actual sample collection card to ensure that there is enough “real estate” to allow for the number of punches necessary to run all of the tests required if and when the follow up tests for psychosine are necessary.
    - Optimal timeframe for treatment for Krabbe depends on whether it is the severe form of infantile Krabbe. In this case, the stem cell transplant is ideal to conduct within 3-4 weeks (20-30 days).
    - Screening can identify later onset cases as well.
      - These later onset cases might be more responsive to the stem cell transplant, but would not need the treatment so immediately.
      - These children would screen positive initially, but not require the stem cell transplant until a later stage.
      - This would require intensive follow up.
      - This is all emerging science.
    - Pompe and some other lysosomal storage disorders are related.
      - Multiplexing is when you test for several of these similar disorders at the same time and this is available through the Wisconsin lab.
    - Treatment (stem cell transplant) still remains difficult financially and unclear medically as to whether the treatment is effective in many instances. It is different when treatment is clear and consistently impactful.

- Treatment is not available in Montana and we need to consider the cost of the testing, the impact of the false positives that will affect approximately 10 families each year, and how to balance this with the approximate 1 child who will be accurately identified in Montana over about 10 years.
- More ideal identification of Krabbe would happen in-utero so that treatment could possibly begin earlier.
  - This is not actually possible at this time.

### **Public Comment Period**

- Rep Dennis Lenz provided thanks to the committee for the deliberation and thoughtful approach to the discussion. The driver behind this legislation was a family affected by Krabbe. He recognized that there is a lot of difficult and “gray area” around testing and treatment options and lack of availability.

### **Vote on Krabbe**

- **Explanation of Voting Options**
  - Chairperson Shelly Eagen reviewed voting considerations
  - Voting members can choose to vote between the following options:
    - Yes, I recommend
    - No, I do not recommend
    - I do not have enough information to make a decision at this time
      - Shelly explained that this option means that the voting member is waiting for specific information that they know is coming, and that:
        - The conversation is expected to continue at the next / upcoming meeting
        - There needs to be a clear plan for when the nominated condition will be revisited and with what coming information - this plan must be made during this meeting, and must be addressed before moving on to the next nominated condition
      - This option is not for those who believe there just isn't enough existing data and do not want to make a decision
        - In this case, members should vote “Yes” or “No”
  - **Vote to add Krabbe to MT NBS panel:**
    - **Motion:** It was recommended that Krabbe NOT be added to the screening panel at this time.

### **Vote Record**

- Allison Young - No
- Sarah Sullivan - No
- Jenn Banna - No
- Shelly Eagen - No
- Amanda Osborne - No

- Abdallah F. Elias, MD - No
- Marion Rudek - No

**Member comments on vote:**

- Lack of access to treatment, cost on state and family, efficacy of treatment is uncertain
- I feel that we could reconsider if there are additional treatments or new science available. It seems science has not caught up yet. If selected in the future, would recommend consideration of DNA testing being done concurrently. Seems since not on the RUSP that there are legitimate concerns not to support at this time.
- Given the state of science, coupled with logistical considerations surrounding obtaining prompt effective treatment, it's a difficult no.
- The currently available evidence does not support adding this condition to the MT NMB panel at this time, in my opinion. However, this is a developing area, and I would recommend re-considering this condition when more definitive evidence will become available.
- Recommend we table for 6-12 months to consider other related conditions i.e. pompe. I do feel the information on Krabbe has been inclusive of the most current and up to date.

## **Newborn Screening Advisory Committee Next Steps, Comment Period, & Wrap Up**

### **Next Steps**

- Preparation of the following documents:
  - NBS Program will prepare a package outlining the Advisory Committee's decision and rationale
  - NBS Program will send the package to the DPHHS Director for review
- Next meeting
  - Reminder: Committee is required to meet twice per year
  - Suggested next meeting dates:
    - Spring & Fall
    - Doodle Poll to follow for selection of meeting date and time

### **Questions / Discussion:**

- Crystal Fortune asked to revisit discussion about the order the Committee would consider conditions if many were received at the same time.
  - Conditions will be considered in the order that they are submitted to the Committee, unless they had previously been slated to be "fast-tracked" per HB 423.

### **Thanks and Next Steps**

- Follow up email will be sent soon and will include:
  - Meeting minutes
  - Recording
  - Transcription
  - Presentation slides
  - Next meeting Doodle Poll
  - Post-meeting survey
- Please email if you have questions, comments, or need anything

**This meeting was concluded by Shelly Eagan at 1:31 pm on December 21, 2022, via Zoom.**



# Appendix A

## Nomination Process Flowchart

