PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

BOARD OF GOVERNORS MEETING

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AGENDA

P R O C E E D I N G S

[1:03 PM]

CHAIRMAN WASHINGTON: Good afternoon and thanks to everyone for joining us at the Board of Governors of the Patient-Centered Outcomes Research Institute, and I would say Happy New Year to everyone. Thanks to those of you who are joining us here and present in person today, as well as to those joining us by phone.

We have a very busy agenda and some terrific news related to one of our greatest assets, which is the people who are joining our PCORI family that we're going to hear from Dr. Selby about in just a minute or so.

For those of you who are also joining us via phone or videocast, we are here in sunny Jacksonville, Florida and our next meeting, which is already listed on the website, will be in the great city of Baltimore, Maryland the first week in March.

And with that as the introduction, I'm going to turn this afternoon's program, at least

for this session, over to executive director Dr. Selby.

DR. SELBY: Thank you, Gene, and hello everyone. It is a pleasure to be here. This Executive Director's Report is going to be very brief. I'll come back tomorrow and fill in a few other details, but we have a really packed agenda this afternoon with discussions of our National Priorities and Research Agenda, the draft that's about to be put out for public comment after these discussions, news from the pilot projects, and also news from the Methodology Committee.

So, I want to, just as Gene said, introduce four new staff members who have joined us just in the last couple weeks and report that at PCORI it is beginning to feel very different as 2012 dawns, a lot more thinking power and a lot more staff to support the Board and Methodology Committee and to move our work forward.

So, let me, without further ado, introduce the four people who joined us about ten days ago now. First on the list is Dr. Lori Frank. Lori is

a researcher, first and foremost, with a doctoral degree in human development who does psychological research. She has been for most of the last 13 years at United BioSource, a research consulting firm.

She has particular areas of expertise that are crucial to PCORI's mission that include patient-centered outcomes research, patient-centered outcomes measurement of patient-centered outcomes related to symptoms and also related to cognitive function. She's also particularly interested from a scientific perspective in Alzheimer's disease and in depression in younger women.

She has led a large research team and she is our first PCORI scientist, and Lori, if you wouldn't mind just continuing to smile and standing up for a minute. Thanks.

And Lori's first mission is to support the Methodology Committee in getting the patient engagement portion of the Methodology Report ready for its release in May.

And I will move on to our Director of Stakeholder Engagement, and this is Judy Glanz. Judy -- is Judy here? Judy is not here. She was here and she will be here, but -- and you will see her, but let me just mention that Judy is a graduate of the University of Pennsylvania and has done graduate work at Johns Hopkins in health policy related occupational health.

She has worked at a number of organizations over the years including the EPA, the Campaign for Tobacco Free Kids, and most recently for four years at AARP as the director of outreach -- member outreach.

Judy will be responsible for PCORI's engagement with all stakeholder groups with the exception of patients. She will partner with a Director of Patient Engagement and together they will bring stakeholders together to provide input to PCORI as we make decisions about our Research Agenda and specific research that we'll fund and also as we plan for dissemination.

So, let's see, that is Judy. So, hello,

Judy. Bit wave. Welcome.

And next is Mr. Bill Silberg, who is our newly engaged Director of Communications, and he will be working very closely with Judy and with the Director of Patient Engagement when he or she comes on board to facilitate our work of maintaining two-way contact with all of our stakeholders.

Bill started his career at United Press International. He worked at the University of Chicago as -- in the communications department. He then went to JAMA and worked for a number of years at the Journal of the American Medical Association, after that at a new startup called MedScape, which began to take information to digital communications to the web for physicians and patients, which fits nicely with our ambitions here at PCORI.

After that, Bill was the Vice President for Communications for several years at the Commonwealth Fund where he introduced a new digital communications initiative to the Commonwealth Fund and has been running his own communications consulting business for the last several years.

So, welcome, Bill. Did you do a wave yet? Hello. Thank you.

And the fourth person I would like to tell you about today is Melissa Stern. Melissa comes to us from the Permanente Medical Group in Northern California where she was the Director of Population Care for the Department of Quality and Operations Services.

In that role she recruited, brought together, mentored, trained, and organized numbers of research associates to support sectors of the medical group as they worked on quality improvement and access improvement. So, strategic initiatives from strategic initiatives within the country's largest medical group to strategic initiatives at PCORI.

Here at PCORI she will grow a staff that will support the Board and Methodology Committee and subcommittees as well as staff projects. We see her as leading a strategic planning process within the staff and in collaboration with the Board and Methodology Committee, and also as tying

together the research activities at PCORI with the engagement activities.

We feel it would be a failure if we wind up with two separate camps, one doing engagement and one doing research, and Melissa will be part of the glue that holds those two sectors at PCORI together. So, welcome Melissa.

Just to give you a little bit of news, we have extended an offer to Director of Patient Engagement and we hope that we're able to give you the news very shortly that we have engaged our Director of Patient Engagement. We're very excited about this person.

We have also extended an offer to a Chief Science Officer, who is a very well-known and very accomplished researcher who will lead the recruitment of the scientific staff at PCORI and will have a big hand in working with the Board and the Methodology Committee in planning and then implementing PCORI's research agenda over time.

We're also posted for a Scientific Review Officer because we recognize that some of the

research we commission will be reviewed internally at PCORI and that person will oversee those activities. And we are currently recruiting for scientists. We anticipate hiring as many as nine by the end of 2012.

On the operations side, I'm very happy to say that we have just secured the engagement of a Director of Finance. I'm not quite prepared to give the person's name yet, but that announcement will be forthcoming and that person is on board. Highly experienced controller and chief financial officer in other organizations. With her on board we feel that finances at PCORI will be in wonderful shape.

We're recruiting for grants managers, project managers or associates, and then the staff that go with these positions.

And, in closing, just a snapshot of the organization of PCORI staff at this point in time. So, you'll see that there are -- we have a new meetings manager, that's Mr. Mark Freeman. Mark, would you -- Mark is not new, he's just in a new

position. And then along the horizontal bar, our operations folks on the left, the Director of Finance, and as I said, we will be recruiting grants management leadership.

In the middle, the orange boxes represent the three aspects of engagement -- communications, patient engagement, and stakeholder engagement. They'll be working together.

On the far right are the scientists, the scientific program staff and the scientific review staff, and in between, as I said, is the strategics initiative portion of the organization holding us all together.

And I think in the interest of time, Gene, that's the news from staff unless there are questions.

CHAIRMAN WASHINGTON: Any questions from Board members?

Well, to all of our new staff members, on behalf of the Board of Governors, welcome. We look forward to working with you to achieve the enormous promise of PCORI and advance healthcare and health

in America. So, glad you've joined us.

Next we're going to then move to a report from the Program Development Committee led by Dr. Rick Kuntz. Rick?

DR. KUNTZ: Thanks, Dr. Washington. Over the next hour and a half we're going to review the update of the National Priorities, the Research Agenda, and also an update on the PCORI Pilot Projects.

On Monday we'll be, pending the Board's review of this and the approval, we'll be sending out for public comment both the National Priorities and the Research Agenda, and we'll be anxiously waiting those public comments to process for the final establishment of our first version of National Priorities and the Research Agenda.

So, we'll go through that in a stepwise fashion, National Priorities followed by Research Agenda Presentation and then an update on the Pilot Projects.

First I want to turn this over to Dr. Clancy who will do the presentation on National

Priorities and also acknowledge Jessica Maddler [phonetic] on the staff who's been really instrumental in helping the development and presentation.

DR. CLANCY: Well, good afternoon everyone. I do need to -- this is the Program Development Committee. I do need to just make a very friendly amendment to Dr. Washington's earlier welcoming comments. It is actually not sunny here in Jacksonville, Florida. It could be, but it actually, in fact, is raining for those of you who were feeling envious of our terrific climate here.

CHAIRMAN WASHINGTON: Carolyn, that was a paid announcement. I didn't get you your share, which means they're going to take mine back.

[Laughter.]

DR. CLANCY: So, I'm going to lead off talking about the process that we have evolved for getting to Version 1.0 of National Priorities for Research. We'll then hand the baton, which is to say the clicker, to my colleague Dr. Krumholz, to talk about the first research agenda.

So, from the defining legislation from PCORI, Section 6301 of the Affordable Care Act, for anyone who would like the number, the purpose is shown here on this slide. The defining purpose is "to assist patients, clinicians, purchasers, and policymakers in making informed health decisions by advancing the quality and relevance of evidence relevant to the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through conducting and supporting research and evidence synthesis."

So, that comes straight from the law.

The National Priorities are actually a requirement state from the statute as well, so the statute not only directs that PCORI set National Priorities, but that they take into account factors such as disease incidence, prevalence, and burden in this country with an emphasis on chronic conditions, gaps in evidence, variations in practice, and health disparities in terms of

delivering and outcomes of care, the potential for new evidence to improve patient health, well-being, and the effect on national expenditures, as well as patient needs, outcomes, prevalence, relevance to patients and clinicians.

For those of you listening, if that didn't sound like a sentence, it wasn't. What I was reading from the slide were the specific words in the legislative language that were bolded and highlighted here.

And then the statute goes on to talk about developing a Research Agenda as well, the types of research that might address each priority, the relative value, and other factors that the Board determines as appropriate.

So, this diagram on the slide actually describes some inputs into Versions 1.0 for the National Priorities and the Research Agenda, so those consist of environmental scans, stakeholder input through a variety of vehicles, forthcoming coming surveys, focus groups, public comment, meetings, and so forth, the Pilot Project

applications themselves, the statute, of course, very exciting for us particularly for Version 1.1 or 2.0, depending how we're numbering. The Methodology Committee has some work in process right now, which I think will be highly informative in addition to these other inputs for updating and refining these in the future, the PCORI Board of Governors and the Pilot Projects themselves, not to be confused with the applications, since there was quite an enthusiastic response.

If there's one point I would like to leave you with, it's that because we've got at least a 10-year timeframe from our work together with the Patient-Centered Outcomes Research Institute, Versions 1.0 will be updated periodically over time. It is not hard to find other groups who've developed national priorities, for quality, for research, and so forth, in the past.

This will really set the stage for doing so on a regular longitudinal basis, where it starts to get truly interesting.

So, again, this diagram on this slide

talks about the relationship between the Priorities feeding into a Research Agenda in turn informing individual PCORI funding announcements and feedback from those and the response to those announcements in turn, in addition to other inputs, informing future iterations of the National Priorities in addition to the specific factors I mentioned a moment ago.

So, we're on a journey. Again, for those of you on the phone, this is really a fabulous photo of the yellow line down a highway, so wherever this came from I totally love it, but that is to say that the process is at least as important as the final output or outcome and it's going to be an ongoing iterative process.

So, this next slide shows a timeline for the development of National Priorities. Again, this was set in motion by the statute. You can see a couple of bright red bars that go across both for the Priorities and the Agenda, and that is ongoing dialogue with stakeholders. For example, later today there will be a public session with local

stakeholders as there has been at almost every single board meeting.

Work began in earnest in October of 2011 and I'll tell you more about the specific steps there. The public comment process, as Rick noted, will be launched this Monday, January 23rd.

The Priorities and the Research Agenda began at the same time together.

So, turning, then, to the National Priorities, after that preamble. So, the first thing we did was to actually look at initial stakeholder feedback through a variety of mechanisms, and what was very interesting was that one consistent theme that emerged was feedback telling us not to reinvent the wheel, we weren't discovering the planet for the first time, that we could stand on the shoulders of others, build on previous work, and so forth.

We then reviewed prior frameworks for comparative effectiveness, including the Institute of Medicine's study, which Dr. Selby was a member of that committee as was Dr. Washington, I believe.

The Federal Council for Comparative Clinical Effectiveness Research, which, by the way, was sunsetted with the passage of the Affordable Care Act, if any of you thought they might be emerging again. The National Priorities partners a national collaboration of numerous quality improvement organizations that sets very broad priorities for improving quality of care, the National Quality Forum, and so forth.

We then moved to identify broad priorities from prior frameworks, as well as the statutory criteria set forth in the legislation for PCORI and developed a framework to be used for refining priorities and as well as shaping the Research Agenda and funding announcements.

