SECRETARY'S ADVISORY COMMITTEE ON

HERITABLE DISORDERS IN NEWBORNS AND CHILDREN

Thursday, January 26, 2012

Morning Session–Part 2

10:45 a.m.–noon

Park Hyatt Hotel

Washington, D.C.

CHAIRMAN BOCCHINI: All right, let's go ahead then with the meeting. At the close of the initial session, I'm not sure this was mentioned for the record, but there was no committee correspondence, so there was no need for discussion.

One thing that was brought to my attention, later we will be discussing, after lunch, the nominated condition of 22q11 Deletion Syndrome. A summary statement of the workgroup report is in the agenda book, but the nomination packet was not included. Therefore, it is being e-mailed to members of the committee now, so that it will be available to you, so that you will have that.

We're going to now moved to Sara. Sara is going to talk about orientation to the current charter processes and procedures. There will be a requirement for a vote at the end of the presentation. But as she goes through the different issues, our approach is that, as the issues come up, we would like some discussion about each of them as they are brought forward.

So, Sara?

DR. COPELAND: Thanks. I had a joke, just to preface this so you know to laugh.

[Laughter.]

DR. COPELAND: I've already tried it out on Chris, and he said it was okay.

So have you heard of OCD? I have CDO, it's like OCD, but the letters are in the right order. And then just kind of sets up my whole talk, because that is just really where I'm at. So I'm all about structure and function.

So I wanted to talk to you guys about this. We're working on some changes, and it is to provide some structure to the current processes and procedures. We learn as we go along. And I am OCD enough that I really do need to have some structure for us to work on. Hopefully, it will make things smoother, especially as we go forward in 2013, we want to make sure that we have dotted all our i's and cross all our t's, and especially that we are meeting our legislative requirements.

I don't know if you noticed, but with RUSP and other things, the stature and visibility of this

advisory committee are growing. And we want to make sure that our processes and our recommendations are very well thought out and put forward in an organized manner.

And it's kind of time to look back. There are 26 meetings now. Let's look back and review the current legislation, and make sure that we are meeting all of our requirements.

So these are the four or five different topics I'm good to go over. There should be an order to it. You will see my OCD coming out. And I would like to discuss it after each topic, so we don't get to the very end you have to remember what I spoke about five slides ago.

So the first topic is going to be the condition review. The current process, which I'm sure all of you are very aware of or most of you, Dr. Lu -- it's okay.

So the current process has six real core questions that they are moving through. Is this screening for outcomes? Is there a case definition and what is known about it? The prevalence,

spectrum of disease, natural history. Is there a test for the disorder? Been validated? What is the clinical utility of the test? How cost-effective is screening, diagnosis and treatment for this disorder compared to usual care?

And this is the algorithm the Evidence Review Group worked very hard to come up with, and I think it's a very good algorithm. The proposed revision takes that algorithm and just adds one more step, and it's number seven. It's what is the impact on public health for screening this disorder? The impact on the health of the public and the impact on the public health system.

And that is the proposed revision, that we add another step in the conditional review. So it's not just going to be an evidence review. It's going to be a conditional review.

And the rationale for this is that it's in our legislation. We have to be able to evaluate the potential public health impact of any of the disorders that we had to the RUSP.

So we are trying to harmonize or

collaborate with other events review processes across the Federal Government, one of which is the U.S. Preventive Services Task Force and the other is the Community Guide for the Public Health Evidence, and try and come up with a process that is a crosswalk between the two. Currently, we have a steering workgroup for this, and we will review and present it to the advisory committee. The plan is -- I'm going to hold Alex's feet to the fire for this, is to have of model for you guys to review in May.

So one thing to keep in mind for this is the public health impact has not been assessed and it does not fulfill the model matrix provisions for newborn screening expansion and updates to the RUSP.

So these are things you consider. Questions or comments?

DR. BOTKIN: I just think this is a terrific development, and I'm strongly supportive of the inclusion of this element. I think it's going to improve the overall process and really help states incorporate the recommendations into their

state policies and procedures.

DR. COPELAND: Good.

Fred Lorey and Charlie Homer are on the phone.

