Oregon Health Authority
Northwest Regional Newborn Bloodspot Screening Advisory Board

Meeting Summary July 18, 2019

Location: Oregon State Public Health Lab, Hillsboro, Oregon

Attendees
Board attendees constituted a quorum.

Board Members
Amy Yang, Contracted medical consultant
Anna Dennis, Advocacy association regarding newborns with medical or rare disorders
Cheryl Hanna, Representative of a statewide association of pediatricians
Dana Hargunani, Medicaid or insurance industry
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, Entity that contracts with NWRNBS for newborn bloodspot screening (on phone)
Silke Akerson, Representative of a statewide association of midwives
Wannasiri (Awe) Lapcharoensap, Representative of a statewide association of pediatricians
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, Advocacy association regarding newborns with medical or rare disorders
Kara Stirling, Representative of a birthing center or hospital

Staff
Chris Biggs, NWRNBS Program Manager
Nicole Galloway, Laboratory Business Engagement Policy Analyst
John Fontana, Laboratory Director
Matt Ulsh, Laboratory Contract Coordinator

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

ACTION ITEMS
- The Board will send their nominations for chair and vice chair to Chris Biggs by August 18, 2019.
- Staff will distribute the following documents to the Board: the National and Oregon Context PPT slide show, Board work plan, and NWRNBS organizational chart.

- Staff will revise draft criteria according to Board discussion and send updated language to Board. Board will review and seek consensus on the criteria in a phone conference (open to the public) before the legislative report is finalized. Staff will send materials for the January meeting to the board three weeks before meeting. Staff will send out specific instructions for joining the next meeting by phone or Zoom.
- Staff will line up needed experts in advance of Board's assessment of conditions when additions to test panel are under consideration.
- Board would like more information regarding capacity for follow-up. NWRNBS will draft an analysis and proposals for discussion by the Board.

1. Introductions
Attendees introduced themselves. Note: This was the first meeting of the advisory board.

2. Getting grounded in the process—review and refine draft Advisory Board Charter
Review of HB 2563. The board reviewed the requirements of HB 2563, which establishes the board and prescribes composition of board, terms of office, meeting requirements, other governance issues and requirements to report to the legislature.

Review of draft charter. The board reviewed a board charter drafted by the NWRNBS program staff. The charter reflects requirements in HB 2563 and additional governance issues such as decision making and values. Discussion included the following:

- Role of vice-chair was clarified to include stepping in to help with chair duties if chair is unavailable to assist.
- Co-chairs and vice chair may interface with the legislature alongside OHA program staff. (One Chair, designated by HB 2563, is the NWRNBS Program Manager.)
- The charter is a "living document" that can be revised by the board as needed.
- The group will add more information about chair and vice-chair roles as the board evolves.

  Agreement: The Board expressed strong consensus for using a consensus-seeking decision-making process for all board decisions. Members will rate their level of consensus on a scale of 1-5, where 1 is enthusiastic agreement with the proposal/recommendation; 2 is agreement; 3 is I am on the fence, have questions, or am neutral but can live with the proposal; 4 is I have serious questions or concerns, but am not willing to block the proposal; and 5 is I object and will block the proposal. A strong consensus is when all members show a 1 or 2. Weak consensus is when some board members show 3 or 4. If anyone in the group shows 5, there is no consensus. When there is weak consensus or no consensus, points of divergence will be documented in general terms in meeting summaries. Board members will submit any points of divergence or concern about a decision in writing for inclusion in legislative reports.

  Agreement: There was strong consensus in support of the board values included in the draft charter.

  Agreement: The entire charter as drafted was adopted with a strong consensus by Board members present at the meeting.

  Action item: The board will send their nominations (self or others) for chair and vice chair to Chris Biggs by **August 18, 2019**. Chair and vice chair will be approved at a future Board meeting.

3. Getting grounded in the substance—National and Oregon context
Chris Biggs, NWRNBS Program Manager, presented information to the Board. Her PPT slides will be made available to the Advisory Board after the meeting.

National context.