So, this very, very busy table more or less depicts the points made in the prior slide. Down the left hand side you have a number, although not an exhaustive list, of the various frameworks that have been developed before. Across the top or the columns are different types of disease, from preventive care to acute, chronic illness,

palliative care, care coordination, patient engagement, safety, health IT, to improve patient experience, and the impact of new technology.

So, there's a variety of check boxes to give people a sense of how other frameworks have used these various criteria, and this was all just part of our environmental scan.

Now, this slide actually shows you that not only do we need to be attentive to the criteria in the statute, we also want to be very attentive to the definition of patient-centered outcomes research, for which we've gotten quite a bit of stakeholder feedback, which has been very, very helpful.

So, taking the frequently cited priority areas on the left hand side of the slide, again, from prevention and screening, different stages of disease and illness, different facets of healthcare such as care coordination, patient and caregiver engagement in their care, safety, and so forth, and then filtering that through -- with the assistance of the statutory criteria and the definition of

PCOR. The first draft National Priorities are shown on the right hand slide, and I think the next slide has them -- nope. I'll back up for just a moment -- so there are five areas that are broad by design.

I did, early in this process, probably in the middle of the environmental scan, actually Google the term Research Agenda and National Priorities and so forth, and what is most striking is how many there are and also how diverse they are. Some are extremely specific and focused, many, essentially, create a path for a better life by the time you get done. They're very, very expansive.

So, the first category listed here is the comparative assessment of options for prevention, diagnosis, and treatment. The second is improving healthcare systems. Now, PCORI is not running healthcare systems or paying for healthcare, but it became very clear over the past several years from the Institute of Medicine Committee, the Federal Council, and so forth, that there was a strong and

consistent belief that various aspects of how healthcare is delivered can have a big impact on patients' outcomes.

The third area is communication and dissemination research. The fourth is addressing disparities. And the fifth is accelerating the conduct of patient-centered outcomes research and methodological research.

So, trying to put this in something closer to English, the first category, comparative assessment of options, compares the effectiveness and safety of alternative, preventive diagnostic and treatment options.

For improving healthcare systems, we needed a few more words. This is comparing healthcare system level approaches to improved access, supporting patient self-care, innovative use of health information technology, coordinating care for complex conditions, and deploying workforce effectively, everything that goes into making healthcare actually work for patients and deliver care that is, indeed, focused around

patient's needs.

The third area, communication and dissemination research will compare approaches to providing comparative effectiveness research information and supporting shared decision making between patients and their providers.

The fourth area, addressing disparities, involves identifying potential differences in treatment effectiveness or preferred clinical outcomes across patient populations and the healthcare required to achieve the best possible outcomes in each population.

And then the fifth category, accelerating PCOR and methodological research, speaks to improving the nation's capacity to conduct patient-centered outcomes research by building data infrastructure, improving analytic methods, training researchers, patients, and other stakeholders.

So, it is, by design, a very, very broad set of priorities.

These were, as I said, developed over

roughly a five month period, build on and were informed by prior prioritization efforts. Stakeholder input has been incorporated along the way, so for those of you who have had the opportunity to make your voices heard, whether that's through a published article, direct input to PCORI, giving our esteemed Executive Director a hard time when he presented this in earlier iterations, thank you very much.

And we believe the first draft is now ready for public comment, after which this initial draft can be modified based on public comment or thereafter through a transparent process of stakeholder engagement.

So, we're very, very excited about this, excited about the opportunity for public input. And now I'm going to turn the clicker and the baton over to Dr. Krumholz.

CHAIRMAN WASHINGTON: Before you proceed, could you turn back to the slide where you showed the bridge between what's been done -- I mean, what the emerging themes were from the original

synthesis and -- right.

DR. CLANCY: This. Yes.

CHAIRMAN WASHINGTON: And I'd just like, because you were moving through it quite fluidly, but have us reflect on this if there are any questions from any Board Members?

DR. CLANCY: Harlan?

DR. WEISMAN: Carolyn, in the list on the left, are you -- the priorities that are the draft priorities from PCORI today, do you believe all of those are subsumed under those five categories? I guess that's one question. And the other is, and maybe we'll hear more from Harlan Krumholz, I'm assuming -- some of these are -- can be overlapping priorities, I guess. You could imagine that, for example, addressing disparities could be part of a healthcare systems question or part of a comparative assessment, too. Is that how you were conceiving of this?

DR. CLANCY: First, we do believe that we have addressed all of the frequently cited priority areas, although, frankly, looking forward to input

from this Board as well as from a variety of stakeholders would be very, very helpful.

To some extent, the draft five priorities do have some overlapping features, there's no question about that, and at the same time reflect some strategic decisions about the need for emphasis.

So, for example, the topic of disparities is fairly prominent if you read the section of the statute and for a variety of reasons we thought that deserved very specific focus, but that is one of the tricky parts.

I will also say that the last one about accelerating patient-centered outcomes research and methodological research is one area that other efforts have really been all over the map on. Sometimes they'll talk about the topical focus areas and then, by the way, have these extra chapters at the end, a variety of different approaches to that. So, I don't think there's a perfect approach.

CHAIRMAN WASHINGTON: Sharon.

DR. LEVINE: Carolyn, just to clarify, is it fair to say that, at least for the first four boxes, comparative assessment could be applied to each of those boxes? So, comparative assessment of the likelihood of different delivery system designs to deliver optimal health or access or something?

DR. CLANCY: Yes, and I think part of the reason that second category needs so many words is that we don't have a standard terminology or taxonomy for, for example, care management.

DR. LEVINE: Right.

DR. CLANCY: What it means at Kaiser is different than what it means in the rest of the fragmented system, and so forth.

DR. LEVINE: So, and we can do that with communication and dissemination research and with addressing disparities also.

DR. CLANCY: Yes, and one point, I'm not sure I'd leave the fifth box, the infrastructure and data and so forth, out of it, because I think there's a great deal to learn and if there's one theme that comes out of a lot of our Board

discussions, it is the notion of being a learning organization and I don't think we know the best way to do that.

I should have asked Dr. Epstein if he wanted to chime in here.

DR. EPSTEIN: No.

DR. CLANCY: No, thank you. Okay.

DR. LEVINE: So, then a suggestion, which would be to somehow indicate that on the slide because I think because comparative assessment is so much a fundamental notion around this, being clear that it applies to all five boxes in some way, I think will help people understand the pathway.

DR. CLANCY: That's a great idea.

DR. EPSTEIN: That's a great idea. I'm surprised nobody's brought that up in 31 presentations of this.

CHAIRMAN WASHINGTON: Okay. Gray, first.

DR. NORQUIST: So, I'm sitting here and I'm thinking, so now we've made this public and we're asking for feedback from the public, so one

of the things that I think we need to be careful about, and I've said this before, is that when you say PCORI National Draft Priorities, I think one of the things we also need to think about is that you may want to think about this as priorities on patient-centered outcomes research.

And then the question becomes, what is the weight of each of these and what is the weight for PCORI itself? You see what I mean? So, we might like to have feedback in the direction of, are these appropriate priorities for patient-centered outcomes research? And then when you think about what we as a group do, because we don't have all the money in the world and there are others who are funding some of these areas, I dare say that AHRQ would fund a number of these boxes here. At some point I think that's the kind of public feedback I'd also like to hear is, what's the weighting of these, because as we get a number of grants and stuff, we're going to have to make a decision over how many of what to put in each box, do you know what I mean? And then, what is our role as PCORI

compared to the broader context of patient-centered outcomes research.

DR. CLANCY: Dr. Selby.

DR. SELBY: Yes. In response to Gray's comment I just want to let those listening outside of Jacksonville as well as those in the room know that in the Research Agenda, in the Draft Research Agenda that will be posted, there is an initial proposal precisely as to the proportions that we thought made sense to allocate to each of these priorities, but there's also, in the forum that will be on our website for public comment, there's actually a request for input on that.

So, we will be looking for input on the appropriate apportioning of PCORI resources across those five priorities.

DR. CLANCY: And, Gray, just to your point about are these for PCORI or are they national. It seems to me that that's a point that most prior efforts have glossed right by, some of it comes back to real time, ongoing, sort of, strategic planning for PCORI itself, but I think at the

moment we're leaving it somewhat high level, that it is for the field of patient-centered outcomes research and leaving it at that.

CHAIRMAN WASHINGTON: Harlan.

DR. WEISMAN: Yeah, I think it was on addressing disparities, the detailed slide that you had that follows this one. Next one. There was one where you expanded out, I guess that was it.

DR. CLANCY: Yeah.

DR. WEISMAN: Okay, so it says across patient populations, and I certainly understand that, but one of the things that we've talked often about as PCORI is the patient-centeredness and, in fact, in the definition of PCOR, the top part of it, which was directed towards patients, was to really provide answers that are very specific to an individual rather than to a population or very specific to the -- as specific as possible -- to people like me and the available options that I have and what I can expect based on my particular medical conditions and medications, geography, preferences.

And I know that's implicit, just because we carry it around, but in this presentation the only word that had to do whether this is population based or individual based is there. We never really say that we want the research to be aimed to help people answer those questions. And I guess that's an underlying criterion, but -- and maybe it was before when we overtly had the criteria on the slide that you could see it, but it seems to be missing for me.

DR. CLANCY: Well, I guess to some extent it speaks to -- I love the phrase "people like me" -- but "people like me" can have a whole lot of different meanings, and if there's one thing we've learned painfully and far too often, and AHRQ reports on every year to the Congress, it's how pervasive differences in care are that seem to be associated with individual characteristics, whether that's racial or ethnic background, income, education is a huge predictor, and so forth.

So, to some extent "people like me" is about factors that you don't control -- what

disease you have, what background you're from, and so forth -- as well as things that you choose. If you have suggested wording tweaks, I'll speak for Ernie too and say that we'd be all ears.

CHAIRMAN WASHINGTON: Okay. Sharon-Lise.

MS. NORMAND: Hi. Sharon-Lise Normand, Methodology Committee.

I just wanted to follow up on Dr. Weisman's comment. I'd like to emphasize, at least from the Methodology Committee's point of view, that that last box is really -- our goal is to provide methodology to be able to answer the questions that you're interested in. So, for the "people like me" question. So, while the fifth box is -- it is there, I would emphasize that -- I would argue, and I think Dr. Gabriel would agree with me, that that box is really meant to facilitate the scientific rigor of the prior four boxes.

So, any questions you have with regard to "people like me", you know, we think of that as treatment heterogeneity, how do we estimate that,

how do we conduct studies to be able to do that, that's, in my mind, subsumed in that fifth box.

DR. CLANCY: I think that's well said. And if you have a visual for that, I think that -- I'm serious -- like rocket fuel or something.

VICE CHAIRMAN LIPSTEIN: Can we go back one, Carolyn? So, for my colleagues who are listening in on, hopefully, the web who either represent healthcare systems or hospitals, that second box, and the descriptions that go along with it, are really important because I know that many people from that domain are very much interested in how you can improve outcomes through the use of either information technology, manpower deployment, care coordination approaches, or self-care.

But my question, I guess, for the Board, and I want to just be clear about this, so if someone wants to conduct research that shows that certain approaches to care coordination approves outcomes, they don't necessarily have to compare outcomes with somebody who undergoes care coordination versus somebody who doesn't. So,

there doesn't have to necessarily be a control group in every research study, because PCORI's interested in helping people to see what actually does improve outcomes, and you can do that, I think, by evaluating what you're doing, not necessarily having to compare it or contrast it with doing nothing. Am I correct in that assumption, Dr. Selby?

DR. SELBY: Absolutely.

VICE CHAIRMAN LIPSTEIN: I'm not the researcher here, but --

DR. SELBY: [Off microphone.]

VICE CHAIRMAN LIPSTEIN: Right. In other words, so, PCORI's agenda will include evaluating approaches to improving healthcare systems performance that doesn't necessarily have a control group. Did I say that right?

CHAIRMAN WASHINGTON: Except for what you just described, you did have a control group, it's just that it was the previous experience as a cohort.

DR. SELBY: Yeah, or usual care or

existing --

CHAIRMAN WASHINGTON: So, it still is a comparison.

VICE CHAIRMAN LIPSTEIN: Thank you, but new interventions versus previous interventions, if they result in improvement, we want to learn about those things.

DR. SELBY: I think the comparisons need to be practical, they need to answer a question that a patient and a clinician need to know the answer to. Sometimes the most practical question is, is doing something better than doing nothing, or is doing something new better than doing what we've been doing for years.