DR. LOREY: Yes, I had a little trouble hearing the last person, but I guess I'll discuss the question. Is this in any way going to be tracked from what the committee standards are in evidence-based review, because when you talk about public health effects --

DR. COPELAND: So, I'm sorry, go ahead.

DR. LOREY: I'm just concerned about organizations that will not pay attention to the Secretary's recommendations. Is this in any way going to dilute that by adding this component?

DR. COPELAND: From my standpoint, and of course you're going to get kind of a biased answer which will be no, but I think -- I mean, I'll just be honest. But I also think that, in collaboration with the USPSTF and the task force, I think actually it will make the recommendation stronger.

But we're not really changing the review.

We're just taking a condition review one step further to include the public health impact, which we have to have according to the legislation. So unless the legislation gets changed, we really have to have it.

DR. LOREY: Okay, that's fine. Then in the opposite direction, you don't think it will affect when the committee makes a recommendation not to screen.

DR. COPELAND: No, in fact, we're not changing the choosing matrix at all.

DR. LOREY: Okay, thank you.

DR. COPELAND: Denise had a question first.

DR. DOUGHERTY: Yes, I agree with both of these, but I guess I don't see the connection between revamping the review process and aligning more closely with the USPSTF and Community Guide. That seems to be a separate issue to me.

DR. COPELAND: So the USPSTF and Community Guide, we're trying to work with them because that way we can actually make these streamlined efforts.

So if we have an evidence review process that is amenable to the task force, then the conditions that they might be asked to review like PKU or whatever, they would accept our evidence review in place of doing their own. And if we get something that is more appropriate for the task force, the Community Guide, in terms of screening then we would in turn develop a mechanism for possibly referring it to them.

DR. DOUGHERTY: What the connection to the public health impact? So when we take a U.S. Preventative Services Task Force or the Community Guide recommendation, then take the step of having the public health impact of that done, because the Preventive Services Task Force doesn't do a public health impact?

DR. COPELAND: Whatever we look at will have to have a public health impact in the Community Guide. We're collaborating with them in order to use their experience, as opposed to the whole process, because they have the public health impact built into their system.

Coleen was first.

DR. BOYLE: So first of all, this was an excellent recommendation. I think it does track back to the actual law and the recommendations. I thought the committee had tried to do that. So I'm going to ask a little bit of the process question, so I better understand it, and maybe those around the table, too.

So we're sitting here not talking about the evidence review itself that will continue to go as outlined, but that after the evidence review is done is what is sort of the next follow-up to that process?

DR. COPELAND: More or less, yes, but instead of having two separate groups to it. At one point in time, we considered having two separate groups do it. We're going to have it all be done as a condition review.

And I don't know if Alex wants to comment, but I think my impression has been that the evidence review hasn't really had a public health impact but this really needs to be clearly delineated.

Don?

DR. BAILEY: I was going to ask the same question that Coleen asked. I do support this direction. I think it's very important for us to think about the public health ramifications of our recommendations. I was curious about who would be responsible for gathering the data on this, but related to that, would this be -- what kind of data will we be asking for in terms of making this decision? If we're asking for the same kinds of data were asking for in terms of the Evidence Review Group, that is going to be very challenging because it really will require implementation studies, cost-effectiveness studies, process studies.

And so I don't know if the decision-making -- the decision matrix will address that. I don't know, but I think it's going to be an important question, because if we're asking for hard-nosed data on it, that is really going to delay a lot of our decisions.

DR. COPELAND: That's exactly it. I can't tell you what it's going to ask for. I think we're

going to take the models from the Community Guide and other models for health impact analysis and try and incorporate into a model.

But what we're asking for is not really determined yet. That is the purpose of the workgroup.

Yes, Chris?

DR. KUS: One of the comments is I think we've had discussions about public health impact in the group, and I think the idea of saying this means to be part of it is a good idea, so it means that that evidence group will comment on report.

DR. COPELAND: They're going to be the condition review group. Not changing the name.

DR. KUS: Okay, but it's the same?

DR. COPELAND: So the process, yes, the process is going to be expanded to include the public health.

Any other questions or comments?

Yes, Coleen?

DR. BOYLE: Just one more clarification. So we will hear this next time, sort of a draft

consideration?

