SECRETARY'S ADVISORY COMMITTEE ON HERITABLE DISORDERS IN NEWBORNS AND CHILDREN

Thursday, January 26, 2012

Morning Session–Part 1

8:30 a.m.–10:00 am

Park Hyatt Hotel

Washington, D.C.

APPEARANCES

COMMITTEE MEMBERS:

DON BAILEY, PhD, MED

JOSEPH A. BOCCHINI, JR., MD (Committee Chairperson)

JEFFREY BOTKIN, MD, MPH

CHARLES HOMER, MD, MMPH

FRED LOREY, PhD

DIETRICH MATERN, MD

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CATHERINE A.L. WICKLUND, MS, CGC

ANDREA M. WILLIAMS, BA

EX-OFFICIO MEMBERS:

COLEEN BOYLE, PhD, MS

DENISE DOUGHERTY, PhD

KELLIE B. KELM, PhD

MICHAEL LU, MD, MPH

DESIGNATED FEDERAL OFFICIAL:

SARA COPELAND, MD

APPEARANCES (Continued)

ORGANIZATION REPRESENTATIVES:

NATASHA BONHOMME, BA
FREDERICK M. CHEN, MD, MPH, FAAFP
JANE GETCHELL, DrPH, MT (ASCP)
CAROL GREENE, M.D.
WILLIAM A. HOGGE, MD
CHRISTOPHER KUS, MD, MPH
BENNETT LAVENSTEIN, MD
JOE LEIGH SIMPSON, MD
BETH TARINI, MD, MS, FAAP
MICHAEL S. WATSON, PhD, FACMG
MARY J.H. WILLIS, MD, PhD

C O N T E N T S

P R O C E E D I N G S

CHAIRMAN BOCCHINI: I would like to welcome everybody to the Secretary's Advisory Committee meeting. This is the 26th meeting of the Advisory Committee on Heritable Disorders in Newborns and Children. And because of the changeover that has occurred from the last meeting, we thought that -- and the new members that have joined -- it would be good to start off by orienting the committee to the Secretary's Committee and to HHS.

So we're going to start this morning with a review to provide that orientation, and then we will get into the regular committee meeting. So we will start first with a discussion of ethics for special government employees. Dheeraj Agarwal is here from the HRSA Office of Management, Division of Workforce Management. He is an ethics advisor in HRSA's Division of Workforce Management. His role is to advise HRSA employees on all ethics matters and ethics specific to special government employees.

Thank you.

MR. AGARWAL: Thank you very much. Thank you all for coming.

What I would like to do, in the interest of time this morning, because I know Dr. Wakefield is on the agenda for 9 o'clock, we have an excellent video, that the Office of Government Ethics has produced, that specifically talks about ethics for special government employees. It covers the key areas, such as financial conflicts of interest; misuse of position; teaching, speaking, writing; and appearance of bias or loss of impartiality.

So what we would like to do is go ahead and run that video. And at the conclusion of the video, if we have time, we will take questions. And if we do not have time, what we will do is take any questions that are particular to your situation as it relates to ethics off-line, and we will research those and get back to you.

So I can ask the folks to please turn on the video. I would appreciate it.

[Video presentation.]

MR. AGARWAL: So I think we will have to

work with OGE to make that high definition, because it is kind of old. But while it is an old video, it actually covers all of the ethical areas that apply to special government employees. We think it is an excellent video, because it does talk and give you all examples of such things as an appearance of conflict or how to get an exemption or what you can do in the teaching, writing, or editing arena, and so forth.

One of the cardinal rules that we advise in the ethics department is that if you think that you have an ethics issue, or even if you have any particular matter that you think may come up in the future that presents an ethical conflict, always come to us first. It is our job to research it to see if there's a real conflict or to see if there is an appearance of conflict.

In either situation, our job is to work with you and the appropriate parties to make sure that there is a remedy. As the video spoke to, there are different ways to remedy a conflict of interest, everything from waivers to authorizations,

to the divestitures, to actually resigning from your special government employee position.

So are there any questions that I can take at this point in time? And if they're not, what we can do is, if you come up with any particular questions after you take in what you saw today, we will be able to research those and get back to everyone, as appropriate.

CHAIRMAN BOCCHINI: Thank you very much.

Any specific questions at this point?

If not, again -- I'm sorry?

Dieter?

DR. MATERN: Since this meeting is public, so we don't have to worry about others?

CHAIRMAN BOCCHINI: Correct.

All right, thank you.

MR. AGARWAL: Thank you very much.

CHAIRMAN BOCCHINI: Now I have the pleasure of introducing Dr. Mary Wakefield. Dr. Wakefield is the administrator of the Health Resources Services Administration, HRSA. She was named administrator in 2009 by President Barack

Obama.

And she took this position while she was at the University of North Dakota, where she was Associate Dean for Rural Health at the School of Medicine and Health Sciences. She has considerable experience both on Capitol Hill and in leadership roles and policy development at multiple institutions, and including serving as director of the Center of Health Policy Research and Ethics at George Mason University, and working as a consultant for the World Health Organization's Global Program on AIDS.

Dr. Wakefield, we appreciate you taking the time to be here.

DR. WAKEFIELD: Thank you very much. It is a pleasure to be with all of you.

And congratulations and let me also thank you, Mr. Chair, for your willingness to lead this advisory committee's very important work going forward. We have had the pleasure of being able to meet and talk a little about the agenda and the way the committee conducts its work prior to today. I

appreciated the opportunity.

And as I said, we are most appreciative of your willingness to chair the committee.

I also would want to welcome new advisory committee members, and extend my appreciation to each of you for your willingness to join the ranks of the committee. It is a committee, for those of you who are new, that has a terrific record of substantive contributions. And we of course expect that that reputation will be carried forward.

