Oregon Health Authority
Northwest Regional Newborn Bloodspot Screening Advisory Board

Meeting Summary **October 4, 2019**

Location: Zoom conference call

Attendees
Board attendees constituted a quorum.

Board Members
Anna Dennis, Representative of an advocacy association regarding newborns with medical or rare disorders
Cheryl Hanna, Representative of a statewide association of pediatricians
Joanne Rogovoy, Advocacy association regarding newborns with medical or rare disorders
Marilyn Hartzell, Person or family member of a person affected by a disorder on the Newborn Screening Panel
Philip Dauterman, Representative of an entity that contracts with NWRNBS for newborn bloodspot screening
Silke Akerson, Representative of a statewide association of midwives
Kara Stirling, Representative of a birthing center or hospital
Chris Biggs, NWRNBS Program Manager (co-chair)
Cate Wilcox, Honorary Representative
Collette Young, Honorary Representative

Absent
Deb Wetherelt, Representative of a birthing center or hospital
Jill Levy-Fisch, Representative of an advocacy association regarding newborns with medical or rare disorders
Amy Yang, Contracted medical consultant
Dana Hargunani, Representative of Medicaid or insurance industry
Wannasiri (Awe) Lapcharoensap, Representative of a statewide association of pediatricians

Staff

Chris Biggs
Nicole Galloway
John Fontana

Oregon Consensus Facilitation Team
Robin Harkless, Facilitator
Cat McGinnis, Note-taker

BOARD CHAIR AND VICE-CHAIR

Volunteers for board co-chair, Cheryl Hannah, and vice-chair, Amy Yang, were approved. Both will serve one-year terms as officers per guidance from HB 2563.

A member suggested there be a process for anonymously providing feedback to the board chairs. Another member suggested the importance of transparency. The facilitator suggested one possible approach: annual or (other interval) self-evaluation of Board including chairs and vice-chair. She suggested a subcommittee convene to flesh out the roles of chairs and vice-chair, and to establish a process for feedback and concerns. As of the meeting, the subcommittee will include Chris Biggs and Marilyn Hartzell. The facilitator offered to work with the subcommittee as needed to assist in developing proposed language for Board review and approval.

Action item: Program will send out an invitation for others who want to be on the subcommittee to clarify board roles and establish a feedback mechanism for the board. Committee will provide a draft before the next full-board meeting and will report out at the meeting.

Draft Evaluation Procedure and Criteria for Adding Disorders

Before the meeting the program had sent the board a draft of a procedure and criteria for adding disorders to Oregon's newborn bloodspot screening panel. The draft incorporated the board's discussion and proposed changes from the July 18, 2019, meeting. The Board reviewed and offered the following recommended changes -- note that the changes were adopted and the Board approved, by strong consensus, the protocol and criteria for ADDING disorders (see Appendix A: Approved Protocol and Criteria for Adding Disorders to the Bloodspot Screening Panel):

Re: Proposed Procedure for Disorder Evaluation

- **CONSENSUS: The three-stage procedure was adopted as drafted with a strong consensus of the group.**

Re: Category One Criteria *(Evaluated by the Program as Yes or No)*

- **Regarding Criteria 8**—"The specific condition appears in the funded region of the Prioritized List as determined by the Oregon Health Evidence Review Commission (HERC)."
    - Member clarified the issue is whether, if a condition was identified there would be funding to treat it. This would be a context issue.
    - When it refers to "funded region," what does this mean? One member said it doesn't refer to a geographic region, rather a 'region' of the HERC prioritized list.
    - Another said that anything above the line is funded, but below the line is a moving target. Member is concerned that not being funded should not be a hard stop for adding a disorder, but that instead need to involve HERC in a discussion.
    - The facilitator asked whether, since this criteria is more nuanced than a 'yes/no', should the criteria (category 1, criteria 8) move to category 2?
    - A member pointed out that when SMA was added in other states there was concern about insurance not covering the $2 million treatment. Due to the uproar, insurance decided to cover treatment. It was an ethical dilemma. The question is where is the line today? How far above or below the funding line is the disorder? If it's far below the line, there should be a hard stop on adding the disorder. Could leave this criteria as it is, but also want to know where the disorder is in relation to the funding line as a potential deliberation point for the Board. Thus, the Board would need the Program to report where the disorder falls relative to the funding line.
    - Another member said the board should not approve an unfunded condition. If there needs to be an ongoing discussion about adding it, that's why someone from Medicare is on the board—so it will be on their radar.