DR. COPELAND: I'm happy to include this in our process, but what -- yes.

Okay, so that's the first proposal. The second one is voting on the conditions.

The current process right now is the Evidence Review Group presents their data to the advisory committee, and at several different points in time, the advisory committee discusses and votes. And it could include recommendations of adding a condition to the RUSP. The proposed revision is not great, but it includes the advisory committee or two specific members of the advisory committee from the very start in listening to the evidence review and hearing what the evidence is from the very beginning. And they in turn frame a perspective of recommendations, so that when the Evidence Review Group presents, much like with the hyperbilirubinemia condition today, the AC members also present what they would recommend the advisory committee do. However, this is not binding; it is up for discussion.

But again, my OCD and my framework comes into play. It provides a framework for the discussion as opposed to having all of the evidence given to you and say "vote."

And the reason for this is really there's not enough time for the full discussion of pros and cons. I don't care how long we have; there's just a lot of thinking that needs to be done. And it's very similar to that have process we currently have for the Prioritization and Nomination Workgroup, regarding evidence review. And as I said, it allows for a framework and reference point, and more participation by the advisory committee from the very start.

So questions or comments on this?

Yes, Denise?

DR. DOUGHERTY: As you know, I've had concerns about having two AC members kind of take the lead on this, because of past experience. And I'm not sure what it means to have the AC members present perspective vs. present their recommendation to the committee, and whether they will be

presenting, in effect, their recommendation to the committee.

DR. COPELAND: I'm going to have Joe answer that, because this is based on his previous experience.

CHAIRMAN BOCCHINI: I think the term is probably better "recommendations to the committee," because I think the two advisory committee members will really serve as the voice of the committee. They will be involved in the sense that they will, based on what they understand the decision is that has to be made, provide back to the Evidence Review Group what the committee may need to make a decision. And then they will be up to date on the data as it becomes available, so that they can then make preliminary recommendations to the committee for the committee to vote on.

When those recommendations come to the committee, they will come from the two members. Those two members will then have the rationale, background and reasons for those decisions. The committee will then look at those recommendations,

and based on their understanding of the data, potentially revise, accept or recommend changing those recommendations.

But I think what this does is it provides two members with a really good look at the data as it is being developed, an opportunity to develop formal recommendation, so they can focus the discussion of the working group of the entire committee. And I think what this does is it enables the committee to then make a better decision.

ACIP has been doing this for years. In fact, the working group that is set up for individual vaccines or recommendations always consists of members of the ACIP. And they are responsible for, along with the CDC, updating the entire committee as necessary on background information, also that they inform the committee along the route, so that when a vote is ready the committee is up to date. But then the recommendations come specifically from that committee and they are modified by the entire committee from the working group.

DR. DOUGHERTY: So can you say a little bit more about how these two members get selected and who they are?

I think we've had some issues before with the committee members being selected who are advocates for certain conditions, and that has been very difficult for the committee as a whole to go against that recommendation.

CHAIRMAN BOCCHINI: I think that is a very important point, and it's something that we need to be very careful as we select committee members to serve. Obviously, the committee members need to be chosen because they have some expertise, but that they are going to be able to provide the information, that they are going to be able to in an objective way look at the data and make recommendations to the committee based on the committee's responsibility for making a final formal recommendation for whether to include the condition and the recommended uniform screening panel.

So I think it's going to be very important for the committee to choose effectively those

individuals who will serve. And I'm sure the responsibility will primarily be mine and Sara's for that to happen in an effective way.

DR. DOUGHERTY: So one suggestion is that, given our past experience, we kind of have a trial period on doing this, rather than have it implemented as a final recommendation, but it is up to the committee to vote.

CHAIRMAN BOCCHINI: Okay. Well, in a sense, we're going to try that with the hyperbilirubinemia presentation tomorrow. We have selected two committee members to work with the group, and they are going to come forward with some recommendations to the committee. The committee has all the data. The final report from the Evidence Review Group will be made tomorrow, and then two committee members will come forward with some recommendations to help frame the discussion. Let's see how that goes.

DR. COPELAND: Yes, Don?