And a special thanks to the full advisory committee members who have been serving to date. Their work has been very important to us, in terms of guiding and influencing national dialogue as it pertains to infants and children and families that are impacted by heritable disorders.

So thanks to all of you and a special welcome to new committee members.

I also want to take this opportunity to recognize Dr. Lu, Dr. Michael Lu, who is our new associate administrator for the Maternal Child Health Bureau, through which of course this advisory

committee is supported.

He is well-known in his field, and we're just delighted that he has agreed to be part of the HRSA leadership team. We look forward to a lot of great working contributions that will come both from him and from the team he leads going forward, in terms of maternal child health.

So welcome to Michael, who, for those of you who may not know, is relatively new on the job, although he has now been there for over a week. And we had a lengthy conversation yesterday, and it was, "Well, here are the 44 things I would like to see happen, Dr. Lu. How quickly can we make them happen?" And he is still trying to find his way around the Parklawn Building.

So again for those of you have been to the Parklawn Building, you would appreciate that even that in and of itself was a challenge. But the good news is that he has not just expertise but high energy and commitment. And there's a lot of great work to be done.

So you will be interfacing of course with

him more as our representative on the committee going forward.

I appreciate the opportunity and the invitation to be here and spend just a few minutes with you to talk a little bit about your work and some of the broader work out of which your deliberations are part. Clearly, your committee offers consumers, family members, healthcare providers, national organizations, and advocacy organizations, other advocates, researchers, state health officials, a vehicle through which they can join forces, if you will, to really try to bring expertise to inform the work that all of us are engaged in, with the aim of reducing mortality and morbidity associated with genetic disorders.

The utility and the relevance of your work is apparent in a lot of different ways. And I want to just give you a few examples.

For example, the systematic reviews that this committee has coordinated have provided new mechanisms for evidence-based reviews of rare conditions, an important contribution.

Additionally, this committee has offered really a unifying force for the consideration or modifications or additional conditions to the newborn screening program.

And this committee is also a link to a very dynamic and expanding field of genetics and public health. What an interesting nexus. At least in my former training as a nurse, I wouldn't have been thinking about that in the late '70s or mid-'70s, to be perfectly honest, when I graduated from my nursing education program.

And that link also helps to provide a framework for the discussion of those interactions in the context of public health and the field of genetics.

I would also say that just for illustrative purposes, with a little more specificity, in other words, that we can consider what has followed your 2010 recommendations to begin screening for severe combined immunodeficiency disorder. Pilot studies in various states currently now cover approximately 25 percent of births in the

United States.

And according to your May 2011 report, nearly 962,000 newborns have been screened, and 60 infants, or about one in 16,000, have been identified with some form of immunodeficiency. Fourteen infants with SCID have been diagnosed and received treatment. And your committee report concluded that screening for SCID has almost certainly saved lives. Just one concrete example of the impact, if you will, of the work of your committee.

In many ways, I think that this advisory committee helps to level the playing field for infants across the nation. No longer are some states, for example, only screening for three or four conditions and others screening for more than 30 conditions. But now we have a standardized approach across the country, a more robust set of screenings, with a minimum requirements of 29 disorders screened. So that infants don't any longer, across these 29 disorders, miss the potential for life-saving and life-altering

screenings just by virtue of where they live.

So it's an important contribution, another example of an important contribution, of this committee's work.

The CDC has recognized advances in newborn screening and your important role in guiding the field and setting the standards with the uniform screening panel. CDC found that "improvements in technology and endorsement of a uniform newborn screening panel of diseases has led to earlier life-saving treatments and intervention for at least 3,400 additional newborns each year with the selected genetic and endocrine disorders."

Furthermore, for example, another example, the percentage of infants who now receive hearing tests has risen to less than half of all babies born in the United States in 1999 to 97 percent today. So simply put, those illustrations, I think underscore that the efforts of this committee have had, in very real and direct ways, an impact on the health of infants of children.

Five years ago, you advised that newborn

screening was one component of a larger process. Your 2007 roadmap report envisioned "a broad enterprise of which long-term follow-up is an essential part and enterprise that involves many players who need to work in partnership."

Somewhat related to that, in your 2009 advisory report recommendations, you prompted primary care professionals as a subset of those key important players. As you noted, "primary care providers should recognize that genetics and genetic medicine will be an integral component to care and should development an effective strategy for incorporating clinical genetic medicine recommendations into their clinical practice."

So very important contributions. HHS and HRSA are very proud to have the committee as a partner and as a resource to inform our work, too.

In addition to reflecting back, though, in the minute or two I have left, I also want to take just look forward at a couple key areas.

And I want to stop for a second, because I failed to do something that I should have, and that

is to also acknowledge some other HRSA staff that have done just a spectacular job in supporting the efforts of this committee and that have made major contributions in their own right, in the context of improving health for children and women, and that would be Bonnie Strickland in the back of the room.

Bonnie has been a key link to this committee's work and is probably in my office talking about your work more often than she would like to because this is part of her portfolio of activity. But she is a very enthusiastic and committed supporter of what you do.

And even more directly, Dr. Sara Copeland, who is here at the table with you, has also been just terrific in terms of providing a guiding hand and very thoughtful approach to ensuring that we can maximize the contributions of the committee in very important and direct ways. And her expertise is much appreciated as well.

So I wanted to call out some of the assets, human assets, that are here to support the work that this committee does.

I also wanted to comment just a bit on your work going forward, both in terms of the operations, how you go about your work, as well as what is underway and the broad environment in which you are conducting your activity, and I'll just make a nod in both of those directions.

First of all, it goes almost without saying in a room like this that in the United States today, of course, our infant mortality rates remain a really harsh reminder that we still have unfinished business from the 20th century that we have carried over into this century in terms of rates of infant mortality. It is pretty clear that even for all of the strengths of our healthcare system, of which there are many, in this country too frequently there are gaps in our ability and our execution to protect the very smallest and sometimes the most vulnerable of our population.