So, it's not always, you know, one very specific treatment versus another very specific treatment. That's my take.

DR. CLANCY: In your example, Steve, I would guess that we actually think we all want to coordinate care, and sometimes we think we're doing it, it just doesn't always work out that way.

VICE CHAIRMAN LIPSTEIN: Right, and I

guess one of the things we're hoping that we get by having this particular priority is that we'll figure out which approaches to coordination actually produce the best outcomes.

CHAIRMAN WASHINGTON: Absolutely. Sharon.

DR. LEVINE: And another great example is what we call team-based care. You bring a group of people together and give them orders and in some settings that's a team.

But my comment, actually, I wanted to go back to what Sharon-Lise said -- I've worked in those environments -- as a clinician, there is a big difference between people like me and me. So, when we talk about people like me, we really are talking about a population of individuals who share some characteristics with me, but when I'm in an exam room with a patient, the "people like me" isn't what I'm interested -- in what my patient is interested in, it's, okay, but what about me.

And, so, I think we need to be careful about understanding that population and subpopulation data is the beginning point, not the

end point, of considerations in terms of impact.

CHAIRMAN WASHINGTON: Well stated. Okay, I have Gray.

DR. NORQUIST: I just had a clarification. On the fifth box, I agree with Sharon-Lise, it's about -- but it's not just that, about -- it's also about infrastructure, right. So, I mean, we don't want to miss that point, that it's also about building an infrastructure of potentially -- you know, groups who might do research and stuff like that, right?

DR. CLANCY: Mm-hmm.

CHAIRMAN WASHINGTON: All right, I have Debra and then Harlan.

MS. BARKSDALE: I have a question about the previous slide, I believe, where you talk about -- yes -- assessment options for prevention. I'm really interested in how the group envisions prevention. Is it more in line with health promotion, disease prevention, or more in line with comparing one, for example, drug option for obesity against another? Or all of the above?

DR. CLANCY: So, my own personal opinion is, yes, it's probably about both of those things. That's an area where I would hope we would get some very rich and thoughtful comment from our colleagues in public health because, arguably, there are some preventive interventions that are much, much better delivered at a community or a population level than one-on-one in an office.

I suspect that probably the true magic is when those two are aligned, but it is an issue that we struggle with a lot under the Recovery Act investments.

CHAIRMAN WASHINGTON: Harlan K. and then Harlan W.

DR. KRUMHOLZ: I just wanted to highlight one opportunity that exists within these priorities that centers on the interface of the comments that Sharon-Lise made and what Sharon said. Sharon-Lise was talking about how you can understand, to the extent possible, the heterogeneity of treatment, why people respond differently, what are the ways in which we can tailor our information for the

needs of individuals.

Sharon said, well, what about the person sitting in front of you who's never really exactly the same because you have to draw inferences from groups, ultimately, to try to make some of these. And that's why I think in these priorities you also see this emphasis on research into communication, because we are always dealing with issues of uncertainty with the person in front, and the decisions you're making are, you're taking your best hunch that this is the best thing for you given what your values and preferences and goals are.

And, so taking the research product and then figuring out, what's the best way to explain that to people, what's the best way to interact with the me, the patient sitting in front of you? Because we often, most often, aren't able to tell -- there's no answer, there's no verdict here. There are options available to them, which have tradeoffs, and particularly with patients with complex disease.

And the question sort of is how -- I think what's here is our investment in not only generating the knowledge, but I think that what we were trying to do, and for those who are listening, is also invest in the research that's going to help us be most effective in helping practitioners and patients have the conversations that they need to take full advantage of the products of the research that are put out.

And I'm really excited about that because often that kind of research is very difficult to fund. It's at the interface of medical science and a lot of social science, communication science, and for us to be able to bring those groups together that ordinarily aren't talking and for them to be attuned to the needs of specific populations, cultural differences, literacy differences, and so forth, is a rich area for us to invest in, which I believe can bring a return in elevating the quality of decision making because of the way in which we're concentrating both on the production of new knowledge and new knowledge that's going to help us

leverage that new knowledge for the benefit of individuals.

CHAIRMAN WASHINGTON: Harlan.

DR. WEISMAN: Debra's question triggered a question in my mind, and I'm sure it's in there, maybe even in the first one, and I really like the language of the comparative assessment of options as opposed -- for prevention, diagnosis and treatment, because a lot of people, when they think of comparative effectiveness look at it as treatment A versus treatment B. But Gray, among other Board Members, has talked in a very compelling way about behavior and the role of behavior in prevention as well as in treatment.

And it is -- I would venture that in many of the chronic diseases that the United States deals with, if we could get people to do, or if people would choose to do the right things, they could prevent a lot of the diseases that may afflict them, or if, once they have the disease, if they would be compliant on the regimens that were prescribed for them, they would do better,

independent of whether it was A or B. In fact, A or B may play a lesser role than the very fact of this issue of compliance.

And I'm assuming that we're putting that kind of thinking in terms of impact under what might be comparative assessment or healthcare system approach -- I'm not sure where exactly those notions fall but, you know, I don't want to lose that and, you know, Gray has also pointed out in terms of disparity, that what you do in terms of modifying behavior can be very different depending on peoples' socioeconomic status or level of education or where they live. Very complex, but might have a huge impact on improvement if we could facilitate that type of research.

DR. CLANCY: Well, I would say, A, it's unbelievably important and, B, I think it crosscuts a number of these boxes. What can healthcare systems do to help facilitate that? What are the best kinds of strategies? For example, if treatment A is a pill you take once a month versus something that you take every three hours, that's

going to be easier or harder for some people to comply with the latter, and so forth.

So, I think it's there, Harlan, but I guess you may have a few minutes before show time, and we will do a final read before this all gets posted and put out for public comment on Monday. But please remind us in the event that it still feels too implicit after we've gotten all the public feedback.

CHAIRMAN WASHINGTON: Bob, please.

DR. ZWOLAK: Carolyn, that was very nice. As I read through the NORC assessment of the patient interaction groups and the focus groups, I was almost embarrassed; those of us who see patients everyday are thought to be pretty poor by our patients at communicating with them about their options. I almost thought to myself, gee, we need to put a better course in medical school about how actually to sit in front of a patient and talk to them and ask them questions and communicate back to them.

And, so, based on the feedback that was in

that NORC report, and so my question to you is, where are we going to address that? You have a communications bullet and a dissemination bullet, but is that more toward science or is there a place where we're going to focus or potentially fund research that would focus on how to make us, as doctors, better communicators with our patients?

DR. CLANCY: I would guess that that fits squarely within communications and dissemination research. It's not just about taking the finished products of research and figuring out how do we get it out there.

And, frankly, I'd argue that if we knew a really easy way to do this that was wildly effective and we weren't using it, that would be negligence. I think we don't actually know. I think we've got no shortage of aspirations to do it well among clinicians, but it doesn't always bring the ball home.

CHAIRMAN WASHINGTON: Okay. Bob it is reassuring to know that since you and I were in medical school there's been, I think, more

effective attempt to get medical students to communicate with patients? It's a big issue. And, so, I expect that we will see submissions related to more effective communications. And AHRQ has funded research in this arena over the years. We had a program project entitled *Promoting and Effecting Better Communication*.

Okay, again, for those who are listening and those present, we're asking you to comment on everything that's here, both in terms of the framework as well as the detailed information we're giving you related to these priority -- draft priority areas, and know that we will appreciate you taking the time and we will certainly be listening and incorporating your feedback into the next version of the National Priorities, which in turn will guide the development of our initial Research Agenda.

That was just on the National Priorities and we're going to now transition into a discussion about this initial Research Agenda.

DR. KRUMHOLZ: Thank you, Gene. This is

Harlan Krumholz and I speak on behalf of my co-chair, Leah Hole-Curry, and the entire PDC and the Board, as we present this piece.

I want to go back, start this presentation, just for framing, at the -- I'm sorry -- here, because I think it's important for people to realize as we present this agenda, I think there's been terrific progress, and that progress has come as a result of a lot of help. And what we've done from the very outset, with our eyes clearly focused on the need for us to develop these Priorities and Agenda, was toward figuring out how could we best take advantage of all the resources in the environment and synthesize, coalesce the best ideas and to bring us forward.

You know, when we assembled, in a statute, put together 21 people with very different perspectives and the idea of patient-centered outcomes research is not one that is a discipline where there are clear boundaries or clear definitions, even, that exist.

So, our charge really was to forge a

common understanding of what were the boundaries, and they may be porous boundaries in the sense there may be overlap with other fields, but what was the area that we were going to target? What is it, really, that we should focus our attention on? And how can we best bring the promise of the legislation forward?

And I want to emphasize, within that legislation there is this piece that really suggests that we should be thinking about return on investment. This is ultimately an investment by the American people, it's an investment by our government in an independent group, and we want to be able to derive marked benefits for patients and the public, and in order to do that, we need to listen carefully to those out in our environment, and we are really vehicles for the kind of, I think, hopes and aspirations that exist throughout the community.

This slide, which Carolyn showed, gives some sense of what we've been doing since the very outset. The agenda that we are going to talk about

today, importantly contributed to by a very diverse and talented Board and a very diverse and talented Methodology Committee, who played a central role in this, and we looked to the statute and have each read it probably hundreds of times as we've thought -- we want to make sure that we're fully aligned with it.

We implemented a Pilot Project process where we were interested in getting some funds out the door and to stimulate some creative projects, but also as a mechanism for us to learn, build a foundation, about how we were going to pursue these priorities and the agenda, and we've already been able to take advantage of them by reading these applications, by understanding the topic areas that they cover, many people on the Board spending many hours really not only working to move them forward in the process of our ultimate goal of funding some of them, but getting as much knowledge as we can out of the applications themselves.

Stakeholder input has been a piece. Every one of these meetings has had stakeholder input.

Many of us have presented to stakeholders. Joe, since he's gotten here, has spent a large amount of his time talking to groups, and we -- the website is open, people have written articles where they're almost open letters to us that have appeared in various journals, blog posts.

We survey the entire environment and people should know we are listening carefully. We circulate among the Board the information that comes out every week so that we can see what people are saying.

So, there are many mechanisms by which we are trying to capture the tenor, the sense, the feelings that exist throughout the country, not to pay attention to any specific group, but to be open to the wide array of input that we're getting from sources, and then commissioning environmental scans and landscape reviews so that we can, in a systematic way, figure out what actually has been done, what can we build on, how can we ensure that we're not redundant, where is that sweet spot where we can put our investment where it will make the

biggest difference, how can we best fulfill our mission.

And so I think this is an important thing for anyone who's listening and for the public to understand, that we have this great orientation outward, that we were necessarily built as 21 people who represented various different constituencies, there is a diverse set of perspectives on this Board, but our focus has been to be vehicles to bring in from the outside, not to feel in any way that we're sequestered or that we are trying to build something internally that doesn't reflect the kind of input that we're getting.

Second important point, I think, to make is that the slide that Carolyn showed about the road, people need to understand that our view of this is that we're on a journey, and, in fact, it's a developmental journey. I mean, we started as a complete new organization, independent organization, taking this very seriously. And in the course of this we've tried to coalesce views,

we're trying to bring ourselves to the point of consensus that we're ready to go out for public comment about these Priorities and Agenda. No one on this Board believes that this is a destination, no one on the Board believes that this is a final version, in the sense we have a lifespan as an organization and we will continue to learn, we will continue to iterate, we will continue to refine.

We believe that at this point in our development, this represents a big advance. It's a lot of progress in terms of defining those areas that we're most interested in, that we think the nation most needs based on the input that we have received, based on everything that we have heard, everything we have read, everything we have listened to, this is a distillation of that to say, here's where we are now as an organization -- and the feeling of a sense of urgency that we need to move, but we recognize that over time this is going to be refined, changed, the specificity, the targets may evolve, and this is going to evolve how? Based on what we hear, based on the input,

based on the wisdom out in the communities, and being distilled through those individuals on this Board who -- and when I say Board, by the way, I always mean Methodology Committee with the Board. I consider us hand-in-hand and I think every member of this Board does too, that there is a sense that we're all in this together and through those individuals on the Methodology Committee and on the Board, we are seeking to bring forth the very best ideas, to bring forth the very best in the agenda.