- NWRNBS staff reported that more than 4,000,000 infants are born each year nationally and over 12,000 infants are born with a condition requiring early detection and intervention to prevent serious illness or death.
- Newborn screening is just the first step in determining whether an infant has a condition. To confirm disorders, diagnostic testing is needed.
- Since it is screening both false positive and false negative results will occur.
- Information about the history of newborn bloodspot screening nationally was provided.
- The process for adding disorders to the national Recommended Uniform Screening Panel (RUSP) was explained.
- Currently the RUSP has 34 core conditions and 26 secondary conditions.
- Current trends and influences on screening were discussed. They include timeliness of screening. Information on the 2016 GAO report was presented. Labs are encouraged to reach 95% for each of the following timeliness goals:
  - Birth to collection—48 hours
  - Collection to receipt—24 hours
  - Laboratory testing and reporting—not defined
- Very few states met the benchmarks in 2015, and none met the benchmark of receiving samples in the lab within 24 hours of collection.
- Timeliness barriers included:
  - Lack of understanding of timely screening
  - Lack of standardization in laboratory information systems
  - Lack of courier or expedited shipping methods used to transport specimens
  - Lab operating hours
  - Lack of feedback for submitters
  - Reliance on mail for results
- Other trends and influences on screening include:
  - Changes in testing technology, which require new forms of lab expertise and staffing
  - New disorders—the pace of adding new disorders has increased, as has the complexity of the disorders and testing
  - The digital age is presenting new opportunities for electronic communication of orders and results, and new ways of analyzing data
  - Screening issues are increasing in visibility—parents now have a voice, pharmaceutical companies and manufacturers are becoming more involved, lab staff now work in an environment of increased sensitivity to public perception and lawsuits.

National context Q&A.

- Board member: How many parents opt out of screening? Staff: Very few.
- Board member: How does Oregon rank as far as the number of screened disorders? Staff: It's hard to say since each program 'counts' a little differently.
- Board member: How many screening programs are there nationally? Staff: There are approximately 38-40, and few of those are regional like Oregon's.
- Board member: Do the states/regions that Oregon does screening for screen for different disorders? Staff: No, currently the same panel is used for all. Except for LSDs- Update

- Board member: Who pays for screening? Staff: Oregon pre-delivers kits to providers who pay for the kits in advance, so the lab has no need for billing infrastructure. Insurers or parents pay for the screen. Screening cannot be denied due to inability to pay.
- (***Facilitator's Note***: the following comments and questions signal an interest and need by the Board for more information and discussion.)
    - Board member: How does the lab determine that a sample is unsatisfactory? Staff: There are different factors, for example blood on top of blood is "layered" and unsatisfactory; too little is also unsatisfactory. Board member: I collect samples and have found the determination seemingly arbitrary. I feel there needs to be more education about what makes a sample unsatisfactory.
    - Board member: Is there any evidence of improvement where a nurse educator has visited a facility regarding how to collect samples? Staff: There is anecdotal evidence of improvement. Providers are sent a report about the quality of their submissions.
    - Board member: What kind of education is needed on how to collect satisfactory samples? Staff: While education is needed, the program currently doesn't have capacity to do much of this work.

Oregon context.

- NWRNBS is governed by statutes ORS 433.285, 433.290, and 433.295, which were established in 1963. It has been 20 years since the statute was thoroughly updated to reflect current best practices. Oregon Administrative Rules 333-024-1000 to 333-024-1110 describe how the program implements the statute. Rules were revised in 2019 and additional revisions are being considered now.
- Details about the program:
    - It screens for Oregon, Idaho, New Mexico, Hawaii, Guam, Saipan, parts of the Navajo nation and several military bases around the world.
    - It tests samples collected about 24 hours after birth and again at 10–14 days after birth.
    - It screens about 110,000 infants each year, which requires about 10,000,000 tests to be performed.
    - It screens for all conditions on the RUSP, except X-ALD and SMA.
    - It provides follow-up for abnormal results and unsatisfactory specimens, and education for providers and families.
    - The lab currently has 12 testing personnel and 4 follow-up staff. Unlike most other programs, the lab does not have a nurse or genetic counselor to do follow-up.
    - Adding new staff requires legislative approval.
    - The program is totally fee-funded with no state or federal funds.
    - Historically it takes the lab 2–4 years to begin testing for a condition after the condition is added to the RUSP (2.5 years is national average). Before a condition can be tested for, the lab needs to assess staffing and contract with additional experts to provide follow-up. The lab needs to locate an effective test. They need to obtain equipment (through the state procurement process) or special expertise to perform the test. They often can't afford the equipment until a legislative fee change is in place. A rule change is required. They must validate that the testing works and how the results will be reported. Procedures need to be documented and approved. Educational materials need to be developed. The Practitioner's Manual needs to be revised. Providers need to be informed of the change.