So we continue to have a lot of work to do around improving the health of the nation's infants and children. That focus, that aim, is of paramount importance to this administration. And both to

Secretary Sibelius, for example, not just HRSA or CDC, but to Secretary Sibelius and to the President as well. And they have, at higher levels of the administration, committed resources accordingly, recognizing the importance of doing everything that we can to protect the health of our smallest and often most fragile.

For example, just to underscore that point, the President has of course engaged very early on in his administration, the expansion of the Children's Health Insurance Program. That boosted insurance coverage for children from low income families from what it was then back in 2009, right around 7 million children with insurance, to where we find ourselves now as a result to CHIP, 11 million kids that have access to health insurance coverage. I think everyone of us knows in this room what that can mean in terms of screening, access to screenings for illness prevention and access to health-promoting healthcare services.

So that was a marker that was laid down very, very early in the administration. And since

then, of course, the Affordable Care Act has started to roll out provisions that are guiding what we're doing both within HRSA and more broadly across HHS with a new focus on expanding access, improving quality, and really, not talked about very much in newspapers, et cetera, we're really focusing on preventing illness and mitigating chronic conditions, promoting help for all Americans in no small part by breaking down barriers to screening services.

So you see immediately a nexus between what many of the provisions of that broader piece of the law look like and the very work that you do right here, a real crystallized focus on ensuring access to screening services, primary care and preventive care as well, especially for the young.

Some of the provisions that I want to just mention, because I think they have clear implications for individuals and families impacted by heritable disorders, are worth noting, again because they provide the environment and context for your specific work. So for example, the Affordable

Care Act now bans copayments for basic preventive healthcare and screenings, and ends the practice of denying coverage for pre-existing conditions. That is an incredibly important provision, ending the practice of denying coverage for pre-existing conditions. That is about as important as we can get, I think, in some respects in protecting children and families with heritable disorders and impacted by heritable disorders. The Affordable Care Act has another important provision that allows dependents to remain on their parents' health insurance plan until age 26. Our recent data just a couple weeks old shows us that we have about a million more individuals, young adults under the age of 26, that now have insurance coverage as a result of that provision.

Why does that matter to you why should you care about that? It's pretty obvious. Because many of those individuals under age 26 have and had heritable disorders that would have been, until now, previously uninsurable as they entered young adulthood. So it's an important provision of the

population that we are here talking about.

Also, of course, in the law and relevant to the population that is the focus of your work, we have the establishment of high-risk insurance pools that provide a safety net for people with pre-existing conditions that were previously denied coverage.

And another important provision relevant to this population, the elimination of lifetime caps on benefits. That really is a particularly cruel burden that families and individuals, infants and young children have had to face in order -- a barrier if you will to their obtaining necessary often life-sustaining regular costly therapies.

The law also reauthorized until 2015 the Family-to-Family Information Centers Program. Those are about 41 centers across the United States staffed by parents with children that have special health needs. And through those 41 family-to-family centers, you have seen the linkage of about 170,000 families with special health needs, through federal programs, state programs, clinics, special insurance

pools, rehabilitation services and so on, to give kids with disabilities, including heritable disorders, a better shot, and their families a fuller understanding of what resources might be available to them.

Also, a brand-new provision within the Affordable Care Act that HRSA has been very involved with implementing is the deployment of the home-visiting program that is administered by HRSA in conjunction with the Administration on Children and Families. Under that program, you might know, social workers, nurses, other healthcare providers, visit expectant mothers and their families in high-risk communities. There those providers engage counseling services that are really designed to improve health outcomes for mothers and infants focusing on issues like school readiness, for children, for example, focusing on economic self-sufficiency and other dimensions important to ensuring that we have the best possible platform that we can help build under those families in those high-risk communities.

The research underpinnings of that program, of the home-visiting program, are solid. They clearly indicate the interventions sooner; decrease the need for more costly clinical care and social services later.

So that is an evidence-based provision in the law that is being deployed across the country now and certainly will have implications too for families, that subset of families that have children with heritable disorders.

And of course, the last part that I want to raise for you that again has the potential to impact the same population that is the business about which you are engaging here, your committee's work, is to call out to the committee the health centers investment. It might not be readily evident for you about how that work also impacts this population, but just to give you a frame of reference, currently, health centers provide prenatal care to about half a million expectant mothers every year, about 67 percent of those mothers there are seen in their first trimester in

the health centers. And the health centers are linked to about 170,000 births in this country. So people don't always think about that in terms of the role of community health centers, in terms of prenatal care and care for neonates, but they have a very significant footprint.

Health centers also, just to extend this a minute more, also immunize about 233,000 kids under the age of 2 each year. They administer lead tests to about 300,000. They manage over 540,000 cases of asthma, provide early detection and screening that identifies an average of thousands of children per year who have hearing, developmental, perinatal disorders and other conditions.

So the point is that as a community health centers system, that peace in the Affordable Care Act expands as it is now and will over the next few years, as it expands, so too will the population that is served by that safety net infrastructure. And over time, prenatal care, as a result of that expansion, will be available to tens of thousands of additional mothers, and immunization and screenings

will be offered to tens of thousands more infants.

And those patients, as an aside, are more and more cared for in the context of health homes or medical homes. Health centers across the country are increasingly picking up on, where they didn't have them already, dimensions that are used to coordinate care and in the process boost quality.

In addition to the Affordable Care Act, we have a lot of other things underway, too many to call out now. But I wanted to just mention two of them. One of them that I hope you have heard of is HRSA's partnership with the National Healthy Mothers, Healthy Babies Coalition, and it is partnership that we engage with CDC and which the White House is very much part of when this was first initiated. This is the Text for Baby effort that is underway. It is an opportunity that allows us to provide expectant mothers and mothers that have given birth with free text messages, reminding them of how they can take care of their own health and the health of infants and really help to give them the opportunity to give their babies really the very

best start in life.