- One member said that it does more harm than good to discover a condition that can't be treated due to no funding.
- One member felt uncomfortable with including this criterion, because it is like making a decision for the parent. Member said they won't block inclusion of the criterion, however.
- Facilitator pointed out that one way to deal with it would be to put it in category 1 criteria as a yes/no and also in category 2 as a board deliberation point.
- One member expressed very strong concern about testing when there is no funding for treatment. It puts the board in a bad spot ethically. The point of medical care is to offer treatment and not do harm. Diagnosing without available funding for treatment can do harm, e.g. parents experience very high stress while waiting for follow-up test results for a condition diagnosed in the screening and then, do not know or have options.
- Another member said they value that perspective, but think, as a parent, they'd like to know if their child tested positive even if there is no funding for treatment.
- The facilitator noted that there is interest by the Board in better understanding the HERC's process for negotiating funding, particularly for disorders that teeter right at the funding line. It was suggested that board member Dana Hargunani provide more information at the next meeting.
- **AGREEMENT**: The Board agreed that the Program will evaluate this criteria as a yes/no analysis within category 1, and also report to the Board where the disorder falls relative to the funding line.
- **Action**: Board will ask a follow-up question of Dana Hargunani about the FERC process for determining where a condition will fall on the funding list.

- **Regarding Criteria 9**—"The NWRNBS Program has sufficient information to perform a fiscal analysis."
  - Member asked for clarification of "sufficient information."
  - Program explained that sometimes they can only do a "best guess" fiscal estimate. They will give the board a fiscal analysis,

but will point out the unknowns. However, program suggests that criteria 9 would be a “no” if they are not able to conduct a fiscal analysis due to insufficient information.

- **Regarding Criteria 10**—“The impact to the NWRNBS partners has been assessed.”
    - Who are the Partners? It was clarified that “Partners” refers to contracted state partners within the regional NWRNBS laboratory.
    - Agreement: “Partners” will be more clearly defined in this criteria description.

CONSENSUS: **The Board reached consensus on the recommended Criteria 1 with simple changes offered today and with a caveat that the Board will have further discussion about Criteria 8 if a condition is evaluated as a ‘no’ in this category.** Most members registered level 1 consensus, but two registered level 3 consensus. One member who registered a 3 said their concerns would be addressed by having the department explain to the board where a condition is in relation to the funding line. The other member who voted 3 had no additional comment, except to say they would not block the decision.

Re: Category Two Criteria *(Evaluated using the Consensus Method)*

- A member pointed out that criteria 6 and 7 in category 2 are related to criteria 5 and 6 in category 1.
- OHA Program staff suggested that the Program felt criteria 8, category 2, regarding equity, is best included as an item for the board to seek consensus on.
- Facilitator clarified that category one items are for program to deem yes/no. Category 2 items are for the board to seek consensus on.

CONSENSUS: The criteria in Category 2 were adopted as drafted with strong consensus.

Discussion of Protocol for Removing Conditions from Oregon’s Screening Panel

- The Board was invited to consider whether the same criteria should be used for removing conditions as will be used for adding conditions to the screening panel.
- The facilitator relayed email comments submitted from one Board member regarding concerns about using the same criteria for removing conditions, who asked these comments to be shared during the discussion. The content of that comment is as follows:
    - To remove a condition from being screened is a different process that adding. Ie, “what is benefit to NBS for a condition” is a different question than “what is to consequence of no longer screening for a condition”
    - To be considered for removal, by definition, it will not have been on the RUSP. Thus, to use the RUSP as both the initial consideration and then criteria for removal is ‘double dipping’ the use of the RUSP.
    - Thus, we must further define, beyond the RUSP, on additional criteria in which we consider a condition removable from the NBS panel. Start the conversation with “what is the consequence of removing such condition and missing that initial diagnosis?”. This leads to:
        - “What is the natural hx of disease? Is it benign or progressive?”
        - “What is the treatment, if any?” or “Is there any benefit to screening for this condition?”
        - “What is the cost of missing a diagnosis versus managing the diagnosis from the beginning?”
        - “What is the legal implication of disparate care between those children who had the benefit of screening versus those who did not?”
        - Etc…(I’m sure the group may come up w/ more questions to ask ourselves).
    - Perhaps we can then formulate those questions to more formal criteria for removal.
- Another member said there would be value in having a removal protocol that is not identical to the protocol for addition of conditions.

- The facilitator checked on urgency of this issue and suggested a subcommittee to address the protocol. A Board member requested that the Program draft language as a starting point.

Action item: The Program will draft a protocol for removing conditions based on the board's discussion and will present it to the board before the January/February meeting. Chris Biggs will take the lead and recruit additional expertise from the board.

Action item: The program will share a reference link to another program from a Board member regarding the protocol for removing conditions.