This other slide that Carolyn showed, again I want to emphasize, because it has equal relevance and importance in discussing the Agenda as it does for the Priorities. It shows, for those of you listening, really three basic dimensions of our work in trying to get to a funding announcement, and it starts with National Priorities. Again, a statutory requirement for us to develop and get public comment on, and we're in the course now of getting ready to go out for public comment in the Priorities, and those Priorities are already built on stakeholder input,

a lot of stakeholder input, but even with that we are going to go out for formal public comment.

There is then an agenda, which is a further distillation of the priorities, a little more specific, more directional, helping to provide greater guidance and guideposts to help us move forward to the point where we're going to ask for proposals. And this, again, still is not at the point of specificity that you would see in a funding announcement, but it's a further maturation of a vision that sits in the Priorities as a description of where we are going.

And, again, what you'll see as we describe this is that the specificity that currently exists in the Agenda, and there is a fair amount of specificity, but it's around the types of questions that we want to ask, it's about the types of knowledge that we value, it's where we see our investments going for now. And on the right hand side of this, and same for the Priorities as for the Agenda, the inputs continue to be the statute, the Board, the Methodology Committee, and the scans

and all the same things that you saw on the prior slide, which is going to lead us -- and the pilot grants -- it's going to lead us to our program announcements where we're going to come out and announce and say, hey, we're ready to start our program.

Now, we have felt this sense of urgency, which is why we're coming out with Priorities and Agenda at the same time. All things being equal, with the luxury of time and if we had healthcare problems that didn't demand our immediate attention, we might put them out sequentially, we might say we've got years to do this, but I think everyone on this Board feels it is time for us to move, to put these dollars to use, and to produce knowledge that patients can use. And in doing that, at this point, we feel it's wise to put out both the Priorities and the Agenda together because that gets us to the funding announcements faster, and both have already been informed by stakeholder input, so they haven't been developed in isolation.

But in the future we may get to a point

where sequential is we iterate these. We'll see, we'll see where we are and how this goes, but we're also going to be listening very carefully in this comment period, which will bring us to the program announcements, which will have a greater degree of specificity, will tell people with some greater detail what exactly are we looking for. There still may be some latitude here because our emphasis may be thematic more than condition-based, it may be in areas of greatest need, but without specifying that it's in a very narrow area of all of healthcare, but these are points of discussion and ones that we need to decide on.

There may be some on the Board who will advocate for us to narrow, there may be some who will advocate for us to see more about what teams coalesce and what opportunities are presented to us, and we are in the active part of both listening to our communities, the stakeholder input, the public comment, and discussions on the Board to get to the point of the program announcements and we're trying to move with all deliberate speed.

VICE CHAIRMAN LIPSTEIN: Harlan, can you pause for just a minute, breathe, and let's let the Board comment on this particular issue, because this is about process, and want to make sure that the Board is both comfortable with the iterative feedback process, as you've described it, and if there are any ways to make it even better than what you've laid out here, so Gene if it would be okay, I thought it would be good just to take a pause in the presentation here to talk just about the process of procuring stakeholder feedback and how that would lead to greater specificity of the research agenda.

CHAIRMAN WASHINGTON: Steve, I'll start with a comment based on a statement that Harlan just made. While at least I envision that this would be an iterative process, both involving the National Priorities as well as Research Agenda, I guess I saw the National Priorities of being a sort of higher order and so there would be a slightly higher threshold in terms of changing that versus the Research Agenda is designed to be changed on an

ongoing basis.

And so the iterative process, while at the end of the day could longer term lead to some reordering of the National Priorities, the real focus over the shorter term would be in updating and revising the Research Agenda. Is that a correct interpretation?

DR. KRUMHOLZ: I think that's an excellent point, Gene, and I think that does reflect the sense of the Board. I think the Board would say, we would never say anything is closed. If there was a compelling reason, but it would have to be, as you suggest, a very compelling reason to suggest we're going to change the Priorities, but whereas the Agenda, we expect to iterate more often.

CHAIRMAN WASHINGTON: Again, that's change the Agenda after we have established it, which is not established at this point, this is still draft and so we're still open to it, but we want to just be clear about intent.

VICE CHAIRMAN LIPSTEIN: I think there was, in Harlan's presentation of this, one of the

things that came to mind was, you know, whether you get two bites at the apple. So, we go through this public comment period, people will, you know, weigh in with what they think some of our Priorities should be, some of our Research Agenda should be, and that will add specificity.

But then when we refine it and redo it with all the benefit of that public input, there will probably be stakeholders who will say, now I want to see it again, so I can say, yes, do that, no, don't do that.

And so what I want to encourage people who are listening who are stakeholders is to take this opportunity in this first round of public comment not only to tell us what you would like to see in the Agenda, but what you might like to not see in the Agenda.

So, if there are specific areas that you don't think should be in the purview of PCORI or in this Research Agenda, you need to submit those as part of this public comment period, and then to keep in mind that this won't be our only Research

Agenda, that there will be multiple bites at this apple over the life of PCORI, but certainly over the next several funding cycles, and with each one of those agenda development processes, we will be asking for stakeholder input and comment.

And, so, there are multiple bites at the apple here.

CHAIRMAN WASHINGTON: Steve, you're running this session for now. Okay.

DR. WEISMAN: Harlan, I was wondering whether you could comment on how we go -- we've been detailed about how we got where we are and that the Research Agenda is going to drive us to the actual funding proposals, but how that process actually lays out. We're going to get feedback and then we're going to consider it and then there may be some revision and then there's going to be RFAs. Can you talk about how people who were out there wondering what this is really going to look like -- is there a way we can -- we'll get to a point where we can explain it and they have a change, what you're calling a bite of the apple, not at this

bigger thing, but also at the littler part? In other words, they have a better understanding of what a request for funding might look like. Like, what kind of area or what we're going to emphasize and have at least some comfort around the process we're going to follow to get to those requests for funding.

DR. KRUMHOLZ: Do you want to take it Joe? Or I can.

DR. SELBY: I will. I just want to, you know, draw attention to two periods of time, one is the period of time that goes from next Monday, I believe, to mid-March, and that's the public comment period, and in addition, you know, we obviously will have the web-based collection of information, we'll have ongoing occasions where we meet with stakeholder groups, but you'll also hear tomorrow about, if not this afternoon, I think most of its going to be discussed tomorrow, the idea of a national forum, a public meeting that will be webcast in late February, February 27th, where we will be inviting people to present prepared

comments, brief comments, but prepared comments, and expect to have, you know, numerous additional comments through that webcast forum.

So, that's period one, and we'll also hear tomorrow about how we're going to digest this information and get it into the final version that we then -- that he Board then has to approve before we can begin rolling out funding announcements.

But that's really only the beginning, as Steve said. The legislation is replete with advice to convene fora, to convene advisory panels, to convene workgroups, multi-stakeholder workgroups, and that's what we -- we now have the staff. You just met some of the staff who are able to do that.

2012 will be a year of engagement and I think it's very reasonable to anticipate that that engagement is going to help us focus on, as Harlan says, funding announcements that are more specific. I think you can anticipate seeing some broad funding announcements and then some more specific funding announcements as we, together with stakeholders in a deliberative process -- this

doesn't happen overnight, it doesn't typically happen at one meeting, it's a complicated process, we need our Methodology Committee to even just help us think through some of the methods of getting from all the possible questions we could ask to the ones that make the most sense, that meet our criteria the best, that, as Harlan says, that the research that returns the greatest bang for the buck really provides information patients and clinicians need.

So, I think it's fair to predict that over time, through this process of ongoing, deliberative engagement, you will see more specific -- and by specific I don't necessarily mean honing in on disease, but I mean honing in in one way or another on narrower questions that are important to this country.

VICE CHAIRMAN LIPSTEIN: Other comments about process before we move on with the presentation of the Agenda?

DR. GABRIEL: Sherine Gabriel, Methodology Committee. One other area of specificity that will

come in as we roll out these grants programs is that once the Methodology Committee puts out guidance for methodologic standards for how to do A, B, and C, and once those recommendations are, you know, we have public comment and they're approved, then we need to incorporate those into our review process and so that the projects that come in and the projects, certainly, that are funded align with that.

So, that's going to add some further specificity.

VICE CHAIRMAN LIPSTEIN: Sherine's point is really well taken. Those of you listening in who thought you were taking the afternoon off, be sure you stay around for the full afternoon session because you'll hear a lot more about the Methodology Committee and the work that it's been doing in the second segment of this afternoon's agenda.

That was a paid political announcement.

MS. NORMAND: This is Sharon-Lise Normand and I have a comment about process. I think we all

need to introduce ourselves. I think Sherine and I are the only ones that are doing that, so, you just need to remember when you speak to say who you are.

VICE CHAIRMAN LIPSTEIN: Gosh, she's always thinking methodology.

DR. KRUMHOLZ: Harlan Krumholz -- I'm sorry.

DR. ZWOLAK: I was just --

CHAIRMAN WASHINGTON: Name.

DR. ZWOLAK: Bob Zwolak. Sorry, Sherine. I was just going to make a very brief comment that I think all of us have spent a lot of time thinking about the serial process of the Priorities and the Research Agenda in a parallel process and given all the discussion and thoughtful input, I do think it's appropriate at this point to have this parallel process.

There is substantial pressure for us to get off the ground and fund some really important research and this will be, unlike the Hanford Nuclear Plant that I was reading about in the USA News this morning on the airplane where they're

trying to build this plant in Hanford at the same time as they're designing it, ours will be an iterative process and we can, in fact, rethink things as we get more information, more information from the Methodology Committee. I do think that the iterative process works here and that the parallel take off is appropriate given the circumstances.

VICE CHAIRMAN LIPSTEIN: Thank you, Dr. Zwolak. Dr. Krumholz.

DR. KRUMHOLZ: Harlan Krumholz. I just wanted to respond to Harlan Weisman's point too because I do think many people listening may be wondering and I think the idea is that we're trying to get as much input into this process as possible. We recognize that in sifting through that input there may be some that's immediately actionable, very compelling, should actually affect this current iteration of the Priorities and Agenda. There may be others that we can lock in for the next iteration that require more reflection and discussion but are getting us thinking in different

ways so that people shouldn't feel that if it doesn't get into this one that we've lost it.

But an even more important point, I think, is that everyone listening should know that we are committed to transparency and we are already talking about ways that we can make sure that people see, how do we think about these comments that we're getting, how are we going to respond to these comments, how are they influencing our process so people don't have to think they're throwing comments into the abyss and then we're just coming out with a decision, but we are trying to think hard about how we can make this prioritization process clear.

The Methodology Committee is spending a lot of time thinking about what are standards for that. We may or may not even be able to incorporate their new ways of thinking in this iteration, but we will ultimately and this, I think, we may set a new standard for the way in which this occurs in a body like this.

But we are, I think, breaking new ground.

I think there should be no mistake about our commitment to doing that. We don't want this to be a mystery. We were designed in order to have these kind of conversation/dialogue.

The Research Agenda builds on the criteria from the legislation and this next slide just identifies those areas and I just do want to bring out among all of these the notion of this rigorous research methods. We want to do the very best science. We want that science to have consequence, meaning. We want it to change things for the better for people, but we believe that it drives through really great science and, again, that's why the investment in the Methodology Committee, that's why, I think, everyone on the Board recognizes that we are a research institute, it's just that we are a research institute that wants to see our results translated into action and our questions to be guided by the real needs in the community.

This is a slide that we've shown before, which shows the Priorities filtered through the criteria, which leads to the Agenda. And there is,

you will see when this comes out publically, how this is all presented and framed, but we wanted to just highlight a couple of areas in the presentation today.

So, for example, a Research Agenda item from a National Priority. The Priority is comparative assessment of options for the prevention and diagnosis in treatment. Taking into account their criteria and all of the input that we've gotten and the discussions that we've had, we said that we are really interested to compare situations in which the effectiveness of strategies for prevention, treatment, screening, diagnosis or surveillance have not been adequately studied against alternative options where better evidence is needed to support decision-making by patients, caregivers, and healthcare professionals.

Now, note, this doesn't tell you exactly where we want this -- the precise area. What we're saying is we want to be in these high leverage areas for which there is -- are significant gaps in knowledge which have consequence for people. And

note the word compare. Yeah, we are an organization that's interested in comparing options and illuminating tradeoffs and being able to talk about pros and cons for individual patients, generating that kind of evidence which can be helpful in informing decisions.