DR. BAILEY: So I'm very much in favor of setting up some processes of things that happen in

between the meetings, so that we can have more efficient -- make our meetings more efficient. I do think we have to think very carefully about who these people are, and also about what are the multiple perspectives represented with respect to the criteria that will we will ultimately be using, especially when you're adding the public health impact question.

So I wonder if we might want to think about a strategy where it may be the chairs of the three or however many subcommittees that we have constitute this group, because the education committee, for example, the follow-up treatment group will certainly have some advice, or maybe they will take the perspective not just of the condition but also the public health impact of it, impact of it on providers and so forth.

And if we set it up, I don't know if we can or should have a formal executive committee of the chair and the subcommittee chairs, but that might be a more formal mechanism for something like this, if the subcommittees represent the broad range

of themes that this committee is supposed to address, and each committee is a member of subcommittees, it might help.

I don't know, Denise, if that would help address some of your concerns or not.

DR. COPELAND: I think that is a burden to put on the subcommittee chairs, who already have quite a burden.

But the selection process needs to be really thought through.

Coleen?

DR. BOYLE: Just one idea, just be very explicit about conflicts of interest. You know, subject matter expert maybe don't -- people who have vested interests, who have worked for years and really want to push the issue, they shouldn't be those representatives. We want a fair, objective voice there. They can serve as SMEs, but not necessarily on these committees. So somehow just being explicit about that.

DR. COPELAND: The nice thing about this is the determination of who works with evidence

review is in the policies and procedures, and so it doesn't have to be decided today. But this is something to consider as we go forward with policies and procedures.

Yes?

DR. BOTKIN: A process question: Do you anticipate that the two members will prepare separate recommendations, or do you expect they will get together and present one? And will those recommendations be available to the committee prior to the meeting, or would that be presented at the time of the meeting for the other committee members?

DR. COPELAND: I will let Joe answer that.

CHAIRMAN BOCCHINI: Yes, I think, ultimately, as we go through this, I think the best thing to do would be to have that presented prior to the meeting with the agenda book, so that it is available to everybody to review and consider. And I think that would then help frame things even better.

I think also, as a part of this, this is a process and so that really is the end of the

process. By having the Evidence Review Group and the two committee members working in concert, they as a working group can come forward to the committee at various time frames with data on the background, other aspects of whatever the condition is, so that the committee is informed along the way, and so that the committee is then ready to look at recommendations when they come forward, when the evidence review is completed.

But the goal would be that the evidence review at a time of its completion and when it is coming up for a vote, the recommendations would be available to committee members before the meeting.

DR. COPELAND: Are there any comments on the phone from Fred or Charlie?

DR. HOMER: No, thank you. This is Charlie.

DR. COPELAND: Okay, next topic.

Here is where my OCD comes in again, because I like processes, and I would like to have a formal process for reports and products. Currently, reports are presented to the advisory committee.

The recommendations are decided on, and they are sent to the secretary. What I would like to have is each report or product reviewed by the appropriate subcommittee, which most of them are right now, but this allows for potential outside products that groups want to have considered could be referred to the subcommittee and then, if deemed appropriate for further processing, will be presented to the advisory committee for official support.

The support would have four different levels. The first would be official advisory committee support, which is high. This is when it is important to the field of newborn screening. It is in the purview of the advisory committee. It is under the authority of the Secretary and there are actions to be taken.

The second would be an affirmation of value to the newborn screening community. It is important, but it is maybe not in the purview of the advisory committee or maybe not in the authority of the Secretary to make these recommendations. However, it does go to forward to the Secretary for

information only, and it is also noted that it was voted by the advisory committee itself.

The third would be an acknowledgment that this report was presented. It is important but not actionable. It's not in the purview of maybe the Secretary or the advisory committee. It is not sent forward to the Secretary for even informational purposes, but it is acknowledged as being a topic of discussion.

The rationale for this is our value is built on our reputation, and it gained through expertise achievements and objectivity. And we want to make sure that we appropriately support at different levels materials that benefit the community. Not all requests require secretarial action or review, and this allows for support by the advisory committee, but doesn't require the Secretary to maybe step out on a limb and say yes or no to it.

Yes, Don?

DR. BAILEY: Are you intentionally limiting this to newborn screening, or is it more to

the broader --

DR. COPELAND: If it is in the scope of the legislation, which includes heritable disorders.