<u>Timeliness in the NWRNBS program</u>

The US Government Accountability Office (GAO) established 3 stages for the newborn bloodspot screening process and established the following timeliness goals for each:

Stage 1: Birth to specimen collection—95% within 48 hours of birth
Stage 2: Specimen collection to lab arrival—95% within 24 hours of collection
Stage 3: Lab arrival to results reporting—unspecified goal

Overall timeliness goals were that all time-critical results be reported within 5 days of birth and all results within 7 days of birth.

Chris Biggs shared specific information about the Oregon Regional Lab related to these national timeliness goals.

- The program is at 95% meeting timeliness goals for birth to collection in 48 hours, 30% for collection to receipt in the lab within 24 hours, and 71% for receipt in the laboratory to reporting results to program within 3 days of receipt and 96% within 6 days of receipt. The "all results within 7 days of birth" benchmark is at 90%. Time-critical results within 5 days of birth varies because of small numbers but was at 38% for the first 6 months of 2019.
- The lab is currently doing the following continuous quality improvements:
  - Adding a partial Saturday shift to increase timeliness.
  - Working on decreasing the rate of unsatisfactory specimens.
  - Reworking the practice profile ("report card") they send providers monthly to make it more useful.
  - Improving the website
  - Using data more effectively
- Possible quality improvement projects to improve timeliness include:
  - Further increase operating hours to include a full Saturday shift.
  - Add courier service or expedited shipping to get specimens on time.
  - Implement electronic orders and results.
  - Increase outreach and education for providers and families.

Action item: Staff will send the National and Oregon Context slideshows to the Board.

4. Public comment period
Commenter: Ashley Monaco

Shared appreciation for the work the Board will be doing on behalf of Oregon citizens. Shared that their son was diagnosed with Krabbe disease in 2016. They became advocates for newborn testing for Krabbe. Their son's story was an impetus for HB 2563. They requested that Oregon add Krabbe to its testing profile to spare other families from similar suffering.

5. Criteria for adding new conditions to Oregon's screening panel
For the board's consideration, staff provided a draft protocol and criteria for deciding whether to add a condition to Oregon's newborn bloodspot screening panel. The board reviewed and discussed the protocol and criteria. With this input, staff agreed to update the criteria and revisit with the Board during a conference call prior to submitting their report to the Legislature.

Facilitator summary of discussion points: Board values around the protocol and criteria are efficiency, timeliness, and transparency; suggestion to add an 'impact' criteria regarding specific populations

(equity-focus); and consider in initial review the impact on other jurisdictions that are part of the Regional screening lab. Board members also discussed the need for the Program to share relevant information -- including fiscal impacts-- as well as provide experts, to inform Board deliberations.

Summary of Board's discussion of protocol and criteria:

- Board member: Supply fiscal impact of adding a test to the panel. Under criteria category 1, item 8, add cost to do the test.
- Board member: Is there a process for stage 1 that specifies how long the lab has to respond to something that's added to the RUSP? Staff: Currently the lab starts thinking about the test as soon as it is added to the RUSP. Historically it has taken 2–4 years to add the test in Oregon after it's added to the RUSP.
- Board member: Category 2 criteria (board evaluation of condition), item 5, related to prevalence and significance of condition in population—is this a values decision for the board? Staff: Yes. Board may want to bring in a medical ethicist to help with these values decisions.
- Board member explained the process by which the Health Evidence Review Commission determines what Medicaid covers based on a prioritized list.
- Board member: It would be poor ethics to include a condition that wasn't covered by Oregon Health Plan.
- Board member: Whenever a condition appears on the RUSP, bring it to the board for review regardless of how many "yes" answers there are in the lab's stage one review of conditions.
- Staff: Lab staff attends national conferences and can sometimes predict what will be added to the RUSP. Board member: Let board know about developing issues when possible. Board might like to get a jump on RUSP conditions concurrently with the RUSP process in order to expedite addition to the Oregon panel.
- Board member: Add a criteria to stage 2 (board's criteria for evaluation of condition), that there should be *access* to diagnosis and effective treatment. Note that sometimes there is only one provider in the state who handles a condition, so access for many people will be limited. Could put treatment availability in stage 1 review and treatment accessibility in stage 2 review.
- Board member: How robust should category 1 review be before evaluation of condition moves on to stage 2? Board member: Category 1, items 1-6, will all be "yes" if the condition is on the RUSP. However, items 7 and 8 are grayer. Staff: Item 6 is also gray and the board will know better than staff if treatment is effective—e.g., effective 40% of the time or 90% of the time.
- Board member: Lab assess category 1, items 1–8 within 6 months of condition being added to the RUSP and frame up gray areas for board review.
- Staff: RUSP may expand quickly as treatments are developed. Board may want to revise review criteria in the future.

