Transcript: Thursday – May 16

Please stand by for real time captions.

Welcome and thank you for standing by. During the conference all participants will be on a listen only mode. We will begin today's conference with a roll call. When your name is called you may respond. I will now introduce your host, Dr. Bocchini. You may begin.

Thank you, good afternoon. And or good morning to those of you on the West Coast, and thank you for joining this first meeting of the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children. As you all know, this committee was developed and now is chartered to serve with the same mission as the Secretary's committee and will do so for up to two years, depending upon decisions made by Congress to reauthorize the original Secretary's committee. So I'd like to welcome committee members, organizational representatives and attendees. As you know, this is a shorter meeting based on the fact that we had an earlier meeting of the Secretary's committee in April. So today, we are going to have a very short meeting and tomorrow we will address the issue of Pompe Disease, the nomination of that and review the data assembled by the evidence workgroup. I'd like to just mention that the Department of Defense has notified us that Mary Willis's term has ended as the representative of that organization, and so we will welcome Adam Landis, Lieutenant Colonel. So welcome Dr. Landis to the committee. A couple housekeeping notes, for committee members the sound will be coming to your phone line, so please make sure your computer speakers are turned off. We are going to hold questions or comments until the end of each of the three subcommittee presentations, and then when invited to speak, please state your name each time. Also speak clearly to ensure proper recording for the committee transcript and minutes. You can press star zero if you have any problems with your phone line. The members of the public, sound will be coming through your computer speakers, so please make sure your computer speakers are turned on. So now, I will conduct a roll call, first of the committee members, and then following that for the organizational representatives, to record the attendance. So we will start with alphabetical order, Don Bailey

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I'm here

Jeffrey Botkin is unable to attend -- Denise Dougherty

Alan Guttmacher

We can hear you

I'm glad to be heard.

Charlie Homer, Kellie Kelm, Fred Lorey,

I'm here

Michael Lu?

Here.

Stephen McDonough, Dietrich Matern.

Here.

Alexis Thompson is unable to attend. Catherine Wicklund?

Here.

Andrea Williams?

Here.

And then Debi

Here

Coleen Boyle and Denise Dougherty

Here.

Kellie Kelm?

Let's go to the organizational representatives American Academy of Family Physicians, Frederick Chen?

I'm here.

American Academy of Pediatrics, Beth Tarini?

Here.

American College of Medical Genetics, Michael Watson?

Here.

American College of Obstetricians & Gynecologists, Nancy Rose?

Association of Maternal and Child Health Programs, Lacy Fehrenbach?

Association of Public Health Laboratories, Susan Tanksley?

I'm here.

Association of State and Territorial Health Officials, Chris Kus

Here

Department of Defense Adam Kanis

Here.

Genetic Alliance, Natasha Bonhomme?

March of Dimes, Ed McCabe?

Here.

National Society of Genetic Counselors, Cate Walsh Vockley?

I'm here.

Society for Inherited Metabolic Disorders, Nicola Longo?

I'm here

Just to go back, to see any late arrivals. Coleen Boyle? Kellie Kelm?

I'm here can you hear me?

We can hear you, thanks

Nancy Rose? Lacey Fehrenbach and then Natasha Bonhomme

I'm here can you hear me?

We can hear you.

Thank you, we are going to start the session. Each of you who are on one of the subcommittees participated in this morning's subcommittee meetings, and we will now hear reports on current future priorities and projects for each of the subcommittees, the first on the agenda is the

Subcommittee on Laboratory Standards and Procedures, and Susan Tanksley is scheduled to give this report. Susan?

Hi, everyone, can you hear me?

Yes we can hear you.