And those text messages are timed to the birth date. So they are tailored to where that woman is in her pregnancy and/or postdelivery. Text for Baby sends messages three times a week too young and expectant mothers who otherwise might be hard to reach, but who typically often have cell phones readily available to them. And this has been a public-private partnership that the White House is very much part that has continued forward, and that work needs to be able to engage in as well. It includes of course reminders to mothers about the importance of screenings for their newborns. So it is a great use of technology and resources, and as I said, an important public-private partnership. In fact, we are now finding ways to extend that out to older children ages 1 to 5 and using that technology in other ways as well.

So there certainly are opportunities to expand what we are doing and how we exert our influence more broadly on ensuring the importance of screening and the access to follow-up services

through the array of provisions that I have mentioned thus far.

The other point, the last point that I just make with all this, is just to say that hopefully this provides you with a sense of what we are engaged in at HRSA and at HHS and the administration more broadly, that we have the opportunity to, I think now perhaps more than ever, to really couple our efforts with yours, to strengthen the healthcare and the health of infants and children in this country, and to ensure that when we do have challenging healthcare problems that are impacting families across the nation, we have far better more robust tools to bring to the table to impact more favorably those challenges individuals wrestle with across the Nation.

You as a federal advisory committee of course have a different role from what I just described. But I just wanted you to know that your commitment, your advice to us, ensures that we do give infants in our country the very best start in life and that we provide the services needed wrapped

around children with very special health needs. Your advice to us and your expertise to inform the work that we have underwear already is critically important. It is a great opportunity, I think, for all of us now to engage what are extremely important challenges.

And I welcome you again, thank you again, for your service. And if there's anything that I need to hear from you, need to know, or you might have a question about, I would be happy to try to take that for just a minute now, and then I am going to not stay, but I need to chair a meeting back over at that really lovely Parklawn Building that Dr. Lu now gets to call home. I'm sure it rivals his former setting back in California. Not.

So is there anything in particular, to the Chair, committee members, that I either need to hear or can respond to you in terms of your questions or concerns?

CHAIRMAN BOCCHINI: Dr. Wakefield, thank you very much for coming and for that presentation. I think it is very clear the role this committee

plays for HRSA and how HRSA looks at the committee and its contribution to your overall efforts, so we appreciate that.

DR. WAKEFIELD: Of course. I might just add that as you know, and we've already discussed, this committee's reauthorization is up in 2013. And I think this would be a good time for the committee to start to think about examining how you function, how you do your work, in the context of what I just shared. As you know, and I probably should've said this, we have lots of tools, but we are also operating in an environment where there is scrutiny, and appropriate scrutiny, I would say, on how we deploy our resources.

So even as you conduct the important work that you are doing, I would ask you to pay very close attention, to think about how we position the operations of this committee, to consider how you engage your effectiveness in a context of what we're seeing as another backdrop, and that is budget deficits and so on. Your ability to streamline operations of this committee will be important.

Ways that you can identify to improve the efficiencies of the committee to meet your statutory expectations also are extremely important.

We welcome your ideas, and I know that is part of your agenda today. That is extremely important, not just in terms of meeting the expectations of the administration and Congress. It is a look that we are asking all of our advisory committees to engage in, just as, frankly, we're doing it in very real time within our Maternal Child Health Bureau and across every single bureau and division that we operate at HRSA. As a matter of fact, it's a conversation that I will have with our senior -- it's weakly conversation almost now that we're having back in our office in about 45 minutes. Dr. Lu will miss that conversation -- but this is all by the way of saying, you are not immune from those expectations to think about how we can really efficiently deploy our resources, no advisory committee is, no part of HHS is. So please help us with that, too.

CHAIRMAN BOCCHINI: To the committee, if

there are any questions for Dr. Wakefield?

If not, from the committee, let's go to raised hands.

DR. CHEN: Hi, Dr. Wakefield, good morning. Nice to see you. I just wanted to point out a couple other really nice parallel features of this committee's work for the last few years. One is really to focus on evidenced-based medicine as applied to the way we analyze issues that come before the group now. And that is supported as well in the ACA.

The second is, as we move into more attention to service delivery models, the interprofessional work that happens around the care of these children and for these conditions is really worth noting, and I think a very strong example of some of the new models of care that are also supported in the ACA. At the same time, the challenges, as you mentioned, are very real. Budgetary ones facing public health departments around the country are ones that we run into as we think about how we make our recommendations, as well

as the expensive care for many of these conditions.

DR. WAKEFIELD: You make such great points, especially comments about coordinated care that capitalizes on the strength on an array of providers, particularly for populations like these that are the focus of this particular committee. HRSA and the ACA gives us new tools in that area. HRSA has been focused very explicitly on working with national organizations to try to drive that agenda forward.

In fact, it is really quite critical we think about redefining healthcare delivery and trying to support, for example, care organizations. We can't really think about that and get very far, I think, without also thinking about the individuals that are being deployed within those now increasing -- it's a sad thing when you don't turn off your own cell phone.

[Laughter.]

DR. WAKEFIELD: Sorry. Well, it is a great song. So, yes, those who are very young in the room, you probably wouldn't recognize it.

Yes, I have to remember what I was saying now. I know what I was going to say.

I had a little bit of a nod there, I was good to go on a little bit longer but I didn't. I had a little bit of a nod to that in terms of this committee's contribution, one I recognized earlier the statements from the much earlier report that talked about the different providers. It's not just the screening but then it's the delivery of services post to finding things and the importance of engaging primary care providers there. So I think you've already commented on this and you did it earlier than some have recognized the importance of engaging the broader team approach.