Another example of this would be as a Research Agenda from a National Priority --

[Microphone feedback.]

DR. KRUMHOLZ: Maybe someone's listening -- we just are getting a little echo.

VICE CHAIRMAN LIPSTEIN: Harlan, keep going.

DR. KRUMHOLZ: Again, this one is about improving healthcare systems and, again, in this scenario, we are interested in a study, for example, that compares alternative system level approaches to supporting and improving patient access to care, receipt of appropriate care, coordination of care across healthcare services, or settings for patients with complex, chronic conditions, or personal decision-making and self-

care.

The point here is that we lack evidence to guide decisions at organizational levels to produce the most effective systems for delivering the kind of care that people need and want. And we are committed to using comparative effectiveness techniques and the very best methodology to try to generate that kind of science, that kind of knowledge, and that sits in this area.

If you look across all of the Priorities, we identify some key points in each of them that can be areas of emphasis, that we believe if knowledge is generated in these areas that there will be substantial gains for individuals and for the healthcare system as a whole.

I want to note, in addition to the two that I mentioned, the focus on communication dissemination research, again, as previously noted, this is about really trying to advance the field in a wide range of areas, both with regard to how we talk with patients, but beyond that, the kind of skills people can acquire and what are the best

ways to do that.

Again, comparing different approaches so that we can generate knowledge that can provide guidance, addressing disparities, very important to every member of this board, that we seek to understand what are effective interventions and strategies that can eliminate disparities.

We've done a lot to describe disparities in the research community. We have not done as well in helping people understand the relative effectiveness of different strategies that can help us make gains in these areas, and not only pay attention to eliminating disparities, but also understanding patient's preferences within different communities and how that's driving differences and what we can do to address it.

And the final one, which is really built off of the good work that the Methodology Committee is doing and their guidance in this area is, how can we invest in research that's going to accelerate patient-centered outcomes research and methodologic research? It is often hard to fund

research which is going to advance our capacity, that is going to help us with the challenges that we have with current data systems and to really achieve our aspirations with regard to knowledge.

So, finally, I just want to give a sense of the select features of the PCORI Research Agenda, and we don't claim that these are exclusively our attributes, but they're ones that we find very important. We want to be sure that our work promotes the best interest of patients and their caregivers and key stakeholders in the implementation settings as partners with explicit roles in the design, governance, review, and dissemination of research.

We are talking about new ways of thinking about research teams, not just about traditional investigators, but investigators tied closely with patients and their caregivers and healthcare providers in sort of a sharing of power and a sharing of perspective and a sharing of expertise that strengthens the end product. We're going to seek to understand the core questions from the

express perspective of patients and their caregivers. When we try to figure out the needs, it's going to be by listening to the communities.

We're going to emphasize open and transparent science that involves participants in decisions about making data available for further study, and not sequestering or keeping data out of the public eye, seeking to ensure that the research produces as much new investigative activity as possible and that the sharing of information and knowledge among diverse groups is required. If you're going to get our money, we're going to want to work with you to make sure that the most is made of the research that's done.

We are committed to a diverse research portfolio with respect to patients, geography, healthcare professional, investigators, and organizations, seeking to catalyze activity across a broad range of patient sites, conditions, and questions, and particularly looking for areas that are less mature with regard to questions, and knowledge, and evidence.

We want to emphasize knowledge that's likely to make a positive difference in the life of patients and their caregivers and is suitable for dissemination application that's scalable.

And we want to emphasize outcomes that are important to patients and their caregivers and are likely to be useful in their decision-making.

We want to emphasize ideas that emerge from the community of patients, caregivers, clinicians and researchers, seeking to listen and learn from the wisdom of those whose lives are most affected by these conditions and who are committed to generating the knowledge that will promote better decisions and outcomes.

We recognize that the wisdom just doesn't reside in this room. Again, we're looking outward for input.

This draft Agenda was developed over a four-month period. As we said, stakeholder input was critical, crucial to what we were presenting and ready for going out for public comment, and we're ready to go. And this will, again -- the

point here is just of emphasis to put on the slide that the modified and expanded through this transparent process of stakeholder engagement.

VICE CHAIRMAN LIPSTEIN: Comments on the Agenda or especially on what Harlan just presented on the essential features of the Research Agenda?

MS. HOLE-CURRY: [Off microphone.]

VICE CHAIRMAN LIPSTEIN: Yes, Leah, do you want to add to what Harlan has presented?

MS. HOLE-CURRY: No, he did a great job.

DR. KRUMHOLZ: I love working with her.

VICE CHAIRMAN LIPSTEIN: Any other comments? Gray? Gray, do you have a comment.

DR. NORQUIST: Yes, I thought he was pointing at somebody else. So, one thing, I can't believe I'm saying this because I don't want Sherine and Sharon-Lise to get on me, but I think we need to be very careful about using this term "rigorous research" when we're trying to engage communities because it's a kind of a threatening term -- I'll be honest with you, I've asked people and they see that as, well, I don't know how to do

that, so therefore I'm not coming in.

So, I think -- I just want to go back to the emphasis that I want to have on the infrastructure development and training and really working with communities with the researchers to be able to carry out some of this stuff, because we'll never get to some of those things that you really want to be key about unless we do that. Not to say we don't want to have good data on which we base our studies, but when we use that and we do this publically, it is -- it does turn a lot of people off because I've heard that from some people who decided not to apply.

And I think we just need to be very clear about what we're talking about here and to emphasize the fact that we understand that and we're going to work to try to build some of this infrastructure also so that's possible.

DR. KRUMHOLZ: Can I just quickly respond? My own personal hope in the way in which I think this has come in is that we're going to bring people together who bring their own expertise, so

we're not expecting people who don't do research to do rigorous research, but we don't expect the investigators to say, I know what it's like to be a patient or I know what it's like to be a member of the community, that in order to do the kind of good work that we're talking about, we need people to bring what they uniquely have and can learn from each other.

But maybe you can help us with better words.

DR. NORQUIST: I think we all mean the -- we all mean the same thing, I just want to be clear that we're always saying this, so that we're very clear that we want to really make this something that encompasses everybody.

MS. NORMAND: Sharon-Lise Normand, Methodology Committee. So, maybe I'm going to say something --

DR. NORQUIST: Wait a minute, I forgot -- Gray Norquist, I forgot to introduce myself.

MS. NORMAND: Yeah. And I'm still Sharon-Lise Normand from the Methodology Committee. But

what I guess -- may say something that is not going to be agreeable by everybody, but I don't think we should shy away from saying rigorous research methods. If that frightens people then maybe we just have to -- we're all on the same page in terms of how we're thinking about things, but we need to use rigorous methods, and I don't think we should back down from using terminology that some perceive as I'm not going to apply if that's the case.

So, I'm sort of a little bit put back on that. I'm not so sure if I'm comfortable saying let's take it out. We're all on the same page, that's fine, but let's not -- let's not, you know, make things so weak that we may have problems down the line. You need to do good science and I think

VICE CHAIRMAN LIPSTEIN: He's still Gray Norquist.

DR. NORQUIST: Yeah, I'm still Gray Norquist --

MS. NORMAND: You won't be still Gray Norquist after I'm done talking. I'm joking, I'm joking.

DR. NORQUIST: No, no, I'm not disagreeing with the fact that you want to do it correct, I think it's the way you have to present your message to people and if you want to engage certain communities, you're going to have to talk the way people want to talk.

So, I'm not disagreeing with you on the level that we want to do things correctly, but it's the way -- if we really want to engage people and bring them in, we've got to understand different communities and talk to them in their language. We cannot use their language and think that they're going to come forward. That's all I'm saying.

VICE CHAIRMAN LIPSTEIN: Hold on. Sharon Levine, then Larry, then Christine, then Arnie, then Gail.

DR. LEVINE: We've talked before -- I mean, Harlan's made the point several times and we've talked before about how do we match up rigor in scientific methodology with community-based good ideas to ensure that the end product meets both the criteria of actually serving the needs of the

community from which the idea came as well as meeting the standards for rigor by which all of our research is going to be judged. And I think it's somehow marrying -- coming up with language that marries those two without compromising on either.

VICE CHAIRMAN LIPSTEIN: Larry?

MR. BECKER: Larry Becker, Board. So, I'm more -- in terms of the rigor, I think about this in terms of the patients, and remember, one of the things we said in our mission and our vision was, trust in integrity, and so there has to be some level of rigor so that the patients, the consumers, the caregivers, even the clinicians who are using whatever we're providing, producing, have a trust and have integrity in what we're doing.

And, so, I think there has to be a balance, but I think that's a critically important thing if we're going to be the provider of certain products to the U.S.

DR. GOERTZ: Christine Goertz. I think it's important to keep in mind that this is not just a language issue. I think we have a

responsibility to create the opportunities where we're -- and, you know, build an environment where we are able to conduct very rigorous research, but making sure that we have the level of input and involvement, Gray, from the people that you're talking about. I'm confident there are ways to do that. There are some models out there, but I think it's something that we really need to be thinking a lot about and striving towards more and more.

DR. EPSTEIN: This is going to follow the crowd, so to speak -- sorry, Arnie Epstein, Board of Governors. I think everybody agrees, and I found myself nodding with what Sharon said, and the truth is, on the other side of the coin, Harlan, whose research is exemplary, has been shameless about saying that orthodox researchers -- I resemble one, he resembles one -- are going to have to find new partnerships with stakeholders, which are going to be new and strange and different and may be uncomfortable. And you've been -- you've made it clear that you have no patients otherwise.

And, so, I want to draw Gray out a little

bit, not in disagreeing, but really asking for your help, which is, I think we want to get there and maybe we have to follow Christine's idea that how do we get there is really the issue, because I think there's no pushing back -- Sharon just said it as baldly, but I agree with what you said.

VICE CHAIRMAN LIPSTEIN: So I've got Gail then Freda then Joe and then Bob. Anybody else? Harlan. Okay. Gail?

MS. HUNT: Yeah, Gail Hunt, on the Board, and maybe, Gray, you wanted to respond to -- you'll do a summing up response?

Okay, I wanted to agree, actually, with like jumping on with everybody else, but really what Gray says really resonates and also what Harlan K. says resonates. We have to have a new way of thinking about how this research is going to be done and it's got to be involving patients and caregivers and other stakeholders, clinicians, as well as the researchers it's always involved before, and I -- not to take away from what Sharon-Lise says, but this is like a new paradigm, I

guess, I think.

DR. HALL: Yes, Freda Lewis-Hall, the Board. I just wanted to underscore -- it almost sounds as though these partnerships are compensatory in some ways and I think they're really complementary. And I wanted to underscore the notion that the Priorities are written in such a way, I believe, the Agenda is written in such a way, that it forces partnerships and collaboration in a way, even between more typical or the usual investigator suspects.

So, I think the platform that's being built really will encourage a different set of collaborations that will deliver results under these headings that have been addressed as opposed to a set of priorities that look very familiar to the typical group of researchers. And I do want to do the play on words to say I think that rigor is an adjective, right, and you just have to be careful that it's not a noun, as in rigor as in mortis.

[Laughter.]

VICE CHAIRMAN LIPSTEIN: Dr. Selby?

DR. SELBY: Just the small additional point that we have taken steps in our review criteria that we've sent to the NIH for their review of our grants, that indeed the study teams be composed very clearly of community and patient and stakeholder participants as well as researchers, so I think that over time we'll have ways to strengthen those kinds of partnerships, but I think just the notion that if you want to apply for PCORI money you've got to demonstrate that in the application is another one of the building blocks we have to ensure that the research teams change --

UNIDENTIFIED: The study section has it.

DR. SELBY: The study section has representatives of more stakeholders than study sections at NIH have ever seen, three per study section, but I think the addition of that criterion is strong evidence that we want a different kind of research.

VICE CHAIRMAN LIPSTEIN: Dr. Zwolak and

Dr. Weisman.

DR. ZWOLAK: Bob Zwolak, Board Member. Thank you. Harlan, I wonder if you might address what you haven't addressed. Many people have asked me questions about disorders and disorder specifics, and I think that some people were expecting that we would publish a list of the top 117 disorders that we wanted to study, and instead we have this very creative conceptual set of research agenda.

You're not necessarily excluding a pill versus another pill for hypertension or a pill versus a surgical treatment for hypertension, as long as they abide by this research, right? You're not excluding anything in terms of disorders?