Okay great. So we had a short meeting of the Lab Standards and Procedure Subcommittee this morning and I substituted for Dr. Fred Lorey, who was unable to be able to be there this morning. I'm going to briefly go through what we discussed, so the first thing we did was just review the priorities for the Lab Subcommittee and so those priorities are to review new and enabling and disruptive technologies, to provide guidance for state newborn screening programs and making decisions about lab implementation, integration, follow-up and quality assurance and to establish a process for a regular review and revision of the rest. And what we focused on this morning was the middle priority, with updates on the CLSI guidelines for SCID. So SCID -- in regards to guidance to state laboratories —this priority focuses on conditions that are newly added to the RUSP, and in regards for SCID, there's an NBSTRN monthly phone call and that is on the fourth Friday of each month, 1:00 Eastern Time and that call is open to anyone, and it's advertised on various newborn screening luster's and it provides guidance on lab implementation, follow-up and QA. So as I said that's an open call. Any Brower and Mei Baker and Jane Getchell comprise the workgroup on this effort. We then talked about the CLSI side guidelines, which have now been published, and that is NBS06 they entitled, Newborn Blood Spot Screening for Severe Combined Immunodeficiency by Measurement of T cell Receptor Incision Circles. So as you recall, SCID was added to the RUSP in May of 2010. The CLSI first convened a committee in August of 2011 in Atlanta and in June of 2012, that committee voted to drop the draft CLSI document. It was open for comments to approximately 1100 CLSI delegates in October–November of 2012, with final on — after the comment period — of March of 2013 and approved by the consensus committee, and then published in April. So that is available now to be purchased by CLSI. There was a large group of authors on this document. I want to note that Dr. Harry Hannon Dr. Rossini Abraham, Dr. Lisa Kobrynski and Dr. Bob Vogt were primarily responsible for assembly that document and incorporating comments from delegates and the editorial staff. Dr. Kobrynski, who was on the committee, called to give us an overview of that document so we will go over some of that now. On the next slide, please -- one more -- one more -- sorry about that – so, as I said Dr. Kobrynski provided an overview of the document for this meeting, this morning's subcommittee -- it follows the same kind of table of contents as other CLSI documents. But some of the unique sections in regards to SCID include the terminology, the biology of the condition itself, biological and clinical features of SCID, an overview of the real time PCR assays, implementation of the T-cell receptor excision assays and follow-up activities, communication and diagnostic testing. There are several appendices as well which can serve as an aid to newborn screening programs in implementation of the condition. There's also noted that at least two copies of the CLSI document will be sent to each state newborn screening program by the CDC. And so in addition to being available for purchase from CLSI directly, the states will receive copies of them. Okay. Next slide -- one thing that we discussed a little more deeper in detail was Appendix A, which is

entitled Immunodeficiency Disorders and T cell receptor excision circle (TREC) values in newborn screening. This is basically a list of conditions that may or may not be picked up by the TREC. And the Appendix is separated into four different categories based on whether not they will be picked up and the type of immune deficiency or -- other disorder that it is. And this Appendix is based on specimen collection at 24 to 72 hours. So for those states that do a later screen, they may see a little bit of difference in those where it's actually T cell deficiency that would be apparent later in life. So the four categories are primary immunodeficiency disorders typically associated at all receptor values below the expected range in the newborn screening period. So these are the things that you would expect to pick up in the TREC that have a low or no track value in the assay. And this includes the kind of typical SCID in, complete DiGeorge. The second category is primary immunodeficiency disorders variability associated with TREC values below the expected range in the newborns in the period. These are the things that we may or may not pick up, so we'll probably pick up some of these but we won't pick up all of these cases of SCID and other syndromes with T cell impairment. So that seems like a leaky SCID, variant skid, syndromes. The third category are primary combined immunodeficiency disorders that are not likely to be associated with TREC values below the expected range, so these are ones that would not -- we would not expect to pick up in the TREC because we are measuring T-cell deficiencies and not other types of deficiencies. So this is like partial ADA deficiency, and other defects like CDA deficiency, which don't cause severe T-cell lymphoma. And then finally are the secondary disorders that are variably associated with TREC values below the expected range in the newborn screening period. These are ones that may be picked up in the TREC assay as lower undetectable tracks but they are secondary to another condition. So these are not immune disorders that we are picking up, but one of the manifestations is low T cells. We did have a conversation around the T-cell deficiencies. It is the being investigated by some researchers in New York and there may be a couple of other states that are looking at this assay. It could eventually be added into the CLSI document in future revisions. If that's something that's determined to be needed. The one category that may -- that we may be able to pick up with those would be that second category -- the primary immunodeficiency disorders variability associated with TREC values. Dr. Francis Lee commented that we might be able pick up some of those, but not all of those if the correct TREC assay was added in addition to [inaudible]. Finally the last thing we discussed on the subcommittee call was tentative agenda items for future meetings. So, based upon the vote that occurs tomorrow, we would talk about implementation of Pompe screening, if it is recommended to add that to the RUSP. We also have a survey on Tyrosinemia Type I from the January meeting and so we hope to have a final report of that survey and Dr. [inaudible] in turn commented that we might also have a draft of the document -- there's also extensive interest in talking about various genomic sequencing initiatives and following was going on with regard to genomic sequencing initiatives in the newborn screening period. And we also discussed possibly presenting the Mayo study that's a comparison of technologies for LSDs and other conditions that's currently underway. That study is not anticipated to be completed until the fall, so this would be in future meetings, past September. So that's the gist of what we discussed this morning. And I'll be happy to answer any questions.

