Secretary's Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children

Summary of 12th Meeting by Conference Call
Nov. 14, 2007
Washington, DC

The Secretary's Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children conducted a special two-hour meeting by conference call beginning at 2 p.m. on Wednesday, Nov. 14, 2007. In accordance with the provisions of Public Law 92-463, the meeting was open for public comments.

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I. WELCOME, OPENING REMARKS

R. Rodney Howell, M.D.
Chair, Secretary's Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children
Professor, Department of Pediatrics
Leonard M. Miller School of Medicine
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Dr. Howell explained that the special two-hour conference call of the Advisory Committee had two primary purposes: (1) to advance the Advisory Committee's process for nominating/evaluating conditions for inclusion on the uniform newborn screening panel; and (2) to review and vote on revisions to a proposed statement by the Advisory Committee on long-term followup following diagnosis resulting from newborn screening.

Dr. Howell explained that there would be two proposals related to the Advisory Committee's process for nominating/evaluating conditions for inclusion on the uniform newborn screening panel:

- Dr. Nancy Green, the chair of the Nomination Review and Prioritization Workgroup (NRPW) established by Dr. Howell at the September 2007 meeting, would present recommended criteria for determining whether a nominated condition is ready for an external evidence-based review and for handling the prioritization of multiple nominations.
- Dr. James Perrin, the chair of the external Evidence Review Workgroup (ERW), would present plans for the ERW and its approach to reviewing and reporting on the evidence related to conditions nominated for inclusion on the uniform newborn screening panel. He had presented preliminary plans at the September 2007 meeting of the Advisory Committee.

Following these presentations, Dr. Alex Kemper would present for the Advisory Committee's final review and approval a revised version of a statement from the Advisory Committee on long-term followup after diagnosis resulting from newborn screening. The Advisory Committee had approved the statement in concept at its September 2007 meeting, with the understanding that the changes discussed at that meeting would be incorporated and there would be vote on the revised statement as soon as possible.

Dr. Howell also reported that the Advisory Committee had several new members who would be recognized again at the next meeting: Rebecca Buckley, M.D., from Duke University; Ned Calonge, M.D., M.P.H., from the Colorado Department of Public Health and Environment; Tracy Trotter, M.D., from the San Ramon Valley Primary Care Medical Group in California; and Gerard Vockley, M.D., Ph.D., from the University of Pittsburgh School of Medicine.

II. RECOMMENDATIONS OF THE NOMINATION REVIEW AND PRIORITIZATION WORKGROUP (NRPW)

Nancy S. Green, M.D.
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The Nomination Review and Prioritization Workgroup (NRPW), chaired by Dr. Green, was created by Dr. Howell at the September 2007 Advisory Committee meeting (1) to develop criteria to determine the readiness for referral to the external Evidence Review Workgroup (ERW) headed by Dr. Perrin of nominations of conditions to be added to the uniform newborn screening panel; and (2) to develop criteria regarding the prioritization (if any) of nominations received from the Health Resources and Services Administration (HRSA). The workgroup consisted of Dr. Nancy Green (chair), Dr. Amy Brower, Dr. Rodney Howell, and Dr. Marie Mann from HRSA.

Dr. Green reported that the NRPW met by conference call on Oct. 25, 2007, and unanimously agreed to recommend that when selecting nominated conditions for consideration for inclusion on the uniform newborn screening panel, priorities for review by the Advisory Committee and the ERW should be based on all of the following criteria:

- Nominated conditions are medically serious.
- Disorders for which pilot data (U.S., international, and/or other) are available for population-based assessment.
- The spectrum of the disorder is well described, to help predict the phenotypic range of those children who will be identified based on population-based screening.
- The characteristics of the screening test(s) suggest a reasonable rate of case ascertainment —that is, the ratio of false to true positives (e.g., not 1000:1) (and low rate of false negatives).
- If the spectrum of disease is broad, those who are most likely to benefit from treatment are identifiable—especially if Rx is onerous or risky.
- Treatment is well described, Food and Drug Administration (FDA) approved/cleared (if applicable for a drug or device), widely available.

The NRPW also recommended that HRSA send formal letters to the nominator(s) following administrative review if HRSA does not forward a nomination to the Advisory Committee for formal consideration and that it provide supportive information regarding areas of the submission that are deficient.