DR. BAILEY: Right, so maybe change some of the language.

DR. COPELAND: Definitely.

Yes, Denise?

DR. DOUGHERTY: Could you go back to the slide before this one? Number three says important, but not actionable, but number two does not address actionability.

DR. COPELAND: Number one is the one that has the action, so number three doesn't go forward to the Secretary. Number one is for those that have actions, and number two is that for those that are for informational purposes only, but go forward to the Secretary. The third would be those that the advisory committee acknowledges as being important, but there are no actions, and we're not going to send it forward.

DR. DOUGHERTY: Okay, this might be important in number two to say important but not

actionable, because it is not under the purview or something like that.

DR. COPELAND: Okay.

Yes, John?

DR. BOTKIN: Yes, just a language issue, I wonder if we might change that left column to nature of support, because I think they're going to be things in two or three that the committee thinks are high level of support, but they are just not, as described in the second column, fitting within certain -- so I don't think we want to dilute the level of support with moderate language here.

So maybe say support given the nature of the document.

Dr. Copeland: Okay.

Coleen?

DR. DOUGHERTY: I'll let Coleen go first.

DR. COPELAND: It's the committee first.

DR. BOYLE: So I guess I'm thinking about -- I'm trying to put this into practice. It's always helpful for me, translation, and the products from our subcommittee that will be presented

tomorrow, I feel like there's a gray area between one and two for me, because I get stuck on the action part of it for two, because I do feel like some of our products from our subcommittee, we want affirmation of value by the committee, so we want them to be products of the committee. I don't think there's necessarily an action for the Secretary, other than FYI. I also don't think I agree with important but neither in the purview of the advisory committee under the authority of -- I'm getting confused on it.

DR. COPELAND: The language needs to be revised, obviously, with non-policy experts, but the idea of being it's the concept at this point of time.

But the bottom line is, number one goes to the Secretary and has action. Number two goes to the Secretary for FYI. Number three is voted on by and approved by the advisory committee but doesn't go anywhere else. And number four is we don't think that this is something we want to have an association with.

Yes, Denise?

DR. DOUGHERTY: I think we need another process underneath this one for what it means to have support. Does it mean that the entire committee has to agree? Does it have to be a majority? Can it be the chair?

DR. COPELAND: it has to be voted upon. This is like voting upon a condition, so it would be majority.

Okay, now to Dr. Simpson. I'm sorry.

DR. SIMPSON: This is a nonvoting comment. Since the charge of the committee is beyond newborn screening but heritable disorders in general, I wonder as long as you have the levels of support codified on the slide for us, whether you would comment what would happen if we extended it beyond newborn screening.

For example, home genome sequencing is around the corner. We're going to find out lots of information, and I know as a fact you've had that discussion. But where would that fit into this plan, the unintended and serendipitous findings that

you have? Is it a three, is it a four? Does it come back to it to a two, or where?

DR. COPELAND: So it introduces a different level of conversation. It needs to be within the scope of the advisory committee, but that does include heritable disorders. So if it wasn't clear-cut, newborn screening-related product, we would make sure that we have our legal counsel just make sure that they agreed with us that it was within the scope of the committee and the legislation. And it gets back to legislation and what the scope is there, which is quite broad, very broad.

Yes, Don?

DR. BAILEY: I'm trying to understand, the committees usually have priorities of what they're working on, including some products. Does this create added work that somebody would submit a report to the committee and say I want some action to it? Is it as they nominate conditions?

DR. COPELAND: It is a similar process. One of the areas that I can think of that this might

be of use in would be, in our legislation, the advisory committee is to report on and develop issues related to quality indicators in newborn screening. At this point time, no one is working on in the committee, but there are groups outside the group working on it. And instead of trying to re-create that work, once those indicators are validated and approved, to have that work presented first to the lab standards and procedures subcommittee, and if it is seen as being valuable and something the advisory committee should vote on, then brought forward to the advisory committee, but realizing there are other people that are working on heritable disorders outside of this committee that I think would be valuable for everybody to know about.

DR. BAILEY: Just a follow-up, if we have had something that has come to us that has gone through this process previously?