Susan, thank you very much. Let's open the phone lines for the committee members and the organizational representatives, and see if there's any questions for Susan and the information that she presented.

If you'd like to ask a question, on the phone dial star one.

Hearing no questions, let's move to the second presentation and obviously additional questions come forward, we can ask them at the end of the entire session. So the second subcommittee report is from Don Bailey from the Education and Training Subcommittee. Let's prepare for Don's presentation -- thank you.

Great and good afternoon everyone. I'm presenting on behalf of Beth Tarini, the co-Chair, as well as myself as Chair. Committee members, so just to remind you are subcommittee's charges—to review existing educational and training resources to identify gaps, and make recommendations regarding five groups: parent and the public health professionals that screening program staff and hospital births birthing facility staff. We had a great meeting today. Just about almost everyone in our committee subcommittee was on the call. And we discussed a variety of topics, so we had a very full very full agenda the whole time. I'm going to summarize a couple things that we discussed. First, before we really got into our agenda, we talked about the challenges inherent in the virtual meetings that we are having as opposed to in-person meetings. We feel that our subcommittee, particularly, because we interact with advocacy groups and different stakeholders -- we don't think we can as effectively accomplish our mission on the subcommittee through virtual calls. We know there's been discussion about this before, but we felt like it might be useful for a formal request from our subcommittee to go to the full committee so we would like to ask Joe, as the chair of the Secretary's discretionary committee, to use best political and kind approaches to talk to the secretary, but request in the strongest possible terms that we reinstate at least some regular in-person committee meetings. I guess we'll come back and maybe have a chance to chat about that at the end of my presentation, but we just feel that for the whole committee, especially for our subcommittee, this would be very useful. I'm going to go in backwards order, from Priority C to Priority A; because we start with the one with the we spent the least amount of time on, and then the greatest. So the first priority is to provide better guidance for advocacy groups and others regarding nomination and review process. So our ongoing project is collaborating with Alex in the Condition Review Group to develop public friendly summaries to previous data as well as evidence review nominations that have not gone forward. And the goal here is to increase public transparency for what we do, and primarily this is been aimed toward future nominators, not people who have submitted packages already and have not been approved for one reason or another. These are for people who are thinking about nominating something to the committee and we want them to see what's happened with conditions got stuck at one point or another in the process so they could be most effective in preparing their packages going forward. So the main goal and the main activity has been to create playful, probably friendly, summaries of the results of the nominations. We do have some draft documents prepared by Atlas research, which is a direct research organization. We are in the process, now engaged in discussions with the Condition Review Group and with HRSA. In our subcommittee, the

subcommittee chairs right now are discussing these documents further and talking about next steps, for how we can for further revise them then we will send them out to our subcommittee members for further discussion and feedback. We had hoped to have a draft document available to the full committee in September. I'm not sure whether that will happen or not, we will see what happens in the next month or two. The priority is promoting newborn screening awareness among the public and professionals. So we have two current activities -- the first one, which has been ongoing, which is to provide support and input on the 2013 Newborn Screening Awareness Campaign plans that activities that are being, primarily by APHL, and also from the Centers for Disease Control. This is what we spent a good bit of time talking about is, that is identifying what is awareness of the goal that we should be taking on, after 2013, because awareness doesn't end after the 50th anniversary celebration. So the campaign activity so far has been great. APHL developed a traveling exhibit, we have several members of our committee saying that the exhibit display had been used at some of their major professional meetings, also going around to selected state laboratories -- couple weeks ago there was a very successful combined meeting of the Newborn Screening and Genetic Testing Symposium, sponsored by APHL this year, being in conjunction with the international Society for Neonatal Screening, this was May 5th to the 10th in Atlanta, and there was a great turnout for the meeting and a number of our subcommittee members were there. And just a great celebration of 50 years of newborn screening. And there's been a number of public service announcements that have gone out, including a Times Square display in New York City. APHL distributed at the meeting a coffee table book that was very nicely done, and questions about how to access, will be able to provide information regarding that for the committee and for the public soon, but there's a nice product and I think everybody would be proud to have a copy of that. There's been some educational brochures developed. Our subcommittee members were involved in reviewing those posters and those brochures, both as representatives of the organization; those are now available for states Media coverage and DC reception and awards ceremony, in Washington DC, sometime in September. So the question we ask ourselves today is what you be the focus of our post-campaign awareness activities and so far our work has been, the overall goal has been promoting awareness among the general public in our professionals. What should be the biggest need what is the most pressing need in the next two years. Of course, promoting awareness among the public and professionals is a huge task. You're talking about very large audience -- awareness is useful to a certain extent but would do you want to accomplish with that awareness? So we talked about a possible theme of where it is being, harkening back to establishing a theme of state harmonization of screening targets and making suggestions regarding awareness that would be aligned with that thing. So what's the problem that needs to be solved here? The fundamental problem is that not all states have established the RUSP as their primary screening protocol. So everyone sure knows that state discrepancies in screening, the focus of several major campaigns and initiatives about 10 years ago, as well as a Government Accounting Office report, March of Dimes campaign and so forth -- American College of Medical Genetics reporting and ultimately the establishment of this committee, and so a national organization has been a goal of harmonization as a public awareness—how to go about achieving, for example, our target audience and state legislators would be one example, state advisory board to be another example, state public health departments might be another example. So we had a good and interesting discussion about, first of all, about kind of balancing