But clearly, that is a high priority for us. I think HRSA has a lot of programs that we deploy that deal directly in the area of strengthening the healthcare workforce, especially around primary care. And we will be continuing to push that agenda forward for all the reasons that I think will be quite evident to most of you.

And there are one or two more comments I

will take, but otherwise I will have to leave.

DR. KUS: Yes, Chris Kus from the Association of State and Territorial Health Officials.

One of the things you emphasized was that this group helped in getting states -- it didn't matter where kids were, that they were getting tested. I think in the discussion about the Affordable Care Act, it is important that the treatments shouldn't depend on where they live. And as we discuss the essential benefit package and things, that is a critical point for the work of this committee.

DR. WAKEFIELD: Sure. And the essential benefits package absolutely is relevant, and I think one more good example of a set of tools that we have broadly now, because of the law, that we can deploy going forward, so that's an extremely important point.

There are many other provisions that are important -- there are just too many of them to mention this morning -- but all of which, directly

or indirectly, affect and impact and relate to your work or at the very least the populations that your work is about.

So with that, thank you. I'm going to execrate myself and my cell phone

Thanks again to each of you.

[Applause.]

CHAIRMAN BOCCHINI: Next, we have Beverly Dart from the HHS Office of General Counsel. She is senior attorney with the council public-health division and has been with HHS OGC for 7 years, served 21 years with the Navy and the Department of Defense, including positions as a health law, international law and claims attorney. In her current position, she advises various HRSA programs. She is going to give us an overview of the Secretary's Advisory Committee legislation.

MS. DART: Good morning. It is an honor to be here today. I'm glad to have a chance to speak with you. I appreciate the invitation to talk about the legal framework generally for the work of the advisory committee. I think it provides a good

structural foundation for purposes of talking about your work.

I want to talk about basically two points. I should give you a little bit of background about as OGC attorney what my role as, as it has some bearing on the committee, and also talk about the legal framework generally, and specifically talking about the Federal Advisory Act, which provides one leg of the framework; the authorizing legislation for the committee itself, which is as another leg of the framework; and also the impact of section 2713 of the Affordable Care Act, the Public Health Service Act, which was added by the Affordable Care Act.

I think it is helpful to know a little bit about the role of the Office of the General Counsel, because you may have questions about some of the application and implementation of some of the information you here. I should explain the office of General Counsel provides legal advice and assistance to the department. We are government attorneys, so we advise at the broadest level the

department. And then basically from the microcosm down to the microcosm, so we finally will provide assistance specifically to HRSA and Maternal Child Health Bureau. And specifically, that is one of my designated clients across the board, as well as other HHS organizations and officials. And other OGC attorneys might be designated to primarily focus on other operating divisions, other activities.

And then finally we provide advice to program officials, who are specifically charged with implementation of certain programs, so specifically as it relates to the committee, to the DFO for purposes of committee matters.

So given that that is the role of OGC, what we don't do is that we don't give specific legal advice to the committee itself. Essentially, we're in dialogue, if you will, or maybe trialogue, if there is such a thing. But because the law sets a legal framework, it also has aspects of programmatic implementation, so our primary dialogue is with NCHB, the DFO as far as the activities of the committee and obviously informed by what is

occurring on the committee. So I offer that as my role, by way as context as well.

As far as the basic legal framework, the Federal Advisory Committee Act actually is our starting place. This is a federal law that was enacted in 1972, and it is further implemented by the Code of Federal Regulations as much substantive content to it. It is actually a GSA regulation, so the Federal Advisory Committee Act is broader than HHS. It basically stems throughout the Federal Government.

Its application is basically whatever either Congress or an agency establishes, or it manages as a group that essentially the role that is essentially made of people who are not originally Federal Government employees for the purpose of obtaining advice, recommendations, information, recommendations on agency issues, policies, practices. That is the general idea of what the Federal Advisory Committee Act was set up to address.

It was established in 1972 and in large

part it was in response to the fact that advisory committees had sprung up over time, and there was a sense that there was a need for greater information and standardization of what their actual practices were. They are also intended to advance the idea that the advice being provided by committees is objectives, is accessible by the public, and emphasizes therefore open meetings, the charter itself for the committee involvement of the public and reporting, all as evidenced by this meeting today.

You'll see that advisory committees under the Federal Advisory Committee Act do not necessarily have to be called advisory committees. The name is less important than the function that they perform. And it can be established by a variety of mechanisms. They can be established by statute, as in the case of this committee. They can be established or used by the President or by one of agencies. And again, the primary objective is to obtain advice and recommendations for whatever the designated federal organization is for the purpose

of doing the work that is established for them.

So in essence, the function of the advisory committees is to provide advice to, in this particular case, to the Secretary. And it's an advisory type function. It doesn't self-execute. It is advisory in nature.

The department also has certain legal statutory responsibilities relating to the administration of the work of advisory committees. It is supposed to establish uniform guidelines, management controls following the GSA directives found in regulation, and it also maintains information on the work of the committee in a variety of different areas.

The agency is required to designate an advisory committee management officer, who fulfills certain basically administrative functions, but you'll see is that they, again, provide a framework for the work of the committee. The first one most clearly so, that there is an element of structuring the procedures and accomplishments of what the work of the committee needs to be. And within HHS, with

respect to this particular committee, of course MCHB performs most of the responsibilities relative to this committee on behalf of the Secretary and HHS.

Now, I would like to move on to the actual authorizing legislation for the committee itself, and this is found in section 1111, I guess we can refer to it as, the Public Health Service Act, which is found codified in the U.S. Code has 42 USC Section 300(b)(10).

Doing a short summary of the essence of the law and what the authority of the committee is, essentially is it advice and recommendation to the Secretary in various specified areas related to newborn and childhood screening. You would see in the legislation itself that there is a lot of specificity, a lot more detail about what exactly is within that. But the law itself sets the basic parameters for the work of the committee. Further elaboration or application of those would be by the program officials who exercise that general structural role.