DR. COPELAND: No, not that I'm aware of.

Any other questions or comments?

On the phone?

Okay, so there is your reports and

products.

And last is term limits for nonvoting members. Currently, we have up to 12 positions. The appointments are based on written requests from organizations, and nominations are sent to me, to the associate administrator in the Child Health Bureau, HRSA, and the Secretary, ultimately. Once nominated, there is no limit to the time despite rapidly changing landscapes.

So what we would like to do is develop categories of liaisons to be determined with a set number of representatives in those categories. This would be developed by HRSA and ex officio members of the categories at some point in time. And every 4 years, the liaison position will roll off or be selected for another four-year term.

So the rationale is all of our voting members have term limits, and the purpose of this is to get influx of new ideas. Newborn screening and heritable disorders represents a huge catchment for the stakeholders other than the 12 positions alone can fulfill. And the last one, equity and

distribution of influence upon the advisory committee.

Yes, Alexis?

DR. THOMPSON: Do you anticipate not only the person but the organizations are changing?

DR. COPELAND: It is the organization if there subject to the nomination process. The nomination process will be very similar to that for the members, but it will be that at the organization level as opposed to the personal level.

Questions? On the phone? Liaisons?

Yes, Chris?

DR. KUS: You're moving us to the outside table and then we fall off the table, is that what you're saying?

[Laughter.]

DR. KUS: I'm just checking.

DR. COPELAND: Well, that's a thought. We're going to put a trap door in.

DR. KUS: That was my one joke for the day.

DR. COPELAND: We're going to put a trap

door under you. Either that or the Gong Show, one way or another.

Natasha, did you have a comment?

MS. BONHOMME: I did. When would that 4 years start? Is that when the --

DR. COPELAND: We're going to stagger them because I don't want to do a nomination package for 12 members. So you will be notified. But no one is rolling off this year. It would start in the next year or so. So you will be notified when you would roll off and how that would work. We don't have a process for that. It's an idea.

Yes, Alexis?

DR. THOMPSON: What if an organization, from their point of view, the person -- say that organization is selected for continued representation. Would that organization -- that individual really is their most effective person for this committee. Do they have any control over the ability to maintain that person on the committee or must that a person change?

DR. COPELAND: No, not at all. It is a

position for the organization, which is what it is currently. And it is at the discretion of the organization to name who sits at the table.

Okay, and this is just kind of a policies and procedure thing. Currently, our policies and procedures include all the details that are considered bylaws as well, so we have a mishmash of everything. So at recommendation by legal counsel, we are separating out the bylaws from the policies and procedures, because you guys don't want have to vote on every time we change a word in the standard operating procedures. So the bylaws require a formal vote by the advisory committee. And again, this is to align it with what the legislation says.

And that is just kind of a formal note for you guys to know. Any comments or questions?

You should have a copy of the revised bylaws. You do have them in your briefing book. And you also have a draft of the revised policies and procedures, which is more programmatic decision, but just to give you an idea, I covered it all in my talk, the broad strokes.

So the vote today is for approval of the bylaws. An aye vote would result in immediate implementation of the processes that do not require a change to the charter.

CHAIRMAN BOCCHINI: All right, so before we go forward, are there any additional comments or questions?

Andrea?

MS. WILLIAMS: I have a question about the other members, the nonvoting members and the process, because I think there is a value in having --

DR. COPELAND: They can renominate themselves to stay on, so it's not like they automatically roll off. That's part of the policies and procedures we haven't established yet, about if there will be seats at the table -- but at this point in time, the plan is that they can renominate themselves and if they're the best nominee for that category, then they would maintain a seat.

CHAIRMAN BOCCHINI: Okay, if there are no additional comments -- Coleen?

DR. BOYLE: Just a clarification of what we are voting on, so that on the bylaws, the highlighted sections and the changes?

DR. COPELAND: Yes.

DR. BOYLE: It was hard to --

DR. COPELAND: Yes, yes. The highlighted changes are the changes to the bylaws, and those are what you're voting on today.

CHAIRMAN BOCCHINI: And then language changes of the rest of the part of that is not is what is being voted on today. Those would be adjustments that would be supplement, be made independent of this vote.