advocacy versus education, and really the goal out of the helping to make sure that these constituent groups are aware of this committee, aware of systematic and regular rigorous process we go through to make a recommendation. We do those processes and while all states should be screening for the same thing so that every child has access to the same screening in every state. We didn't come to a final formal acceptance of this, and some people talked about, well really the committee could be kind of the focal point for -- integrating efforts from different groups. The committee would really be the group to do this. I think we agree with that, that we don't really have the resources to launch a campaign, but we thought we had a really good discussion about this and we think that—my guess is in some version of this will become our ultimate theme subject, of course to approval by the full committee. So then priority A was to track and provide input on and facilitate integration of national education and training initiatives. Our project has been to identify one heritable condition, if not part of the RUSP, in which screening and treatment most likely would occur at a later point in child development. So then to work through some conditions, or a condition, and identify the major education and training needs for that condition. So in January, a week after pretty extensive review of conditions and nominations, with a three exemplar editions of fragile X syndrome, long QT syndrome and Wilson's disease, so the goal of our subcommittee meeting was to talk about what are the questions we are trying to address as we review these conditions, and then we took fragile X syndrome as the first example of one -- to walk through -- over the summer. We became aware, earlier this year; I think I mentioned this on the call. In April, the report that was commissioned by the Office of Public Health Genomics, to be done by Genetic Alliance, did review of population screening, questioned for three other conditions in that report. It has not been released yet, but subcommittee chairs have been able to see a copy of it and we are currently discussing with HRSA and CDC and Genetic Alliance about the possibility of a more broader distribution of that. But, regardless, we will be reviewing that draft report to see what implications it might have for our work. The plan right now is to do a review, continue to take the questions [inaudible] on data, show you in a minute. Talk along QT syndrome and in January, February, talk about Wilson's disease. We would then have a preliminary report to the Secretary's discretionary committee, in that meeting, where we are with things and probably then a more formal report in the meeting that's later that spring. So here are the six questions that we discussed here, that we would be trying to answer for each of these three conditions. So, first of all, was the typical pattern of identification of children with this condition? Then, fragile X, we showed the data, 36 months of age typical age of fragile X, even those concerns began to emerge about development and so forth around 9 to 10 months of age. And what problem exists with the current pattern of identification, one of the harms that occurred because of later identification, and could these problems be ameliorated to any extent through some form of earlier identification? So would population screening then, outside the newborn period, be at all feasible or desirable? What would be the benefits, of some kind of population screening, will be the harms, in the absence of population screening, what could be done for earlier identification that might be more symptom-based, on earlier presenting symptoms, we accelerate the diagnostic Odyssey or the discovery process and then to do what level of effort would be required, would it be pretty easy or would it be a difficult road? And then to get stakeholder groups to be engaged, certainly in fragile X nutrition, geneticists and counselors major advocacy organizations and so forth would need to be engaged. So, I think we've didn't

have enough time to discuss any of the things in our meeting as much as we would like—that I think we feel that these six questions provide a good and useful tool for thinking about the preconditions, and we hope that it's clear that childhood screening is going to be a much more complicated and diverse, even though we think newborn screening is complicated. This will be much more complicated because there isn't a single entity that is responsible for childhood screening, so could be professionals practice standards, it could be a number of different ways to do it. Ultimately get our goal is to identify the challenges and the opportunities and the considerations that would be required for childhood screening. So those are the things that we had a great commitment, discussed all those things and glad to open it up for discussion or questions.