So the authorizing legislation basically

sets the main core legal standards for what is in its purview and what is not, and the essence of it is the advice and recommendations to the Secretary in certain identified areas, and that which is not within is generally without, subject to some degree of programmatic interpretation and application.

The third legal framework piece that we wanted to mention today was to talk about the preventive care and screenings that Dr. Wakefield mentioned. The Affordable Care Act added Section 2713, the Public Health Service Act. That has had a great deal of impact on the one aspect of the committee's work.

The recommended uniform screening panel, as many but not most of you know now, is part of the preventive care in screenings for infants, children and adolescents that are provided for in the comprehensive guidelines supported by HRSA as these are called for by the law. So basically what this means in practical fact is that those recommendations for additions to RUSP that are adopted by the Secretary and therefore included as

part of the RUSP now have impact in terms of being a requirement for them to be covered by non-grandfathered group health plans and health insurance issuers without cost share. So basically, once they are added to the RUSP by decision of the Secretary, there is a further legal impact, a further public health impact, as a result of that adoption. And this is only as the result of the enactment of the ACA.

So it must be, therefore, clear that what is required is -- even though it is the RUSP, the commissions on the RUSP, it is actually the adoption of the recommendations that actually triggers that effect.

So generally speaking, for purposes again of framework, the committee has a statutory purpose. Its purpose is to provide information, advice and assistance to the Secretary, and it is within that context that the committee functions.

At this point, I will just shift a little bit. I would like to mention, just based on the program, a couple programmatic takeaways from this.

This is specific applications of specific facts and what -- and how it programmatic. Lawyers give advice and counsel to our program clients, but ultimately decisions are made by the program.

So the takeaways from this are that the kind of things that are well within the purview of the legislation and basically the program would like you to know that these are things that are on your go-ahead, this is the area that you have a clear pathway forward, is the recommendation of additions of commissions to the RUSP, the recommendation of certain direction and advice to other HHS agencies, and specifically in the area of directing the Newborn Screenings Savings Life Act grant program.

The areas which are trickier, have more cautioning, certainly more need for dialogue with the program on, have to do when you're not providing that kind of advice and assistance and recommendations to the Secretary. So when you think in terms of making recommendations direct to states, that is an area for caution, as well is direct interaction and recommendations to other entities

other than the Department of Health and Human Services.

That's all I have for today. I would be glad to take your questions, recognizing that specifics and what exactly and how it would be applied in specific circumstances is really more of a program issue. So I would commend to you further discussion at other times.

CHAIRMAN BOCCHINI: Would you like to speak to the issues related to reauthorization?

MS. DART: Not really. I think Dr. Wakefield has really said it all.

CHAIRMAN BOCCHINI: Okay, thank you.

Additional questions?

DR. GREENE: Hi. I wonder if you could put back up the slide with the authorization for the committee?

MS. DART: I will try to do that.

DR. GREENE: Great. Thank you. Up on the screen, it has three bullets. And as you went forward, you mentioned only newborn and childhood screening. And then in your examples, you mentioned

only screening.

It is this committee's authorization only for screening? My recollection of the original language is that it was for anything to do with prevention of -- reduction of death and disability from a hereditary disease and not focus solely on screening.

MS. DART: I apologize. I may have shorthanded just a tad bit for purposes of efficiency in the providing of this presentation.

Is there anything else you want to say to that?

DR. COPELAND: Now, apart from the fact that, really, Beverly has been responding more to the previous actions and just making sure -- providing context for that. But as she said, she shortened it. This is definitely a heritable disorders committee. And for the past several years, it has been mostly focused on newborn screening, because that is where you can see a lot of the immediate impact, but definitely the scope is much broader than that.

I would also like to point out that I put together the slides with the pictures. I had to talk her into that.

[Laughter.]

CHAIRMAN BOCCHINI: Fred?

DR. CHEN: It may be a question for the Public Health, but the addition for the uniform panel to the requirements for coverage, I wasn't clear that private insurers, commercial insurers, paid for the newborn screening procedures currently. Is this a major change for labs and the coverage of the cost of screening labs?

DR. COPELAND: So currently, most newborn screening is rolled into the newborn screening fees, but sometimes those screens stand outside -- and those are often billed to the insurance companies, which is why in some states the insurance plans are so involved in what is added to the bill, because of the cost to them. The main point being even if it is on the RUSP, even if it is not on your state newborn screening panels, the insurance companies have to cover it.

So I think what that was the main point.

DR. KUS: So I'm trying to understand. So if a state that doesn't -- or is in the process of adding the condition, it would still be covered. What does that mean?

DR. COPELAND: So Iowa currently doesn't have SCID on their panel, but if a family wanted their baby to be screened for SCID, their insurance company would have to cover that because it is on RUSP.

DR. KUS: So it really is the parent requests in a state that is not covered. Is that fair?

DR. COPELAND: Well, for the most part, yes, but it could be a certain hospital has added --

DR. KUS: I got you.

DR. COPELAND: So there are some states it's not all uniform, the impact being that it's essentially a rulemaking, insurance has to cover it once it's added to the RUSP, whether or not states adopt it.

DR. BAILEY: So thank you for this review.

I'm a little confused about your comment about concerns about us making recommendations to the states, because essentially we're recommending things to the Secretary who then makes recommendations to the states.

DR. COPELAND: Exactly.

DR. BAILEY: Could you say anything more about that?

MS. DART: I think that's the point, if you will, that you make the recommendations to the Secretary, and those recommendations might take a variety of forms.

DR. COPELAND: But the idea being that the Secretary and her ability to influence the states has a limited scope, especially if there is going to be a requirement of additional funds, so it's just thinking about how we frame our recommendations and how we frame what we sent forward to the Secretary, so we don't put her in a position where we're asking her to make recommendations to the states that may or may not be approved.