Public Comment

The facilitator opened the floor to any public callers who wished to comment. There were no comments.

Next Steps

- The next meeting will be held in **January/February 2020** and likely will be face-to-face given the Board's feedback at the July meeting. The Program staff are working to solidify a date via Doodle poll and will confirm the date as quickly as possible.
- The program has identified a medical ethicist and experts for each disorder that will be under review (XALD and SMA) at the next meeting. The experts are available to attend the meeting.
- Most of the next meeting will be reserved for the Board to get informed about XALD and SMA, and walk through their protocol to consider whether to add either of these conditions to the screening panel.
- Collette Young shared that the Program has hired a contractor to serve as an independent researcher to collate materials for the board regarding XALD and SMA.
- Regarding the Legislative Report: the program requested board comments by **October 9**. If there are substantial board edits, the redraft will be sent to the board for review. After revisions, the report will go through the OHA review process. OHA edits will only address

style/format/grammar. The Report is due to the Legislature by **December 15, 2019**.

Appendix A: **Evaluation of the Northwest Regional Newborn Bloodspot Screening Testing Panel**

The process for adding disorders to the Northwest Regional Newborn Bloodspot Screening (NWRNBS) Program testing panel has been that the NWRNBS Program and the contracted medical consultants discuss new disorders that have been reviewed by the Advisory Committee for Heritable Disorders in Newborns and Children (ACHDNC) and added to the Recommended Uniform Screening Panel (RUSP). Once conditions have been added to the RUSP, the NWRNBS Program has considered disorders ad hoc, sometimes with an Advisory Board and sometimes with Rule Advisory Committees (RACs).

Going forward, the NWRNBS Advisory Board has established a process and criteria to evaluate disorders and make recommendations for whether to add a condition to the NWRNBS Program testing panel. These recommendations will be used by the NWRNBS Program when considering new disorders for inclusion on the testing panel.

Additional information on the ACHDNC and the RUSP can be found here: https://www.hrsa.gov/advisory-committees/heritable-disorders/index.html.

Procedure for Disorder Evaluation

Stage 1: Addition to the RUSP
Disorders that have been reviewed by the ACHDNC and have been added to the RUSP will be raised for further evaluation.

Stage 2: NWRNBS Program Evaluation using Category One Criteria
After a disorder has been added to the RUSP, the NWRNBS Program will evaluate the disorder using the criteria in "Category One Criteria" *(Please see below).* This initial set of criteria will be answered using yes or no. The NWRNBS Program will share the evaluation of the Category One Criteria with the NWRNBS Advisory Board. If all criteria are answered yes, the disorder will be moved to Stage 3.

Stage 3: NWRNBS Advisory Board Evaluation and Recommendation using Category Two Criteria
Disorders that have met Category One Criteria will be brought to the NWRNBS Advisory Board for evaluation using Category Two Criteria.

These criteria will be evaluated using the consensus tool (see below). The results of this evaluation will inform the recommendations to the NWRNBS Program.

Criteria for Disorder Evaluation

Category One Criteria *(Evaluated as Yes or No)*

1. The condition is well-defined in newborns.
2. Earlier intervention results in improved outcomes compared to later identification.
3. The population level incidence and prevalence are known.
4. There is a Federal Drug Administration (FDA) approved testing method available using dried blood spots or an accurate testing method is available that meets clinical laboratory requirements for validation and testing by the laboratory using dried blood spots.
5. Diagnostic and specialty testing is available.
6. A treatment is available.
7. The contracted NWRNBS medical consultants have been consulted and appropriate specialized medical consultation is available or can be obtained by the Program.
8. The specific condition appears in the funded region of the Prioritized List as determined by the Oregon Health Evidence Review Commission.
9. The NWRNBS Program has sufficient information to perform a fiscal analysis.
10. The impact to the NWRNBS contracted partners has been assessed.

Category Two Criteria *(Evaluated using the Consensus Method)*

1. The population level public health benefits of screening outweigh the risks and harms.
2. There is adequate capacity and expertise in the NWRNBS program to implement and maintain testing and reporting.
3. There is adequate capacity and expertise in the NWRNBS program to implement and maintain follow-up and education for providers and parents.

4. The NWRNBS Program has adequate fiscal resources for implementing the test, performing the test and conducting follow-up and education.
5. The population level incidence, prevalence and disease burden are significant enough to merit screening.
6. Diagnostic and specialty testing is available and accessible that allows a definitive diagnosis to be made.
7. An effective treatment that is proven to result in clinically significant benefits is available and accessible.
8. There is equitable care and treatment for the disorder.
9. Addition of the disorder is not prohibitive to NWRNBS contracted partners.