  Action item: Staff will line up needed experts in advance of board's category 2 assessment of conditions when additions to test panel are under consideration.

  Action item: Staff will revise draft criteria according to board discussion. Board will review and seek consensus on the criteria in a phone conference (open to the public) before the legislative report is finalized. Staff will work to schedule this in August.

6. Recommendations to Legislature regarding timeliness of screening

Program staff shared a series of potential recommendations to the Legislature that relate specifically to the national timeliness goals and current realities of the NWRNBS Program which were shared during

the morning presentations. Staff suggested that the advisory board use the GAO stages as the basis for the legislative recommendations for inclusion in the December 15, 2019, legislative report. The board agreed, by strong consensus, to recommend the following priority needs for inclusion in the legislative report:

- Increase the number of satisfactory specimens. Set a standard of excellence. (Currently the NWRNBS is at 4% unsatisfactory specimens compared to national average of 2%.)
- Explore the cost/benefit of providing courier service or expedited shipping.
- Electronic ordering and reporting is a high need and should be a priority for the Program. *Possible report language: Electronic exchange is critical and is a priority need. The program needs resources to build infrastructure and possibly FTE to manage the exchange.*
- The statute, 20 years dated, needs to be updated to reflect current best practices.

Action item: The Board requested additional information regarding capacity for follow-up. NWRNBS will bring the board an analysis and proposals.

7. Looking ahead: Advisory board next steps and action items

The Board agreed to meet face-to-face all day for their next 6-month meeting in early 2020. Staff will send the next meeting's materials to the Board three weeks before the meeting.

Staff will distribute the board's current draft work plan which was shared as a slide during the wrap up section of today's meeting. They will also share the Lab's organizational chart per a request by a Board member.

8. Rules advisory committee review

The advisory board stepped into its role as NWRNBS's rule advisory committee. Staff reviewed several suggested rules revisions and Board members were given an opportunity to comment on each change. The following summarizes this review:

- Proposed rule changes are slated to take effect in October 2019.
- Several rule changes are non-substantive. They revise grammar, referenced statutory authority, and edition of the Oregon Newborn Bloodspot Screening Practitioner's Manual.
- Definition of "low birth weight" [OAR 333-024-1010(7)] proposed for removal. The term is being removed in proposed rule language changes.
    - No comment from Board members.
- Definition of "premature" [OAR 333-024-1010(12)] proposed for removal. The term is not used elsewhere in the rule.
    - No comment from Board members.
- Proposed change to 333-024-1030 (3) to require third specimens for babies admitted to the NICU whose birth weight is 2000 grams or less. Weight was formerly 2500 grams. The change would reduce the number of third collections required.
    - Staff will remove new language "special care baby unit" based on Board's information that no such units exist in Oregon.
    - Proposed language will be revised to correct grammatical problems to clarify whom the provision applies to.
- Proposed change to 333-024-1070 (6) to add fluorescent immunoassay as a testing method for Biotinidase deficiency.
    - No comments from Board members.

- Proposed change to 333-024-1070(10)(c) and (d) would eliminate disorders Fabry and Gaucher from screening panel. Neither condition is on the RUSP. Fabry creates many false positive results. Gaucher is easy for a pediatrician to diagnose. Tests were initially added to verify Pompe and MPS-1, which now have second-tier DNA tests. NWRNBS believe there is no longer a need for the Fabry and Gaucher testing to assist with Pompe and MPS-1 and there is a large amount of follow-up work associated with Fabry and Gaucher.
    - Not all Board members were comfortable with this rule change. Some Board members suggested development of criteria for removing screens from the panel. More discussion may be needed.
- Proposed change to 333-024-1090 to revise the specimen retention time from one year to eighteen months to align specimen retention times throughout the NWRNBS Program.
    - No comment from Board members.
- Board reviewed the statement of need and fiscal impact for the proposed rule change.

After final housekeeping comments to set up for the next steps in the Board's work, the meeting was adjourned.