So we need a nomination from the committee to accept these changes in the bylaws.

Stephen, motion to accept.

DR. COPELAND: We have to know, does anybody --

Oh you were asking for second. Go ahead.

CHAIRMAN BOCCHINI: Right.

Do we have a second?

DR. COPELAND: Okay. So does anybody

abstain?

Okay, if you can go through the roll call?

CHAIRMAN BOCCHINI: Okay.

DR. BAILEY: Just to clarify, we are voting on the highlighted wording changes in the document you set us ahead of time?

DR. COPELAND: Yes, in the bylaws.

DR. BAILEY: I vote aye.

CHAIRMAN BOCCHINI: On second?

I approve.

Jeff?

DR. BOTKIN: Approve.

CHAIRMAN BOCCHINI: Charlie?

DR. HOMER: Approve.

CHAIRMAN BOCCHINI: Fred Lorey?

DR. LOREY: Approve.

CHAIRMAN BOCCHINI: Steve McDonough?

DR. MCDONOUGH: Aye.

CHAIRMAN BOCCHINI: Dietrich Matern?

DR. MATERN: Approve.

CHAIRMAN BOCCHINI: Alexis Thompson?

DR. THOMPSON: Approve.

CHAIRMAN BOCCHINI: Catherine Wicklund?
MS. WICKLUND: Approve.
CHAIRMAN BOCCHINI: Andrea Williams?
MS. WILLIAMS: Approve.
CHAIRMAN BOCCHINI: Then for Agency for Healthcare Research and Quality, Denise Dougherty?
DR. DOUGHERTY: Approve.
CHAIRMAN BOCCHINI: Centers for Disease Control and Prevention, Coleen Boyle?
DR. BOYLE: Approve.
CHAIRMAN BOCCHINI: FDA, Kellie Kelm?
DR. KELM: Approve.
CHAIRMAN BOCCHINI: And Health Research and Service Administration, Michael Lu?
DR. LU: Approve.
CHAIRMAN BOCCHINI: And then the NIH is absent.
So the outcome is approval.
So this is the second time that we are ahead of schedule. Either Sara is being very kind to me in my first attempt to run this meeting and gave me extra time so I wouldn't be behind.

So I think we are in good shape, and so I think this gives us a little extra time for lunch.

DR. COPELAND: It does, which is good because there is no lunch provided for anybody here.

Yes, Coleen?

DR. BOYLE: Can I ask a question?

CHAIRMAN BOCCHINI: Yes, Coleen?

DR. BOYLE: You presented a lot of other things that we didn't vote on, which were a part of the bylaws. So when will we come back to those?

DR. COPELAND: Those are programmatic decisions, and I wanted to get a discussion and an assent as opposed to a vote.

DR. BOYLE: Okay.

CHAIRMAN BOCCHINI: All right, Stephen?

DR. MCDONOUGH: I have a question going forward on the reauthorization, since we are involved in making recommendations on some provisions that are not necessarily genetic. Has there been discussion that the mission would be newborn screening and heritable disorders. Going through the process of getting approved 4 years down

the road, do we have to change our title to reflect what we're reviewing and commenting on?

DR. COPELAND: I'm conferring with my legal counsel.

We are not going to change the name. I can tell you that. I think we need to really carefully consider -- newborn screening alone is enough to be within the scope of the advisory committee or not, but I think it could be argued even on hyperbilirubinemia that there's definitely a genetic component to it, so there's a heritable component.

So for things -- as a geneticist, I like to feel I'm very important, and I can't think of anything that is not heritable.

[Laughter.]

DR. COPELAND: But it is a consideration.

CHAIRMAN BOCCHINI: Jeff?

DR. BOTKIN: This is a separate topic, but since we're talking about recommendations and process. We have the congenital heart disease recommendation that came along with several

additional elements to that, recommendations that were targeted to both HRSA and the NIH, and I think those came along kind of at the last minute. As I understand, it created a bit of a conversation about the appropriateness of this committee making those sorts of recommendations.

I want to see if there's any conversation about that aspect of the process, because clearly we're going to have circumstances where we're not going to make a positive recommendation but there are data elements out there that we think need to be addressed. We want to make recommendations for how to fill the data gaps for addressing certain kinds of issues, or we may have positive recommendations along with some specific additional recommendations, the way we made with the general heart disease statement.