Questions & Comments

Steps in the process for deciding which conditions go for evidence-based review. HRSA's Maternal and Child Health Bureau has received three nominations of conditions as candidates for inclusion on the uniform newborn screening panel: Pompe disease, Krabbe disease, and severe combined immune deficiency. In response to requests for clarification of the overall process for deciding which conditions should go for evidence-based review, Dr. van Dyck outlined the

following sequence:

1. HRSA performs an administrative review of any nomination form(s) submitted to ensure that the form is complete.
2. If HRSA finds a nomination form to be complete, it forwards the nomination to the Advisory Committee's chair Dr. Howell. (If HRSA finds a nomination form to be deficient, it returns the form to the nominator with information regarding areas of the submission deemed deficient.)
3. Dr. Howell forwards the nomination form(s) from HRSA to the NRPW.
4. The NRPW reviews the nomination(s) and subsequently makes a report (including a brief analysis and recommendations) to the Advisory Committee about whether the nomination(s) is ready to be sent for an evidence-based review by the ERW and about the prioritization of nominations.
5. The Advisory Committee votes on whether to send the nomination(s) for an evidence-based review by the ERW (or to request additional information) and also establishes which nominations should have priority for evidence-based reviews.

Prioritization of nominations. Dr. Boyle observed that prioritizing nominations for evidence-based reviews by reranking conditions every time a new nomination would be very complicated. As an alternative, she suggested using the chronological receipt of a nomination by HRSA as the basis for a condition's placement in the queue for evidence-based reviews, unless there were unusual circumstances that warranted moving a condition ahead in the queue. Other Advisory Committee members agreed that this approach should be used.

Modifications to proposed criteria. In response to a question, Dr. Howell clarified that it is not imperative that a condition meet all of the criteria proposed by the NRPW to get in the queue for evidence review. Some Advisory Committee members suggested changes to specific criteria proposed by the NRPW:

- *Disorders for which pilot data (U.S., international, and/or other) are available for population-based assessment*. Dr. Rinaldo suggested adding "*prospective*" before the word pilot.
- *The characteristics of the screening test(s) suggests a reasonable rate of case ascertainment —that is, the ratio of false to true positives (e.g., not 1000:1) (and low rate of false negatives).* Dr. Skeels suggested narrowing the ratio of false to true positives to "no more than 10:1, with few false negatives." Dr. Perrin said that asking for extremely explicit differential criteria would slow things down. He suggested not qualifying the ratio of false to true positives at all, but merely saying that a screening test exists that is capable of identifying children at risk for disorder, then allowing the ERW to present evidence regarding the screening test.
- *Treatment is well described, & FDA approved/cleared (if applicable for a drug or device), widely available.* Dr. Rinaldo proposed substituting "There are defined treatment algorithms or protocols" for "Treatment is well described," because he thought the latter was too vague to help prioritize nominations. In response to a question about what was meant by FDA approval of devices, Dr. Green explained that the comment about FDA approval has primarily to do with drugs, although cochlear implants are an example of a device. The comment was not meant to apply to medical foods. Dr. Howell proposed

changing the language to: There are defined treatment protocols for the condition, FDA approval/clearance (if applicable), and availability of treatment. There was no objection.

Action

Dr. Howell asked for a voice vote on the proposal presented by Dr. Green as amended, and the following motion was unanimously approved.

- ***MOTION**: The Advisory Committee approves the Nomination Review and Prioritization Workgroup's (NRPW) proposal with the following amendments:*
    1. *Prioritization: The chronological receipt of a nomination by HRSA will be the basis for a condition's placement in the queue for evidence-based reviews, unless there are data or circumstances that the Advisory Committee believes warrant moving a condition ahead in the queue.*
    2. *Modified criteria:*
        - *There is a screening test that is capable of identifying the condition.*
        - *There are defined treatment protocols for the condition, FDA approval/clearance (if applicable), and availability of treatment.*

III. FINAL PLANS FOR THE EXTERNAL EVIDENCE REVIEW WORKGROUP (ERW)

James Perrin, M.D., FAAP
Professor of Pediatrics, Harvard Medical School
Director, MassGeneral Hospital Center for Child and Adolescent Health Policy
Director, MCHB Evidence Review Group, Systems of Care for Children and Youth with Special Care Needs

Dr. James Perrin, the chair of the external Evidence Review Workgroup (ERW) that will review and report on the evidence relevant to the Advisory Committee in making recommendations about which conditions to add or remove from the uniform newborn screening panel, presented his final proposal for the ERW and its processes for the Advisory Committee's review and approval in the hopes that the ERW can start taking on assignments as soon as possible.