Thank you, Don, for the report. Again, this is an opportunity for discussion questions or comments. Please open the mikes for the committee members and organizational representatives and if you have a question, I think some of the things are going to generate some discussion with this presentation. Please make sure that you identify yourself, so that proper recording of the your comments can be made and attributed to the person asking the question. And for the public, if you wish to ask a question, you can just go to the lower portion of the chat box, type in your question and click the send icon. We will see it and be able to answer the question, so let's open this up and I think that -- Don and the subcommittee raised a couple things, potentially generate feedback, so I guess the first one would be the subcommittee's request to reinstate some in-person meetings, so let's sort of look at that first if there's some -- is there support from committee members for that, or let's go ahead and asked that question first.

Can you back to that slide, Don?

Are the lines open for committee members and organizational representatives.

All lines open.

Thank you.

Committee members? Comments or questions or suggestions related to this proposed -- proposal to the chair?

Charlie Homer, committee member. I think in-person meetings are a good thing to bring an understanding and have the interaction with the advocacy groups and so on. I also appreciate the opportunity not to travel to all meetings, so I think a balance for having in-person and not in-person meetings would be good.

Thank you Charlie -- additional comments?

This is Susan Tanksley, representing APHL.

Yes Susan?

I would agree to some extent with Don's comment, in that I do feel that in-person meetings allow for greater interaction and peer relationship building, allowing for probably more open and honest discussion between members of the committee as well as with members of the public.

Thank you. Other questions? Comments? Anybody like to formally ask that I bring this to the secretary?

Andrea Williams

Thank you, and is there a second?

Second, Denise Dougherty

Thank you, Denise. So by voice, go ahead and say yes and then we'll take the vote. So those who agree, say yes,

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Yes?

No?

Sounds like we have a consensus, so we will bring this forward to the Secretary and then take the comment of about unnecessary travel into account. But we will go forward to see what will be the correct balance for the committee, to bring that forward to the Secretary. So then the second thing that I think warrants some discussion, that Don went for, was the issue of awareness and adding the target of harmonization across the states. Should this be an upcoming target of the committee? To kind of go back and revisit where the states are and then to see about focusing what needs to be done, to harmonize activities with the state newborn screening program. Let's see if there's any questions or comments related to that proposal by the subcommittee.

Again, not necessarily a fall proposal. We were just reporting our discussion on that. If anyone has any input on that discussion or thoughts, we greatly appreciate.

Thank you, Don, for clarifying.

This is Dieter -- I just wondered if this is about the states that may not have completely implemented. Is it all of those conditions at this point or is it also about states that go beyond the RUSP, and introduce conditions that are the committee has found not to be ready for prime time yet?

This is Don, I thank you for raising that Dieter and clearly those are two fundamentally different questions. And the first one is that we would be focusing on is making sure that every child has access to the recommended conditions on the RUSP. I think it's a more complicated question about what states start screening for things that we're not recommending. I think my feeling, at the moment anyway, is our primary focus on explaining the RUSP. Why we are making the decisions that we are and why we think it's important to screen for those conditions. That's my initial response anyway, but I'm sure that will come up in our discussions.

This is Fred Lorey. I have a third focus. I have an echo, not sure if you're hearing it -- third focus would be when a new disorder is added. We don't have the legislation to add [inaudible] that one set. RUSP recommends them; we had ability to out [inaudible] for a couple years ago to that effect that died. I don't know if there's anything that the committee can do to encourage more states to adopt the RUSP as their method of adding new disorders. It would help those of us in the programs because then the committee can do --

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I think I surely think that's in line with our hope, or whether that's a specific strategy of asking states to adopt that formally, or as them, even if they don't certainly pay attention to our recommendations and use that as a primary focus for decision-making. I don't think what you're suggesting is inconsistent with the intent of what we are discussing anyway.

This is Susan Tanksley, representing APHL.

Yes.