So the question is, do we need more thought about how that process works, to make sure those additional recommendations are appropriately vetted before we approve them, so that they are realistic and those other agencies can appropriately

respond to those?

DR. COPELAND: That is my goal with bringing the whole idea about what is in the purview and not in the purview of the Secretary, to really consider recommendations as we go forward.

One of the provisions that are in the bylaws is that we won't vote unless it has been scheduled, so that should give adequate time for discussion of recommendations. But it is definitely -- the advisory committee can do any recommendations it wants, whether or not it is actionable, whether or not it is appropriate. But whether or not we can act on it is a different issue entirely, and it's at the discretion of the advisory committee to make those decisions.

DR. LOREY: Hello, this is Fred, can you hear me?

DR. COPELAND: Yes.

DR. LOREY: I want to make a comment to I agree with both your statement and the previous one. I'm a little concerned about how it has gone in the past, because in the heart defects situation, the

committee really did not consider the effect on public health laboratories. And when the public comment came, they didn't allow any public comment. I know that three or four of us were waiting at the mike and they wouldn't even let us speak.

So although I agree with you that ultimately everything is genetic, we need to narrow it down somewhat, I think. And also, in the future, we really do need to consider the appropriateness, and now that the definition of newborn screening has expanded, who exactly -- should be doing the procedure?

DR. COPELAND: Your point is very well taken, and hopefully, that will be included in any of the public health impact analysis that occurs with the conditions review. And again, with the scheduled votes, people will know ahead of time that things are coming up for vote, so that there is ample time for discussion.

DR. LOREY: If I remember correctly about that, that vote was not scheduled that day, and then they voted to vote anyway. And so I'm hoping that

won't happen again.

DR. COPELAND: Yes.

DR. LOREY: Do you remember that?

DR. COPELAND: Yes, I do remember that. And we learn as we go along, because even though we are at 26 meetings, we're really a very young committee, so we are still learning.

Thank you.

CHAIRMAN BOCCHINI: Any additional comments?

All right, I think that is the other important thing, that Sara mentioned earlier, is interaction with the U.S. defense services task force and trying to harmonize our evidence reviews, so that it will be acceptable to the standards that they have created, so that, as they do with ACIP, the preventive task force does not look at anything related to immunizations. They send that to ACIP.

And so in the case of newborn screening, there could be issues that they would than refer to them, would refer to us, and likewise there might be issues for conditions nominated that come to us that

might be better served there. And I think that looking at it from the beginning all the way to the public health impact is very important for this committee, and that we standardize the way that we approach it all the way through. So I think that's part of the goal with this.

Okay, well, if there are no other comments, because we're early --

DR. BOYLE: I'm sorry. You said something. I mean, this is very complicated, obviously. I'm trying to understand the different ways, and I know that in the early part of this committee that we did have discussions about what is the lane for this committee, and Sara said most disorders have a genetic component, so that doesn't help very much. I'm sorry, I kind of got lost in all the different processes here, but is there a need, since it sounds like we're sort of rethinking the committee a bit, to kind of go back to that? Is that something that the committee can find guidance to both HRSA, HHS, the Secretary, thinking about that? And maybe you already said that, and I

apologize.

CHAIRMAN BOCCHINI: No, no. I think this is the beginning of the discussion, and I think that as Sara indicated, our hope is to develop a method group or a methods policy book that would then enable us try to clarify some of these things. And we're attempting to put together a conference to go ahead and do that.

DR. BOYLE: I see it beyond methods. I guess I'm thinking about the healthcare system, thinking about the opportunities, thinking about what makes sense. I guess I'm thinking maybe more conceptual than process.

CHAIRMAN BOCCHINI: Yes, that is important, and I think we need to have that discussion with entire committee. But you're right, I think that essentially we're beginning that process.

So we are going to move the afternoon session to start at 1:30, so that we can get through the afternoon session on schedule. We may be able to then make sure we get all the public comments in,

as well as get to the rest of the work before our subcommittee meeting.

So if there are no other comments, we will restart at 1:30.

Thank you all very much.

[Recess.]