CHAIRMAN BOCCHINI: Well, again, thank you

for the update. It's very helpful. Thank you.

DR. COPELAND: I have a sheet for roll call now and so I look around the table and see who is here, and on the telephone is Charlie Homer.

DR. HOMER: Yes, I am.

DR. COPELAND: And how about Fred Lorey?

DR. LOREY: Yes.

DR. COPELAND: Is there anyone else on the phone?

DR. GETCHELL: This is Jane Getchell.

DR. COPELAND: Okay, great. Thank you.

DR. GETCHELL: Thank you.

Now I'm going to do my housekeeping notes, so everybody can get oriented. When you exit the general session, the restrooms are all the way down the hall to the left at the end of the hallway on this floor. The food out there is not for us, sorry.

On the public comments, we had a lot of people sign up for public comments ahead of time, and you signed up out there as well. If you still want to speak, please be sure to put your topic area

next to it, so we can know, in order to maybe make sure that other people have a chance, if there's a huge group that wants to speak one topic, it would be nice if people who had other topics had a chance to speak as well. So when you sign up outside, let us know.

The Altarum staff will be at the registration desk to assist all of you and address any questions you may have.

There is not wireless access for everybody in the room, but the hotel offers complimentary wireless in the hotel lobby.

Subcommittees are held from 3 to 5, and they are all down on this floor, but Labs Standards and Procedures are in Salon 1 and 2 on the right.

Treatment is going to stay here.

And Education Training is next-door in the Gallery 3 ballroom.

If any of the presenters change the presentations after submitting them, please save the revised copy of your presentation on the laptop. And just so you know, on the agenda, the tab for the

presentation is indicated in parentheses, so if you want to tab back and forth to look at any of the background materials, et cetera, you can.

All the committee members, organizational reps and presenters should have received a thumb drive that contains a supplement to your briefing book. If you have not, please let us know. Stop by the registration desk.

And if you're going to dinner, please meet in the lobby at 6:15, and also indicate on the sign-up list.

CHAIRMAN BOCCHINI: Let's complete the roll by allowing each committee member to introduce themselves. Just tell us who you are and what area or organization you're involved with. And at the same time, if you would indicate whether you have any conflicts of interest, this would be important to identify individuals who may need to withhold from a vote as we go forward.

So the other thing that is important is that for the people who are recording this meeting, for them to be able to turn on your microphone,

which you just raise your hand before you speak so that they can identify who is going to speak. I think at this point, it will help them if we will start by with Coleen to introduce yourself, and go around the table for the committee members and then we will go to --

DR. BOYLE: Thanks. Good morning, everyone. I'm Coleen Boyle. I'm the director for National Center on Birth Defects and Developmental Disabilities at CDC.

MS. WICKLUND: I am Cathy Wicklund. I'm a genetic counselor and I direct the graduate program in genetic counseling at Northwestern University.

DR. DOUGHERTY: Denise Dougherty. I'm a senior advisor for Child Health and Quality Improvement at the Agency for Healthcare Research and Quality.

DR. THOMPSON: Good morning, I'm Alexis Thompson. I'm the head of pediatric oncology at Children's Memorial University at Northwestern University in Chicago.

DR. KELM: Hello, I'm Kelli Kelm. I'm

from the Division of Chemistry and Toxicology Device in the Office of In Vitro Diagnostic Devices.

DR. MATERN: I am Dietrich Matern, and I'm from the Mayo Clinic. I'm a co-director of the biochemical and genetics lab. Our laboratory provides screening for the State of Minnesota, at least part of the screening. I'm also involved in the NICHD contract that allows us to do comparative effectiveness study of screening of disorders and a few others.

DR. MCDONOUGH: Steve McDonough, I'm a general pediatrician from Bismarck, North Dakota. I've been there 32 years. I also spent 15 years of my career in the North Dakota Department of Health and oversaw the screening.

MS. WILLIAMS: I'm a Andrea Williams. I'm from the Children Sickle Cell Foundation.

DR. BOTKIN: Jeff Botkin, a pediatrician and I do bioethics at the University of Utah.

DR. BAILEY: Don Bailey. I'm a research fellow at RTI International, a nonprofit research organization in North Carolina.

CHAIRMAN BOCCHINI: Joe Bocchini. I'm chairman of the Department of Pediatrics at Louisiana State University Health Sciences Center in Shreveport.

DR. COPELAND: I'm Sara Copeland. I'm the acting chief of the Genetics Services Branch at the Health Resources Services Administration Maternal and Child Health Bureau in the Division of Services for Children with Special Health Needs. And I'm also the designated Federal officer for this committee.

DR. LU: Good morning. I'm Michael, and I'm director of the Maternal Child Health Bureau representing the Health Resources and Services Administration.

MS. DART: Beverly Dart. I'm an attorney with Department of Health and Human Services, and also general counsel.

CHAIRMAN BOCCHINI: And then the three people who are on the telephone? The two people on the telephone. Sorry.

Fred, do you want to start?

DR. LOREY: Sure. Fred Lorey. I'm not supposed to use the title, but I think I'm among friends, acting director of Genetic Disease Screening Programs in Richmond, California, and that includes newborn screening and prenatal screening.

CHAIRMAN BOCCHINI: And Charlie?

DR. HOMER: Yes, Charlie Homer. I am the CEO of the National Initiative for Children's Healthcare Quality, which is a nonprofit working on quality improvement for children. And I'm on the faculty at Boston Children's and Harvard Medical School and Harvard School of Public Health, and HQ also serves as the national coordinating center for two of Maternal Child Health Bureau's sickle cell programs.

CHAIRMAN BOCCHINI: Thank you.

Okay, liaisons? Fred?