DR. KRUMHOLZ: Thanks, Bob. Harlan Krumholz. I think that there are two issues here for anyone listening to know, one is that we are going out for public comment and in the course of comment we're going to make it to a funding announcement and in this stage of our development we felt that this is appropriate. And the second

thing is, in part it's because of the feedback we received and the listening we've done to the community and the kind of stakeholder input we've got.

You know, we began to recognize that there are not a finite number of problems in healthcare. I mean, I know it seems remarkable and striking, but the truth is, there are these gaps in knowledge everywhere, and when you start talking to people, almost everyone can identify where they believe are leverage points where we need more information, where we need to be able to help patients make more informed decisions. It's not like we've got 90 percent covered now we're just going to invest in the last 10. It's like 97 percent of medicine -- 99 percent of medicine has got areas that we can provide this kind of information, and as we really start listening to people, there would begin a recognition that if we're truly committed to this idea of a marrying of the question with the team, strong teams that have this sort of a different approach than has ever been tried before, then we

should probably be opportunity-driven and that we should be looking -- we should define the field, the playing field, but then we should see who shows up to play and what they can pitch and how they can convince us that they've really got the team12 that's going to deliver and what they're doing is going to make a difference.

Now, again, I say that with -- this doesn't preclude that we may, over time, get to a point where we identify particular areas. That's going to be also continuing listening, input, discussion, struggle on the Board about where, again, can we get the best return. But I think the reason we are where we are now is because as we listened, we couldn't -- it became obvious to us that what we need to do is define the field of play, where are those areas where we're looking for the best ideas.

But ultimately one of the best ideas might be comparing -- and we like to say strategy rather than drug -- strategy A versus strategy B, and then producing the knowledge so that when patients are

at that fork in the road, they can get information that can help them make the best choices, but we may get down there eventually, but I know many people were expecting us to say, like the IOM, here are our top 100 and here's a list in order, and I think, in fact, it evolved out of a recognition that everyone's got their top 100, it's a different top 100, and really it's these areas, and the winners, I think, are going to be the ones where we can get the teams with the questions that are most likely to produce the knowledge that's going to matter.

VICE CHAIRMAN LIPSTEIN: Let's go to Dr. Weisman and then let's take a time out for a second, do a process check.

DR. WEISMAN: Harlan Weisman, member of the Board. I wanted to take the little debate we had over rigorous methods and maybe even just generalize the issue.

So, we did some focus groups with patients. Some were Spanish speaking, Hispanic, from the Hispanic population, and we conducted

those in Spanish. We had to do it in a language that they would understand. We have to have -- we know what we want, we have principles by which we're going to follow and that we're not going to compromise, but we can't let language get in the way of our ability to do what we want and to engage people.

So, what's ultimately important is that we have people understand and appreciate what we are doing and ask for their participation with us, and that means, often, that we have to meet them where they are rather than expecting that they're going to come to where we are, and that means, without compromising principles or actually what we're going to do or rigor, we have to speak in a language that's understood by the people we're engaging, and I don't think anybody would argue that if you have a Spanish speaking person you're going to speak Spanish to them. If you have people who look at the world differently than the way we look at the world, things that are obvious to us may not be obvious to them but there may be things

that are obvious to them that aren't obvious to us. We have to bring ourselves to them, and I think that's the spirit under which I took Gray's comment. No compromise, but let's speak -- let's understand by speaking the language of the people that we're dealing with and hear them in their language and get them to understand what we're trying to do and therefore be enthusiastic about participating with us.

VICE CHAIRMAN LIPSTEIN: Gray, since you started us on this journey, do you want to summarize --

DR. NORQUIST: No, I think Harlan has said exactly what my point was and I think if we don't do that, we're not going to be doing something different and we're not going to engage the groups that we need to engage, so that's just where I would leave it.

VICE CHAIRMAN LIPSTEIN: Terrific. Harlan and Leah, incredible amount of work on the Research Agenda, but since our public comment period starts in about seven minutes what we want to end this

section of our agenda with is kind of a consensus among the Board to move forward with the public release of our Priorities and our Agenda for public comment.

Dr. Washington, I don't know if you wanted to vote on that or how you wanted to handle that consensus position. Did you want to have -- just as long as everybody's on board, you're on board?

CHAIRMAN WASHINGTON: [Off microphone.]

DR. NORQUIST: Did you want to make a motion?

CHAIRMAN WASHINGTON: I'm not sure we need a motion. I mean, we've not had motions, generally, on an issue at this level. Remember, we're not voting on the actual Priorities and at this point, I mean, I think you have learned -- I've learned to read you and I'm picking up consensus, but if anyone has comments to the contrary, then I would ask you to speak up at this point.

Thanks, Steve.

VICE CHAIRMAN LIPSTEIN: Gray.

DR. NORQUIST: I would just ask if I were out there on a call, what exactly am I supposed to do if I want to make comments? Do I send comments to the PCORI.org? Do I send it -- I mean, what would be --

VICE CHAIRMAN LIPSTEIN: That's a very good point. Dr. Selby, when we release our Priorities and our Agenda for public comment, can you just briefly describe what will happen next.

DR. SELBY: Yeah, let me preface it by saying that we'll describe this in more detail tomorrow, tomorrow morning, but the short answer is that they will be posted on PCORI's website, www.PCORI.org, and on that website you will be able to look at the detailed document on the Priorities and the Research Agenda and you will be able to make comments both in the form of answering questions that we've put out or -- and/or in free text that you enter as much as you want, and I believe there's even a capacity for you to attach documents as well.

DR. NORQUIST: And if I don't have an

internet connection or I don't have a computer or something, can I send you a written document that you would then read?

DR. SELBY: Yes.

VICE CHAIRMAN LIPSTEIN: I guess, I would like to just -- before we leave this section, just, again, Rick, since a lot of this work emanated from the Program Development Committee, to compliment you, your committee, to compliment Arnie and Carolyn and the people who led the entire effort on the National Priorities, Harlan and Leah and everybody who led the effort on the Research Agenda, Gail and Carolyn, when we get back to the discussion of the Pilot Projects, there's just been an incredible amount of work on the part of so many people around this table that got us to where we are, to the point of, at least, launching for public comment these Priorities and this Research Agenda. So, kudos go out, really, to everybody, Dr. Washington.

CHAIRMAN WASHINGTON: [Off microphone.]

We have two Board Members, I think, on the

phone. I don't know if you're still on, Dr. Collins, Dr. Douma.

DR. DOUMA: I'm here.

CHAIRMAN WASHINGTON: Any comment?

DR. DOUMA: No, I really don't. I think the important thing is to make sure everybody understands that we're listening and there's a lot that I think we need to listen to and listening to the conversation, I myself might send in something.

CHAIRMAN WASHINGTON: Okay. And I was just asked, officially the first draft will go out on Monday, the 23rd of January. It will be posted on the website, but we have a long list of organizations, individuals that we're going to be sending the draft to around the country.

If you're listening and you feel like your name, for whatever reason, is not on that list and you want to receive direct notice, in addition to it being on the website, they should send the note to what address?

DR. SELBY: This is Joe Selby. I would send it to PCORI and maybe -- PCORI's address is

1701 Pennsylvania Avenue.

CHAIRMAN WASHINGTON: I was talking about email, but okay. PCORI.org.

DR. SELBY: Does anybody on the staff have a --

CHAIRMAN WASHINGTON: I thought there was a specific --

DR. SELBY: Pardon?

UNIDENTIFIED: Info@.

DR. SELBY: Info@PCORI.org. Thanks.

CHAIRMAN WASHINGTON: Okay, and with that, I want to join Mr. Lipstein in again conveying my deepest gratitude and appreciation and thanks to everyone that's been involved in each step, including those that are not here on the Board, but particularly my colleagues on the Board, in developing this draft National Priorities and this first iteration of the Research Agenda. So, thank you.

[Applause.]

CHAIRMAN WASHINGTON: Okay, Joe I recognize that we have two parts of this report.

Actually, Rick. What we're going to do is stay on time for the public comment period, people are expecting that, which means that you get to stretch for four minutes rather than having you introduce -- and we'll come back to the Methodology Committee report after the last speaker in the public comment period. Okay?

[Pause.]

CHAIRMAN WASHINGTON: Richard is going to introduce this session.

MR. SCHMITZ: Thank you, Dr. Washington. We have five individuals registered on site to provide comment. We will hear from those individuals and then we'll check on the teleconference line to see if there is anyone else who would like to provide public comment, and so just to remind everyone of the guidelines, we request that everyone limit their remarks to three minutes and we encourage anyone to submit written comments to PCORI by email at info@PCORI.org and the materials that are received will be distributed to the Board, committees, and staff for review and

consideration of PCORI's work.

So, our first registered commenter is Jim Patterson.

MR. PATTERSON: [Off microphone.]

MR. SCHMITZ: That's fine. Jim's electing to speak tomorrow, so our next commenter will be Andrew Sperling.

MR. SPERLING: Good afternoon, members of the Board. I've testified several times before. I'm grateful for the opportunity to do so again. My name is Andrew Sperling, I'm the director of legislative advocacy for NAMI, the National Alliance on Mental Illness, and I'm here on behalf of the Partnership to Improve Patient Care, and I serve on the PIPC Steering Committee.

As you know, some of you hopefully saw a letter that PIPC submitted to the PCORI Board in December. We're pleased that PIPC is getting work on establishing research priorities and agenda and we fully urge an open and transparent process that provides adequate opportunities and sufficient time for meaningful input, and we think what was

outlined here today is a major step forward in that direction.

The single most important outcome PCORI can achieve in the year ahead is to establish decision-making and operating procedures that build in both buy-in and trust from patients and the providers and the public at large, and we strongly urge PCORI to consider four specific recommendations today.

First, we are encouraged that the comment period for research Priorities and the Research Agenda are moving in the right direction. We urge that they be sufficiently specific enough so that patients and providers can comment on the level of responsiveness to the research questions that we view as important and making sure that they're truly patient-centered.

What matters most are the specific research projects PCORI intends to fund and that there be a process in place so that sufficient stakeholder input can be offered and that there's real transparency.

Second, we believe that PCORI should provide separate and sequential public comment periods on the research Priorities and the Research Agenda. We understand that this will be posted on Monday, but we urge that it be sequential, separating the research Priorities and the Research Agenda. It is our sense that the statute envisions sequential development of specific research and projects. We would like to hear more from the Board as this meeting goes on about the anticipated process of how we're going to get to that level of specificity on research projects.

Number three, as PCORI develops these research Priorities and the project agenda, PIPC would again urge you an adequate opportunity for broad input from patients, physicians and others. I think what Dr. Selby has outlined in terms of a public process of having that posted and allowing not just the questions that the Board wants answered, but having broad public comment, is going to be a major step forward in that direction. We understand that there's a meeting plan for February

at the National Press Club to get even more input. That's critical. Looking beyond just limited comment periods so that we can, as Dr. Norquist outlined, really reach constituencies that haven't been at the table in terms of offering input into the Research Agenda and the research Priorities.

Finally, PIPC recommends that PCORI describe a systematic process for describing how input it receives will be considered and incorporated. This is a critical step that the research -- the input not only be offered to the PCORI Board, but there's a process in place for making sure that it's considered and incorporated. For example, there have been several mentions of focus groups that the PCORI Board has undertaken, yet it's still not clear to us at PIPC how these focus groups are being used to drive your decisions or policies.

Public input opportunities, such as web surveys and other things that were administered earlier this year, written comments on the definition of patient-centered outcomes research,

have all been a step in the positive direction, but we believe -- PIPC believes that greater clarity is needed on how this input is deliberated upon by the Board and the Methodology Committee and the Program Development Committee. It's critical that there be a process in place, a transparent process, as to how the PCORI Board is going to take the public comments you get and consider them and incorporate them into the Research Agenda and the research Priorities.

PIPC recognizes that this is not an easy task for the Board, but PIPC does commend the PCORI Board for the work you've undertaken in this initial comment period in the development of the National Priorities for research.

PCORI was created to be different from existing comparative effectiveness programs and to be truly patient-centered, and this input process and deliberation over that input is critical to making sure that you hold to those goals. So, thank you very much.

CHAIRMAN WASHINGTON: Thank you, Mr.