Composition of the ERW and its advisory group. Dr. Perrin proposed that the members of the ERW would be Marsha Browning M.D., M.P.H. (genetics); Anne Comeau, Ph.D. (State perspective); Nancy Green, M.D.; Alex Kemper, M.D., M.P.H., M.S. (who has done a pilot study of Pompe disease); Lisa Prosser, Ph.D. (cost-benefit analysis); Ellen Lipstein, M.D. (health services research fellow); Diane Romm, Ph.D., Project Director (epidemiology, methods); and Dr. Perrin (policy, chronic conditions). Dr. Marie Mann from HRSA would be an ex officio member of the ERW. Dr. Perrin indicated that the person they had hoped would represent the consumer perspective on the ERW had to withdraw, so they are trying to find another consumer representative for the ERW.

Dr. Perrin again proposed that an external advisory group be established to give the ERW broader national representation and advice on its processes. He proposed as members of the ERW's external advisory group the following individuals: Ned Calonge, M.D. (Colorado State Health

Department); Jannine Cody, Ph.D. (University of Texas); Harvey Cohen, M.D., Ph.D. (Stanford University); Robert L. Davis, M.D., M.P.H. (Kaiser Atlanta); and Celia Kay, M.D. (University of Colorado).

Questions & comments related to the composition of the ERW and its advisory group

Dr. Howell noted that Dr. Calonge is now a member of the full Advisory Committee, so he cannot be a formal member of the ERW. The Advisory Committee may ask that he participate but that would be a Committee decision. Dr. Perrin asked the Advisory Committee to help the ERW identify someone to take Dr. Calonge's place.

Dr. Howell asked whether the ERW would add people actively involved with patients once they received a nomination. Dr. Perrin said it would do this, although it needed advice from the Advisory Committee on how to handle conflicts of interest.

Issues in evidence review. Dr. Perrin observed that the evidence reviews performed by the ERW would involve some unique issues because of the rarity of many of the conditions detected via newborn screening (e.g., lack of randomized trials in many cases, limited information on costs and benefits across all potential outcomes, etc.) and because of challenges in obtaining access to evidence (e.g., unpublished investigator findings, proprietary data for new drugs that have gone to FDA). Many of these issues are identified in Dr. Alex Kemper's paper on pitfalls in developing evidence.

Identifying conditions for the ERW to review. HRSA's Maternal and Child Health Bureau will review the nomination forms to ensure that they are complete. If the nomination forms are complete, it will forward them to Dr. Howell to pass on to the Advisory Committee's Nomination Review and Prioritization Workgroup (NRPW) chaired by Dr. Green and the full Advisory Committee. The NRPW will review the nominations and report to the full Advisory Committee whether they are ready for an evidence-based review by the ERW. The full Advisory Committee will then vote on whether to send them to the ERW.

Dealing with conflicts of interests in the ERW. Dr. Perrin said he recognizes that the ERW must have a clear conflict-of-interest policy and transparency in its reviews. As the ERW develops evidence reviews for specific conditions, it may ask the Advisory Committee to assign one or two Advisory Committee members to work with the ERW on a specific review. In addition, the ERW will have to solicit input from experts with ad hoc expertise related to specific conditions. The ERW has developed a conflict-of-interest form based on the Institute of Medicine's committee form. All of the ERW's staff, its external advisory group and any ad hoc experts will be asked to fill out this form.

Dr. Perrin asked Advisory Committee members for guidance on how the ERW should address conflicts of interest in situations where the experts the ERW needs to need to talk to for disease X—both investigators and parents—are also going to be people who signed a nomination form for disease X.

Questions & comments related to conflicts of interest

In response to Dr. Perrin's question, Dr. Howell replied that many of the conditions under consideration are so rare that most of experts who will qualify have very clear conflicts of interest, because they will have been involved in the clinical studies, the development of drugs, etc. The key

thing for the ERW in dealing with such conflicts of interest will be to ensure transparency so that everyone knows exactly what the conflicts of interest are.

Dr. Calonge said he thought more than transparency might be required when votes were taken by the ERW. At the Agency for Healthcare Research and Quality, for example, they grade conflicts of interest. Dr. Perrin explained that the ERW is not going to make recommendations; it is just going to summarize the evidence of screening, treatment, harms, benefits, etc.