I just have a comment, in regard to identifying some of the needs. NewSTEPS is starting a new assessment to identify the education needs that state administrators and policymakers and legal departments have, and they will be doing key informant and interviews of individuals in leadership positions in those various positions, to see where NewSTEPS could help with educational activities for newborn screening. So once they have that information, they would be reaching out to other groups, including the Education and Training Subcommittee, to develop a plan to develop more information and educational tools for those particular groups. So that could assist with those efforts.

Yeah, that would be graded to keep our subcommittee informed of that. We're certainly obviously very interested in those activities, and ultimately it's better for other groups to be doing these things and we can kind of be the stimulus and the sounding board in the feedback group.

Great, and one more comment in regards to the response about Fred's comment about maybe a legislative toolkit kind of thing -- the newborn screening and genetics and Public Health Committee of APHL discussed something similar to this at it our recent face-to-face meeting prior to the Newborn Screening Symposium, and it identified a need for the newborn screening program and so that's something that we in the future will plan to work on.

That's great. I think this is clearly going to require involvement of a lot of groups and individuals. We will continue to discuss this at the next meeting and it would be great to have, if there are other like we weren't aware of, APHL activity so if we can figure out ways to make sure we are up to speed on those things that would help us with our discussion, and perhaps will have a full more formal recommendation, a request of the full committee at our next meeting in June.

Thank you Don and were there any comments related, or questions to the presentation that Don made, that the subcommittee was with the exemplar conditions in the questions that were associated that needed to be solved?

This is Chris. Because I think one question I have is—in your discussion you talk about population-based screening. Have you talk about what that means in terms of childhood and newborn screening, because we are talking about hospitals [inaudible] and most of the time and has been born in hospitals although you have a smaller group -- how does that relate to childhood when you're talking about children on health insurance plans or that might be uninsured, what would population-based the for that?

Exactly why we are doing this work—to figure out what would that mean, what could it mean, when could you do it, would do it, how would we give it for text. So, we only scratched the surface on some of that so with fragile X, and I think we'll probably start with a very high level view of somebody with pressing issues. But that's exactly right, but that's why childhood screenings going to be very hard.

We do have one public question, Don, from Janet Monaco

So Janet is asking about what efforts can be made to assist and guide those already nominated conditions that have not been approved, and so we did have some discussion about that in a committee meeting today. Our original work has been primarily geared toward helping people who have not nominated positions, so they could learn at the entry stage what some of the major challenges to them. We do not have time to fully discuss us in the subcommittee. We realize that this is an issue, we realize that people who nominate conditions that haven't been successful would love to have technical assistance that would guide them specifically into designing whatever study that would be needed to bring their condition forward to figure out how to fund it. I think an aspirational goal for the nation, whether it's something that our subcommittee can take on, I think would be a challenge in terms of resources and mission. Doesn't mean we all don't agree with that. I think we ended up concluding that this would be worth some future -- further discussion by the whole committee, not just by the educational and training -- postpone everything until a future meeting where we can think about other strategies for systematically helping failed nominations, beyond just saying “here are the reasons why, now you go figure it out and come back to us when you're ready.” I think that probably summarizes where we are right now.

Thank you, Don, very much. All right, that will conclude the report in the discussion on Education and Training Subcommittee. Let's move now to the Subcommittee on Follow-up and Treatment. Chris Kus will provide this report.