Sperling, for those comments and for those suggestions.

MR. SCHMITZ: The second registered commenter is Malcolm Foster.

MR. FOSTER: Thank you very much. I am -- I live in Jacksonville. I'm an endowed professor of medicine for the University of Florida, most recent president of a local medical society and of its foundation, current president, and just rotating off as chairman or governor of the American College of Physicians, the Florida chapter, and so each group has thousands, 6,000 in one case, 3,000 in another, physicians and I'm in touch with them.

So, my question -- and I want to, first of all, just welcome you to town and we're proud, we've got a good medical community and I'm thrilled that you're here. I realize that you could be meeting in Atlanta at the Atlanta airport, most anywhere else because you're stuck in a hotel, but we're happy you're here.

I'm very impressed with the deliberations

I've heard. Many of the things I wrote down that perhaps I wanted to say to you I don't think need to be said because you're doing them and I'm very impressed with this discussion.

The American College of Physicians has a longstanding support of comparative effectiveness research and we continue that. That will be, I'm sure, a major stakeholder in one way or another. I might mention the American College of Physician Foundation, which I didn't come prepared to talk to, except I've been on its board for four years, and its emphasis is on patient literacy. It was mentioned here, but just remember that most patients, and I could extend this to tell you that most professionals that I take care of, and I take care of a lot as their doctor, cannot communicate much above a fourth grade level, I'm just telling you, and they can't remember more than about three things. I don't care who they are, it could be any of you.

So, there's all these layers on top of your wonderful things that you're doing that's been

outlined very nicely in the methodologies for research, but then there are these layers that we have, and then I just maybe will emphasize two things, and then I'll shut up, the first is -- and when we talk about the disparities, I think a lot of us think about racial disparities, that's common, but we're still in the dark ages, men versus women, children versus adults, how about Haitians, how about other minorities, Latins, the list goes on and on in terms of problems that we have. Which group has rapid accelerators, for instance, in their liver? Which group cannot take statins? This came out recently. And it just goes on and on.

So, there's a lot there that's got to be layered as best we can about that. And then the only final thing I want to say was actually the first thing in my notes, was I thought maybe we ought to -- we ought to all think about how does the public, meaning the doctors, think of you guys and girls? And you may not like it. I think the -- this is an outfit that's going to lead to cost

containment, cost containment might lead to worse things down the road, so I think because of that you've got to do a good selling job, and I know you will. I know you will. I know you'll come to all our national meetings and they'll come -- and it will drill down as best as it can.

But it's like the moonshiners used to think about the revenuers, the Feds that were coming in, so it is a problem, and I've just made a few casual observations about this before I came over here, and to mention that I think you're so high-minded, I wish everybody could just sit here and listen to this, I think you're just so high-minded that you're probably hurt that somebody would say, gee, we're here from the government and I'm here to help you. I know you're not from the government, but I know where your mandate came from. I've been part of that, by the way.

So, welcome, keep up the good work. I'll say what I can in a very positive sense about you in the future.

CHAIRMAN WASHINGTON: Thank you, Dr.

Foster. Wait, I want to get this right, are we the moonshiners? Or --

[Laughter.]

MR. SCHMITZ: Our third public commenter is Jennifer Graff.

MS. GRAFF: Well, thank you for the opportunity to comment. I've had the pleasure of getting to watch first-hand the progress, the very thoughtful deliberation, and also see the progress in the fact that the PCORI Board is very much listening. In fact, so much so that I think Dr. Selby has tried to take away three of our comment points that we had for later today.

My name is Jennifer Graff and I'm a research director at the National Pharmaceutical Council. NPC is a policy research organization dedicated to the advancement of good evidence in science and to fostering an environment in the U.S. that supports medical innovation.

Since we think about priorities and Research Agenda, we all think that this is very critical and, in fact, for that reason, back in

late 2009, NPC's chief science officer and I undertook a review of nine different organizations, and I know many of you have done this as well because there's lots of learning to see. So, we looked at IOM CER priorities, Blue Cross/Blue Shield Tech, NICE, et cetera, to identify what are some of the optimal processes.

You've already incorporated many of these, but we'd like to offer up a couple of points for your consideration. Because we recognize the tensions that you have, you have the tension of trying to have very open-ended, broad spectrum, to get new ideas with being very specific, you have a tension between trying to get something done very quickly and trying to get it right the first time.

So, in light of some of those pressures, good luck, we're wishing you much success, we offer five points for your consideration.

So, the first is: rank order and weight priorities to identify what has greater and lesser importance. Identifying not only where you're going to spend your money, but where you're going

to spend the precious PCORI staff time and the reviewer time will be very helpful. For example, PCORI might decide to spend 40 percent of its effort on funding comparative clinical effectiveness, 20 percent on healthcare systems, 20 percent on communication and dissemination. But how you identify where you're going to spend the time is going to be an important flag for the U.S. public to understand what is important to you.

So, if you really truly do want to be -- have CER as a self-sustaining environment, putting that flag and that percentage of effort will really help the U.S. public.

We also very much appreciate the idea of incorporating stakeholder feedback to weigh in on these priorities as you move forward.

The second point that we'd like to make is, be specific. And, again, I'm encouraged to hear that you are moving towards specificity with the PFAs. The likelihood that the most critical questions will be undertaken and answered with an adequate research portfolio really requires

specificity and what we mean by specificity is similar to CER questions like many of the Methodology Committee looks at, we're looking for patient, what patient, what's the outcome, what are the comparators and what's the setting.

We recognize that not all of the research questions can be answered, but we also recognize that it's unlikely that a single research project will answer all of the questions for that research director or research decision makers or that, unfortunately, that single project is going to be striking enough to change clinical practice.

We'd all love that home run, but it just doesn't happen very often.

So, we recommend, be specific where you can. And to help you in that process, one of the lessons that we found in looking at what other organizations had done was that engaging all stakeholders in not only the identification, but in how you prioritize the research, is critically important and how that will go on is quite important as well.

Without that stakeholder engagement in the prioritization, it will be up to the funding and research committees to determine what priorities are important you lose an opportunity to really seek broad feedback. So, we suggest that you use ad hoc panels as was suggested previously.

We also suggest, as our fourth point, maintain an open and transparent consensus process to help deal with that inevitable disagreement. It's going to happen. How do you take the feedback? How do you incorporate it? How do you quantitatively assess this? And the Methodology Committee has many very smart and well-versed people on this methodology.

And then, finally, plan for success. Not only should you evaluate the process internally, but also seek that engagement from the external community that are engaging in the process and the public comment period coming up.

So, I thank you for your time. We recognize any prioritization process will be under scrutiny, and we hope that by signaling PCORI's

rank order prioritizations or weightings, balancing the portfolio with a broad set of research priorities, as well as specific research questions, engaging the stakeholders in the prioritization process, and evaluating this, it will be beneficial to not only the Board and the public.

Thank you very much for your time and we wish you much success.

CHAIRMAN WASHINGTON: Thank you, Ms. Graff.

MR. SCHMITZ: Our fourth commenter is Daryl Pretchand [phonetic].

[Pause.]

MR. SCHMITZ: That appears to be all of the onsite registered commenters we have, so at this time we will check with our operator to see if there's anyone on the phone who wishes to make comment.

Carla?

CHAIRMAN WASHINGTON: Maybe she's not on the phone. Okay. So, Allen, would you like to comment?

[Pause.]

CHAIRMAN WASHINGTON: I'm not sure the phone is working. Okay, as has become our tradition, given that we have about ten minutes left, I would ask if there's someone present in the audience who didn't register but would like to make a comment? Please.

DR. HAYES: Hi. My name is Dr. Winnie Hayes and I'm with Hayes Incorporated and thank you for the opportunity. I've been following the progress of PCORI for some time. Dr. Levine, has, I think been to most sessions. But I have two quick comments I want to make.

As I sat here today, one thing that did strike me is to ask the question, how will PCORI demonstrate its return on investment? And as I thought about that concept, return on investment, my interest certainly wasn't just financial return on investment, but the impact, the impact that the research that you'll be funding has, and there's a focus -- one of the focus that you've articulated quite clearly is communication dissemination. And

I'd like -- and perhaps it's embedded in that thinking, but I'd like to suggest we also think about what's the uptake. What's the impact on patient behaviors? What's the impact on clinical behaviors?

I've been in the field of comparative effectiveness and health technology assessment for 20 plus years and I think one of my biggest frustrations is that even in situations where we have decent scientific evidence that would provide direction in terms of clinical care, oftentimes we don't see an uptake on the part of clinicians and we don't see understanding on the part of patients.

So, I would really be interested in seeing how PCORI plans on evaluating the return on investment. I think that's critical to a sustained effort. If we can't demonstrate that to the public and to the politicians, then I think we've got some problems.

The second request I have is, it's thrilling to see the number of researchers who want to participate in patient-centered outcomes

research. It's kind of almost overwhelming. One request I have, as a researcher, is for feedback to those researchers who might be, now and the future, unsuccessful in receiving funding for their proposal. I think it's critical that that interested community of researchers understand why they're proposal wasn't funded.

Now, I know there's a lot of work involved in doing that, but I think we need to understand, if we're one of those folks, was it the cost part of the proposal, was it the basic research design, was it not enough of a priority. So, I just request that some mechanism be introduced to give those researchers feedback. Thank you.

CHAIRMAN WASHINGTON: Thank you, Dr. Hayes. I can assure you that we have certainly taken on the question of uptake as part of our definition for dissemination.

Richard?

MR. SCHMITZ: I thought I heard our operator there briefly, so I want to check again to see if our operator is available and if there's

anyone on the phone who wishes to make comment.

[Pause.]

CHAIRMAN WASHINGTON: Okay, Richard, that's strike two. Since we're using baseball analogies. You can wait a few minutes and we'll ask again. In the meantime I do want to thank all of our presenters. I noticed that two of our presenters, Ms. Graff and Mr. Sperling have traveled with us and presented on previous occasions. So, we greatly appreciate the continuity and benefitting from your interactions with us on multiple occasions. So, thank you.

Okay, do you want to ask one more time and we're going to move on.

MR. SCHMITZ: All right. Is our operator available online and are there any comments from those participating by phone?

OPERATOR: There are no comments, thank you, sir.

CHAIRMAN WASHINGTON: Great. We like closure on this Board.

Okay, with no additional comments, I'm

going to ask that we move, Rick, to the next portion of your committee's presentation.

DR. KUNTZ: So, I think we -- this is Rick Kuntz, with the PDC. We may be a little behind schedule but I think we'll ask Christine to give a quick update on the Pilot Project grants, and this is an effort that's been spearheaded by Christine and Gail Hunt and also by Kim Myers who's been supporting on the staff. Christine?

DR. GOERTZ: Thank you. Does anyone have the clicker?

I also want to really thank Camille Cain on the Deloitte Staff for all of the work that her and her team have put into this effort. It's been really extraordinary at times.

So, just really briefly, I'm going to talk a little bit about some of the things that we've done thus far, give a brief overview, just a reminder of the four stage review process, and then talk a little bit about the merit review process, and then, finally, a little bit about how the PCORI Board is going to be making decisions regarding the

applications, and then finish with a timeline.

I know we're a little bit behind. I'll try to be as brief as I can.

So, as you know, we've -- we have issued our first funding announcement in September. We did a series of webinars. We had 850 people who registered for the webinars. We received 1,375 letters of intent. We answered approximately 1,400 questions from applicants. And we received 842 applications in the end.

Just to give you a little bit of an idea of what receiving 850 applications looks like when you get, I think, four copies of each all on the same day -- I guess we got them over a two or three day period, but most of them came in, I guess, within one 24-hour period is not accurate, Joe, and the picture on the bottom right hand side is a picture of the Christmas card the NIH staff sent to us of the PCORI Christmas tree at the NIH office. So, those are the -- that was the stack that NIH is now sorting through and assigning to reviewers.

Just a reminder of the process that we

have initiated and are now going through, when the applications came in, PCORI was responsible for doing a check to make sure that the applications met our technical requirements so that there weren't -- that the page limits weren't exceeded and other things, before we sent them off.

In addition to that, a minimum of at least two Board Members or senior PCORI staff read each abstract to verify that the application was indeed responsive to the criteria that we had outlined in the PCORI funding announcement, and those applications where there was some question about whether it was responsive or not, we pulled the full applications and read those to determine whether or not it was responsive.