Dr. Fleishman suggested a distinction between an individual who comes to the ERW to give testimony and an individual who works with the ERW, adding that he believes that individuals who nominate a condition should only give testimony to the ERW, not work with the ERW. Dr. Rinaldo suggested that maybe nominators could be involved in the ERW but as nonvoting members.

Dr. Perrin asked Dr. Fleishman and Dr. Rinaldo how the ERW should handle Joan, a hypothetical investigator who has treatment data published and other unpublished data that the ERW wants to use in as unfettered a way as possible. Dr. Fleishman replied invite Joan to the ERW meeting and thoroughly question her, then thank her as she leaves and continue the discussion without her. Dr. Rinaldo suggested that the ERW might also give Joan a draft written summary of the evidence and let her review it and make comments before the ERW prepares its final report. Dr. Fleishman said this would be fine as long as the ERW independently prepared the final review document for submission to the Advisory Committee. He noted that even after the Advisory Committee votes a recommendation, 50 States are going to be making decisions with respect to adding conditions to their screening panels, so it is best to keep people with conflicts of interest as far removed from the recommendations and the ERW's final report as possible.

Definitions used by the ERW. Dr. Perrin noted that the ERW had received feedback from the Advisory Committee on various definitions on the nomination form at the Advisory Committee's September 2007 meeting. For that reason, he did not go over the definitions again.

Proposed structure of evidence reviews by the ERW. Dr. Perrin outlined the proposed structure and outline for the evidence reviews that the ERW would present to the Advisory Committee.

- *Evidence review rationale.* The evidence review will begin by presenting the rationale for the evidence review of the specific condition at this time. This section of the evidence review will reflect deliberations by the Advisory Committee on the nomination form and its recommendations. This section will also summarize recent changes in treatments and/or screening.
- *Objectives of review*. Next the evidence review will identify the generic objectives of the review (provide timely information to the Advisory Committee to guide recommendations for a specific screening protocol).
- *Questions for review.* The questions for the evidence review will be described. These will not vary a great deal but will be specific for the condition being considered:
    - What evidence is there about the natural history of the condition, including variations in phenotypes; the prevalence of the condition, including genotype, phenotype, and phenotypic variations; the impact and severity of the what is known about the impact and severity of the condition?
    - What evidence is there about the methods of screening and diagnosis (in screen-

positive individuals); screening test utilities such as specificity and sensitivity and predictive values based on prevalence; the feasibility and acceptability of screening; and the feasibility and acceptability of diagnostic testing after initial screening?

- What evidence is there of the benefits of treatment in screen-positive individuals and in individuals who were clinically or otherwise diagnosed?
- What evidence is there of the harms or risks of screening, diagnosis, treatment?
- What are the general costs of screening, diagnosis, treatment, late treatment, and failure to diagnose a condition in the newborn period (if there are data)?

- *Decision model and search methods.* The evidence review will describe the ERW's broad decision model and development of evidence questions and search methods:
  - Decision model and development of evidence questions—The review will specify the ERW's broad decision model about the decision points for a particular condition, and for each of those decision points, the ERW will develop questions that will frame what the evidence search. Many of the questions will be the same regardless of the condition, but a few will differ.
  - Search methods—The review will describe the methods the ERW uses to search for evidence, including the timeframe and search engines used. The timeframe will probably depend a bit on advice from consultants and perhaps the specific condition.
- *Systematic review and additional data collection & review.* The evidence review will describe the ERW's systematic review and provide additional details about data collection and review:
  - Study selection and data abstraction and review—The ERW plans to include in its systematic review of the evidence peer-reviewed, published literature. It will limit its review to publications in English. The ERW will try to access the grey literature, which is predominantly going to be limited to the pharmaceutical companies and the unpublished studies from key investigators. It will review consensus statements as guides to what current consensus is and for making sure that the ERW has not missed key articles. The ERW's evidence review will exclude case reports.
  - Data abstraction and quality assessment—The ERW will carry out standard data abstraction from the included studies. It will try to do some quality assessments, but given the paucity of studies, will likely include studies that are only of fair quality in the review. The ERW will also try to do some analyses of additional raw data from unpublished sources, again, pharmaceutical companies and investigators. The ERW is working on developing a data-sharing agreement with these sources.
  - Focus groups of experts (physicians and other clinicians, and families) regarding impact and severity—For certain questions, where the evidence from the literature is not sufficient, the ERW may need to pull together focus groups of experts to help it develop estimates to put into decision analytic frameworks for the Advisory Committee's consideration.

- Data synthesis around the basic questions raised re quality, harms, etc.