DR. CHEN: Morning. Freddie Chen. I'm on the faculty at the Department of Family Medicine at the University of Washington, Seattle, and here as the organizational liaison of the American Academy of Family Physicians.

DR. TARINI: Good morning. My name is Beth Tarini. I'm faculty at the University of Michigan. I'm a pediatrician health services researcher. And I'm here as a liaison for the American Family of Pediatrics.

DR. HOGGE: Good morning. I'm Allen Hogge, from the University of Pittsburgh representing American College of Obstetricians and Gynecologists.

DR. GETCHELL: And I'm Jane Getchell, senior director for public health programs at the Association of Public Health Laboratories, and I'm here representing APHL.

DR. GREENE: Good morning. I'm Carol Greene. I'm faculty at the University of Maryland. I'm clinical geneticist, head of clinical genetics at the clinic there. And I'm representing the Society for Inherited Metabolic Disorders. I'm the current president.

DR. SIMPSON: I'm Joe Leigh Simpson. I'm just starting as a senior vice president for research and global programs at the March of Dimes.

I'm a obstetrician/gynecologist and a geneticist, certified in both, and previous president of American College of Medical Genetics, where I was honored to work with Dr. Watson who just walked in the room.

MS. BONHOMME: Natasha Bonhomme, project director for the Newborn Screening Clearinghouse and representing Genetic Alliance.

DR. HART: Theresa Hart. I'm the -- TRICARE Management Activities, representing the Department of Defense.

DR. KUS: Good morning, I'm Chris Kus. I'm a pediatrician and the associate medical director of the Division of Family Health at New York State Department of Health, and I'm representing the Association of State and Territorial Health Officials.

CHAIRMAN BOCCHINI: Mike, since you -- you just made it in time.

DR. WATSON: Mike Watson, American College of Medical Genetics.

CHAIRMAN BOCCHINI: Thank you.

You'll note we have a different configuration around the table today. The goal here is to follow more of what most Federal advisory committees do, which is having the voting members in the center, followed by the liaisons surrounding. There may be additional adjustments, but this is the reason for the change here.

The first item on the agenda for the committee is approval of minutes from the September 2011 meeting. These were sent to you prior to the meeting. Are there any additions or corrections?

Steve?

DR. MCDONOUGH: This is minor. On page 33, the third paragraph, discuss the disease, the third line. Since then, "a log of progress has been made." It should be "a lot of progress."

CHAIRMAN BOCCHINI: Thank you.

Additional?

DR. MATERN: On page 36, I don't think Dr. Howell said anything about type 1 -- I think targeted immuno type I.

CHAIRMAN BOCCHINI: Thank you.

DR. MATERN: On page 38, I think in the middle, "members feel the committee would benefit from participation by the American Congress of Obstetricians." I think it's the college.

CHAIRMAN BOCCHINI: There is a difference between the college and the congress. I think Dr. Hogge can probably elucidate us. I think for this committee, it is probably the congress.

DR. MATERN: And then there's another one on page 9. I don't know if that was actually stated, but it would be wrong. At the bottom, it says, "At the time of the advisory committee's inception, the majority of states were not screening newborns." I don't think that's right.

CHAIRMAN BOCCHINI: Okay.

DR. COPELAND: So in order to properly record and give the people that are recording this some time to figure out who's doing what, we're going to have people go one by one.

Do I need to ask for abstentions on the vote?

Oh, I guess we need to need a movement

first. Never mind.

CHAIRMAN BOCCHINI: If there are no other additions or corrections, can we have a move to accept the minutes with the corrections as indicated?

DR. MCDONOUGH: So moved.

CHAIRMAN BOCCHINI: Steve McDonough.

Second?

DR. MATERN: Second.

CHAIRMAN BOCCHINI: So then --

DR. COPELAND: Are there any abstentions for voting on the minutes?

MS. WICKLUND: I'm a new committee member.

DR. COPELAND: You get to vote. You don't have to abstain.

Are there any abstentions on the vote?

Okay, I'm going to go through the roll.

Dr. Bailey?

DR. BAILEY: Aye.

DR. COPELAND: Dr. Bocchini?

CHAIRMAN BOCCHINI: Approved.

DR. COPELAND: Dr. Botkin?

DR. BOTKIN: Approved.
DR. COPELAND: Dr. Homer?
DR. HOMER: Approved.
DR. COPELAND: Dr. Lorey?
DR. LOREY: Yes.
DR. COPELAND: Dr. McDonough?
DR. MCDONOUGH: Aye.
DR. COPELAND: Dr. Matern?
DR. MATERN: Approved.
DR. COPELAND: Dr. Thompson?
DR. THOMPSON: Approved.
DR. COPELAND: Ms. Wicklund?
MS. WICKLUND: Approved.
DR. COPELAND: Ms. Williams?
MS. WILLIAMS: Approved.
DR. COPELAND: And for AHRQ, Denise Dougherty?
MS. DOUGHERTY: Approved.
DR. COPELAND: For CDC, Coleen Boyle?
DR. BOYLE: Approved.
DR. COPELAND: For FDA, Kellie Kelm?
DR. KELM: Approved.

DR. COPELAND: For HRSA, Dr. Lu?

DR. LU: Approved.

DR. COPELAND: For NIH, they are absent.

So the overwhelming is approved.

Thank you, guys.

CHAIRMAN BOCCHINI: So we're considerably ahead of schedule. I guarantee you that this will probably be the last time under my tenure that will happen. But that is good.

So what we will do is I think we can initiate a break now, given the fact that we are ahead of the schedule. Shall we come back at 10:45 and then this way we can initiate the second part of the program a little bit ahead of schedule?

So with that, let's go on break and return at 10:45, to return to the meeting. Thank you.

Oh, I'm sorry, there is no committee correspondence.

[Recess.]