Sure, I think what I'm going to go through is the agenda that we had. We had good attendance from our members. Carol Greene, who is the chairperson, was not able to participate but we had input before the meeting to talk about what we are some of the decisions or things we were going to talk about. To go through one of the things we did, is we recapped at the last webinar our initial work on lessons learned from early hearing detection and intervention that may be applicable to Critical Congenital Heart Disease screening, and a we took some of the comments that came in. We framed some ideas, about reframing the lessons learned and just to give you a flavor, one of the things that we highlighted was the idea of really trying to save -- say that the importance of moving to electronic reporting between state health departments, hospitals, and providers with regard to screening. We also talked about the idea of a particular lesson, being that the importance of state-level data in order to help develop the system, and our overall plan is to have a draft paper which would include some paragraphs which would explain some of the major points to our group. We are having a call in about two weeks for that group to look at -- with the end result being presentation of a draft paper to the committee, hopefully at the September meeting. The second part we talked about was a follow-up to the presentation on the Affordable Care Act that happened at the last webinar. We had a lot of good discussion about this. Specifically, the thought has been that we need a follow-up kind of a panel that specifically deals with ACA and its possible effects on children in the newborn screening program. That was a good global presentation, but now are there some specific questions that we want to have clarified, that we thought would be helpful to have a panel discussion on. To give you some flavor about that, there is mention in the ACA about reimbursement for screening tests that are approved -- that are on our RUSP, and the question is that—the screening test—how does it relate to treatment. The other discussion has been, since the ACA has really moved to states defining benchmark plans, the importance that states are going to have to work with regard to the benchmark plans and their coverage relative to this population. When we talked about this, there was also some comment about affordable care oh -- Accountable Care Organizations and how that affected what outcomes they look for this population, that there is a performance that measures newborns relative to hearing screening that may be a way to improve that screening or at least look at how that works. In summary, we thought that a panel, and we had some suggestions, and we be interested in any other people's comments about specific questions that the panel could address. But we thought it would be helpful to have somebody from CMS, probably somebody from MCHB, public health representatives, somebody from specifically the newborn screening program and a parent representative with certain questions that we would highlight for that panel discussion. An example is in my world, in terms of maternal and child health programs—the discussion about using MCH funds to fill gaps for populations, like children identified through newborn screening where they may be underinsured, given the concern that the inch mark plan may not have and edge part plan may not have comprehensive coverage for these children. There was also one recommendation, I think Charlie Homer made this, is that there is some work about how ACA affects children with special health care needs in general -- the Neil Health

Fund and others are working on and we thought putting -- looking at that work in maybe having that kind of set a context for what we are talking about a specific population of children as with special health care needs would also be useful. So, I think in this area we are talking about, can we plan for a panel discussion on these issues to follow-up on the ACA presentation and how does that look for September or when we would plan for that. The other thing that came up, and be classified as new business, will be to think about how we as a committee will follow the implementation of ACA and how it affects this population. What kind of outcomes, what kind of measures can you use to see how well children are being covered, particularly since this is going to have specific differences from state to state, and is that something that one of our committees—our committee or some other committee—should pay attention to, and what are our committee members' thoughts about that. The last thing is we have a clarification. It was at the last webinar where we talked about a framework for assessing outcomes from newborn screening, using sickle-cell disease as an example, and it has been clarified for us that the group has been working in the right direction and that we are really working on a framework using sickle-cell. It's not a specific data-driven activity but it's more of is this framework of looking at what are the expectations for newborn screening, using sickle-cell, something that could be developed and then would be applicable to other conditions. I will stop here and see if there's any questions or any additions.

Thank you Chris. Let's again open the lines for committee members and organizational representatives, and again for the public. A reminder that you could type in a question in the chat box for us to see. So, any questions or comments?

This is at Ed McCabe from the March of Dimes. I would just like to say that our office at governmental affairs is spending a lot of time on the ACA this week. If we can be of any help to you with your panel, the March of Dimes would be very happy to be involved.

It's great to hear. We will probably be in contact with you.

Okay and it may be through Cindy Pellegrini's group at OCA in DC.

Thank you.

All right additional questions comments?

This is Mike Watson. I wanted to say only that HSRA has added a serious focus on ACA and essential health benefits to the National Coordinating Center for the Regional Collaboratives, as well as for the all the Regional Collaboratives. So we are well into developing sort of assessments of what's representative and what's not, and it's mostly what's not.

Thanks. Additional questions or comments? If there are no more questions or comments, then we basically are going on schedule to adjourn this portion of the Discretionary Advisory Committee meeting, so one last chance any questions or comments? All right. Hearing none, then, thank you all for your participation. Thanks to the subcommittees for the work that

they're doing and the chairs who have informed the entire committee. And thank you. We will meet again at 10 AM Eastern Time tomorrow morning. So again, thank you all for your participation. We will adjourn now and reconvene tomorrow morning, thank you.

This concludes today's conference. Thanks for participating. You may disconnect at this time.

[Event Concluded]

Addendum to Transcript

Chatbox discussion during Thursday, May 16 Webinar

Charlie Homer:
also using video conferencing technology could help for the distance work.

Robert Ostrander:
a lot of networking occurs at breaks and in the evening; things go forward in little groups around the table at lunch and dinner--moving things forward in jumps that don't happen in formal sessions.

Jana Monaco:
With efforts to help those wishing to nominate conditions, what efforts are going to be made to assist and guide those who have already nominated conditions, but have not been approved? These organizations or individuals will need further guidance to fulfill the voids in their nominations. Thank you, Jana Monaco