Questions & comments related to systematic reviews by the ERW

Dr. Perrin's comment that the ERW would exclude case reports from its systematic review generated quite a bit of discussion. Dr. Howell and Dr. Vockley noted that although one would not ordinarily include case reports in a systematic review, four or five case reports on an extremely rare condition might yield something valuable. Dr. Vockley suggested that the ERW include a reference list that identifies any published case reports. Dr. Perrin agreed that the ERW could include references and a very brief summary of case reports at the end of the review. He asked Advisory Committee members whether the ERW should try to extract the data even from a single case with respect to, for example, treatment if it is not going to help on the screening side.

Dr. Rinaldo observed that any public information about missed cases (false negatives) is very important to include. A single case report of a false-negative screening test would be relevant. Hypothetically, if there were single case report that when PKU screening began, several children were found to be normal, but one of those children was later found to have PKU, that one case would have been important. Dr. Rinaldo said he would delete the line that says the ERW would exclude case reports.

Dr. Perrin said he would like to take Dr. Rinaldo's recommendation under advisement, because the ERW is likely to find a moderate number of case reports; if it looks systematically at all of those, it will make the task of evidence review take longer, but without benefit. Dr. Vockley noted that the conditions that generate a lot of case reports will also have more compilations of evidence, so at that point the ERW could make a statement: "There are a large number of case reports, but there are sufficient numbers of compilations that we haven't included them." Dr. Howell agreed.

Another question that came up was whether parents would be involved in the deliberations of the ERW if no focus groups were needed. Dr. Perrin explained that the ERW would work with family groups to seek advice on conditions routinely, regardless of whether there was a focus group. He also stated that the ERW was trying to recruit a parent to become a member of the ERW to replace the person who had withdrawn.

Evidence review results & summary. Dr. Perrin noted that the final section of each evidence review will present the ERW's results and summary to the Advisory Committee:

- Results—The results will be presented in the order of the main questions.
- Summary—The key findings will be presented in summary and table form. The evidence review will indicate where evidence is absent and what information would be most critical.
- The ERW will not make recommendations to the Advisory Committee. The ERW will provide the Advisory Committee with as up-to-date and complete as possible evidence with which the Advisory Committee can make its decisions.

Next steps and timeline for the ERW. The next step is for the Advisory Committee to approve the ERW's proposal and give the ERW its initial assignments. Dr. Perrin said he anticipates that the process of evidence review will take roughly 6 months. This timeline assumes that the literature search review and abstracting will take about 3 months, depending on the condition; identification of key investigators (including families) will take a few weeks, with contact in the 2nd month; focus groups (if any) will be conducted in the 3rd or 4th month; and data synthesis and report

development will be done in about the 4th to 6th month.

Questions & comments related to the ERW's next steps and timeline

Dr. Perrin was asked whether the ERW would be working on several conditions simultaneously or doing evidence reviews for nominated conditions sequentially. Dr. Perrin replied that this would depend on what the Advisory Committee recommended to the ERW, but he thought that the ERW was likely to try to get started on one nominated condition, and then a few weeks later, get started on the second one condition, so that it would really be working on two or three or four (or maybe even more) conditions at any given time.

Dr. Vockley asked if the same process used by the Advisory Committee to recommend adding conditions to the uniform newborn screening panel would be used occasionally to decide whether a condition ought to be removed from the screening panel or make recommendations against continued screening. Dr. Howell said he thought that the process should probably be the same. Dr. Vockley, noting that no one is likely to nominate a condition for removal, however, recommended that the Advisory Committee institute some routine review of the recommended newborn screening panel as it stands to see if anything needs to be altered. Dr. Howell agreed and said this was a topic the Advisory Committee should take up at a later date.

Ms. Terry asked whether the timeline for evidence-based reviews by the ERW could be shortened if the Advisory Committee either required or strongly recommended that nominators of conditions submit a literature review with their nomination, with caveat that the ERW could do whatever additional work it thought necessary. Dr. Perrin had to leave the meeting after his presentation, but Dr. Howell said he would discuss the matter with him. Dr. Alan Hinman and Dr. Boyle said they thought that the ERW might find it helpful to have a bibliography submitted, but they emphasized that the ERW would probably want to do its own independent search of the literature.

Action

Dr. Howell asked for a voice vote on the proposal presented by Dr. Perrin, and the following motion was unanimously approved.

- ***MOTION**: The Advisory Committee approves Dr. Perrin's proposal for the Evidence Review Workgroup (ERW) and its processes.*

IV. ADVISORY COMMITTEE'S STATEMENT ON LONG-TERM FOLLOWUP AFTER NEWBORN SCREENING

Alex Kemper, M.D., M.P.H., M.S.
Associate Professor
Department of Pediatrics
Duke Children's Hospital and Health Center
Duke University

At its previous meeting, the Advisory Committee approved the paper "Long-Term Followup After Diagnosis Resulting from Newborn Screening: Statement of the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children" in concept, with the understanding that Dr. Kemper and his colleagues on the Long-Term Followup & Treatment Subcommittee would

revise the document to address specific concerns related to the wording pertaining to the "medical home" raised at that meeting. HRSA would then circulate the revised document by e-mail, and then schedule a conference call for the Advisory Committee prior to its meeting in January 2008 to review and vote on whether to approve the revised document.

Dr. Kemper explained that the primary changes to the revised long-term care document are on page 6 in the section entitled "Care Coordination Through a Medical Home." The language there reads as follows:

> "The ACHDGDNC supports the concept that all individuals diagnosed with a condition through newborn screening should have a medical home to integrate care and ensure quality and safety in care delivery. As the usual place for sick and well care, the medical home should be family-centered, culturally effective, accessible, actively engaged in the coordination and provision of primary and subspecialty health care services within the health care system and across other community-based agencies and services (e.g., other clinicians, educational programs, and community-based counseling and support services), and facilitate requisite referrals. Systems will need to be developed to assure that individuals transition to adult care services without losing a medical home; this is central to the receipt of long-term follow-up care."

Questions & Comments

Several Advisory Committee members, including Dr. Trotter and Dr. Geleske, said that the new language was acceptable. Dr. Rinaldo asked where the statement would be published. Dr. Kemper said *Genetics and Medicine.* Dr. Dougherty said *Pediatrics* might be a good place, too. Dr. Howell said the Advisory Committee was trying to have its material consistently go into one journal.

Action

Dr. Howell asked for a voice vote on the statement on long-term care presented by Dr. Kemper, and the following motion was unanimously approved.

- ***MOTION:*** *The Advisory Committee approves the new statement presented by Dr. Kemper entitled "Long-Term Followup After Diagnosis Resulting from Newborn Screening: Statement of the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children."*

V. PUBLIC COMMENTS

Two individuals made public statements to the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children on the afternoon of November 14, 2007.

1. Jeff Botkin, M.D., VP for Research, University of Utah

Dr. Botkin, noting that sometimes there are no data on the negative impacts of a false-positive test for a specific condition, asked whether the external Evidence Review Workgroup (ERW) would consider analogous circumstances to inform decisions related to false positives. Dr. Howell said he thought it made sense to do that.

Dr. Botkin also asked whether the ERW's evidence reviews would consider nontraditional benefits

of screening and diagnosis such as informing parents' reproductive choices and enabling families of affected children to avoid long diagnostic odysseys. Dr. Marie Mann explained that the ERW was planning to consider such benefits.

Finally, Dr. Botkin suggested that multiplex technologies might lower the threshold for including a test on a screening panel, adding that this consideration might come into the Advisory Committee's recommendations.

2. Lisa Feuchtbaum, Dr.P.H., M.P.H., Genetic Disease Branch, California Department of Public Health

Dr. Feuchtbaum asked whether Dr. Kemper and his colleagues were planning to develop a data collection tool for long-term followup after diagnosis following newborn screening. Dr. Boyle responded that the Subcommittee on Long-Term Followup & Treatment has not yet addressed tools for data collection. The subcommittee's next step will be to identify the roles of various participants in long-term followup. After that, it will take an illustrative condition to see what exists in terms of long-term followup and think about what the gaps are. Dr. Feuchtbaum said that a data collection tool would be very useful for states like California and she looked forward to the development of such a tool.

VI. ADJOURNMENT

Dr. Howell adjourned the meeting at 3:45 p.m.

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We certify that, to the best of our knowledge, the foregoing meeting minutes of the Secretary's Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children are accurate and correct.

/s/ _____

R. Rodney Howell, M.D.
ACHDGDNC, Chair

/s/_____

Michele A. Lloyd-Puryear, M.D., Ph.D.
ACHDGDNC, Executive Secretary

These minutes will be formally considered by the Committee at its next meeting, and any corrections or notations will be incorporated in the minutes of that meeting.