The applications that were responsive were sent to NIH where they have been assigned to three reviewers, and I believe those reviewers are working on PCORI applications probably as we speak.

The review committee meeting will happen towards the end of February and after that the Board will be considering which of the applications

we're interested in funding. This is basically a three-stage process. The first part of that process is for a subset of the Board and Methodology Committee to develop criteria for how we might want to balance our portfolio, and I'll talk a little bit more about that in a minute.

After that, a small group of the Board will be going through the summary statements and the scores received from NIH and queuing those up to slate for consideration by the Board and the Board will make final funding decisions and then the applications that are recommended for funding will go through another staff review just to make sure that human subjects requirements are met and that there aren't any financial barriers to issuing the checks.

Just some reminders about the merit review, so again, each application will -- is reviewed by three reviewers. The reviewers include both stakeholders and scientists. I think we have a minimum of three stakeholder reviewers on each of the review panels, so they will be able to -- they

will be reviewing applications that they're primarily responsible for, but also able to listen to the discussion and comment on all the applications that are reviewed.

NIH has been working to train stakeholder reviewers. That training has consisted of basically a teleconference to provide information about the review process and then some direction so stakeholders or people who haven't done a review before were asked to review their first application by -- basically by now or approximately in the near future, and then they receive one-on-one feedback from the scientific review officer about their review and things that they might want to consider thinking about as they do their other reviews or edits, they might want to think for that particular one. Because, again, we're aware that people may not have done this before and it can be a little bit daunting as process.

And, so, working with NIH we've been doing whatever we can to help people through that process.

Reviewers will assign an initial score to each application. The three reviewers will assign an initial score to the application and then they will meet in person, again, as I said, towards the end of February, for a one-day meeting. We will be discussing the most promising, probably, 50 percent of the applications during that review committee meeting, though any reviewer will have the opportunity to recommend that an application be discussed even if it's not in the top 50 percent. The entire committee, then, will provide a final priority score from one to nine after a discussion takes place that looks at impact, stakeholder involvement, innovation, and significance of the science.

After that, a scientific review officer will -- they will compile a summary statement that includes the comments of the primary reviewers and a summary of the discussion for those applications that are actually discussed and that, along with the priority score, will be sent to PCORI so that we have that as we're considering which

applications we're interested in funding.

Once, again, we're putting together a working group, which will help decide what our criteria for evaluation are. So, when we're trying to seek what federal funding agencies sometimes call programmatic balance or trying to figure out what types of applications we're interested in and want to be funding, sometimes it happens that all of the top applications might be on one topic area. In that case, we might decide that we want to consider other factors as well, and so this committee will be responsible for helping determine what those factors might be, and that could be, you know, perhaps the type of research methodology that's being used, the entity that's being studied, the population -- either the population that's being studied or the disease or condition that's being studied. There are a number of metrics that might possibly be considered. Obviously scientific merit will be the key metric, but coming up with other metrics as well.

PCORI staff will then work with a

committee, another committee, of the Board to think about the analytics that the working group has developed and then to look at the scores and try to develop a slate of applications that would be recommended for funding, again, based on all these considerations, and then the Board will make the final decision about which applications will be funded.

Just a reminder of the timeline, we hope that the review committee will be able to recommend the slate for funding sometime in March. We will not be able to do that in time for our March meeting, but it will probably happen shortly after our March meeting in Baltimore, and then that information will be provided to the Board with the hope of making decisions sometime, probably realistically, in early April, sometime in April, with award notification to still happen in May, which is along the lines of our original timeline.

I'll be happy to answer any questions that anyone might have.

CHAIRMAN WASHINGTON: First, I'll ask Joe.

Do you have any additional input related to this?

DR. SELBY: Thanks. And thanks, Christine, for your persistent hard work on this. I wanted to ask a question about these criteria. So, the priority scores from the merit review come back from the NIH review, and the Board then, working with the staff and that small committee, will apply some criteria to make final funding decisions, and I just -- I wondered what you thought about the possibility that -- or, I should say, we've been advised, and we've also stated that we want to be transparent in these kinds of activities, so I wanted to ask you about the possibility that those criteria, number one, might be available for review and discussion at the early March Board Meeting, and number two, about the possibility of finding a way to include stakeholders in the, you know, the selection of these criteria, not the merit criteria, but the criteria that help us with balance after the merit review is done.

DR. GOERTZ: I think that's an excellent

idea. I don't -- in the same way that we put the -- first of all, absolutely we can come back to the Board with those criteria in March, if not sooner, on a Board call, because I think, you know, by the March meeting we can certainly present what the criteria are, but as we sought public input for our areas of interest before we initiated our funding announcement, we can do something along those lines. Once the working group has come up with draft criteria, I don't see any reason -- I think it's actually an excellent idea to put them on our web and ask people for public comment and so that we can refine those before the final decision is made by the Board so the Board will have the stakeholder input in addition to the input of the group that puts together the first draft.

CHAIRMAN WASHINGTON: Sharon-Lise and then Harlan W. and then Carolyn.

MS. NORMAND: So, I had, perhaps, two questions. But, first of all, congratulations. Nice postcards, nice Christmas cards. To follow up a little bit on Dr. Selby's comment, in terms of,

sort of, what expertise or consistencies are you thinking about to put on this balance committee? So, I was interested in thinking about that. And then with the comments just made, it seems -- I guess I'm having a little bit of trouble of thinking about -- I don't know if fair is the right word, but how to make sure there's no conflict between getting suggestions on how to -- what criteria are used to make the selection when the application's already been received. In an ideal world, I think that should have been done beforehand rather than after, only because, pretend I applied, I could sort of give you all the information because I know what's in my application.

I'm not saying anybody's going to do that, but it seems a little bit it's the wrong timeframe to ask for comments to the extent that people who are giving the comments either had participated in writing them or have seen them.

Just, from a purely scientific standpoint, I think that's my initial reaction, and maybe it's

wrong and people will say, that's okay, it's not an issue for those individuals such as Drs. Clancy and Collins who know more about this. So, that's the comment and the question.

The last one, and maybe I shouldn't ask this, do you have any idea how many will be funded?

DR. GOERTZ: First of all, to your first point, I think that's an important point as something that we'll definitely have to consider as we're trying to develop a process for moving forward. Obviously, the original intent was basically to have a subset of the Board and the Methodology Committee come up with those criteria. So, whether we could put it out for public comment, that's something we'll have to discuss more, because I think you do raise an important issue.

The nice thing will be that when we do our next round of grant applications we have learned a great deal through this process and I think we will do it much better and I think it will go a lot more smoothly when we do our next go around with this.

Regarding how many applications we might

fund, I have -- it's hard to say for sure, obviously, because we don't have a -- we haven't seen what the science is, but I can tell you that I've read almost every abstract and based on that, my guess is that we may be interested in funding more than the original 40 that we had discussed, and so you remember at the last Board meeting I more or less concluded with a plea that the Board consider the possibility of expanding our funding for the PCORI Pilot Projects Program if, in fact, we did receive enough applications, and the flexibility to do that is written into the budget for 2012.

So, again, it's going to really be based on the science in the end, but we do have that flexibility built in.

CHAIRMAN WASHINGTON: Could I just comment on Sharon-Lise's question and her point regarding timing of the development of the criteria for what we call balancing a higher programmatic activity area? And that is, is that scientific merit review is the first and foremost criteria, but I see these

as -- well, first, I think it would have been great if we had had them, but not necessarily for investigators or those who were going to apply because the criteria that we were going to judge the scientific merit on is what was going to drive this process to this point.

Beyond this point, then it's a question of -- I mean, the example that someone threw out to me is if the top 17 projects all related to prostate cancer, we wouldn't -- I don't think we'd want to fund 17 or whatever the number, and so there should be some criteria, which we haven't decided on, that now represents our view of the world given that we're a patient-centered, national patient-centered research center.

So, yes, in an ideal world it would have been nice for us to be able to say, you know, we had developed them and that we had input all along, but at this point, I can tell you, they're not developed. We don't have them in an envelope. And, as we think about developing them -- I think what Joe was saying was not just putting them out

there, but if it wasn't feasible for us to have a process where we actually had some stakeholder input in developing them, and, so, in that sense, we are starting from scratch and we are talking about, between now and the next Board meeting, a process where we would actually have stakeholder involvement in developing those criteria that would then go online.

MS. NORMAND: My only concern -- this is Sharon-Lise Normand, Methodology Committee -- my only concern is, and Dr. Clancy may tell me, be quiet, it's not a concern, is when you open it up to the whole public who've already applied and they give input, and maybe I'm wrong about that.

Obviously, the Board needs to make those decisions because they haven't applied, but it's opening up comments on the criteria, and we can't ignore the comments if we're soliciting them.

So, if we're ignoring -- if we solicit comments from people who potentially applied, I sort of see that not right, but I don't know.

CHAIRMAN WASHINGTON: Leah.

MS. HOLE-CURRY: Just a quick response. I think one way to deal with that is to make sure that if we solicit from the public, in this case we may want to consider having individuals identify themselves so that we could do a cross reference in that way or just disclose that they have been related to one, in this case, not for all public comment. In this case.

DR. WEISMAN: Harlan Weisman. I had a quick and dirty approach to this and I really loved what Joe said and then it got deflated on what Sharon-Lise said and now it's gotten boosted back up. So, I do like what Joe suggested and I understand the consideration, and I think at least asking people to say there's, you know, a minor conflict of interest, that they don't have that conflict, you know, it's the best you can do.

But mine was, these have just been -- the ones -- the process you just outlined has just been publically shown. We're not asking for a formal public comment or comment period, but I would invite the public to comment on what you've already

shown us, you know, in terms of the process we're following, you know, the committee structure, how we're assigning it, because this is critical and if there are any concerns about the way we're doing this, even without the specifics, I think that would be good to learn, and I would hope that it would be a way of engaging people beyond that -- along the ways that Joe said.

I did have another question, and that was, just to ask Dr. Hayes a question, which was, there are going to be people who are funded and people who aren't going to be funded. There are people who have already been screened out and people who have been screened in. Will the people who are not getting funded be given an opportunity to learn from the experience? And, if yes, could you outline what that process is?

DR. GOERTZ: Absolutely. On each -- for each application that -- first of all, the people whose application was not forwarded to NIH, there were relatively few of those and those people we're in the process of notifying them right now. The

ones -- for all of those that were sent to NIH, there will be written comments from three reviewers on each of the applications and on those that are discussed there will be a summary paragraph describing the discussions.

So, we will be -- what we call summary statements at NIH, and those summary statements will be forwarded to the applicant so that they do have that feedback about their proposal.

DR. WEISMAN: And on those initial few that were eliminated really for technical grounds, will they -- I think even that's a type of learning about how to write a grant.

DR. GOERTZ: Absolutely.

DR. WEISMAN: So, whatever the number is, they will also not only be informed, but told what the issue was?

DR. GOERTZ: Right.

DR. WEISMAN: Okay, good. Good, good.

DR. GOERTZ: And actually anyone who was -- who we might have eliminated for technical reasons, because of page limits or such, we

actually did contact those people and give them an opportunity to fix whatever the technical problem is, so all of those people are already aware that that was an issue. I'm not talking about the applications, the evaluations for responsiveness to the PFA, but just the technical review.

CHAIRMAN WASHINGTON: Okay. Dr. Kuntz? Rick? Oh, I didn't see any hands but now we have Dr. Clancy and then we're going to ask you what's next.

DR. CLANCY: Carolyn Clancy, Board Member. And, Sharon-Lise, just for the record, I would never disagree with you publically or privately or tell you that you were wrong.

I had a question about the folks who are not considered in the top -- or the applications that are not considered in the top 50 percent, and at one point it sounded to me like you said those in the top 50 percent where the applications are discussed will be forwarded to PCORI. I would hope that we would also get to see blinded copies of the reviews of the others, because I think it would be

a potentially enormous source of learning, some of that may be learning about the reviewers themselves, for example, there may be some that -- wildly divergent scores or something along those lines. Some of it may be about one or more communities of folks who have fabulous ideas but actually don't know very much about grantsmanship, but that, in turn, could shape future efforts at technical assistance and so forth.

So, I'm hoping that we get to see that content as well.

DR. GOERTZ: Yeah, I misspoke when I said that. We will absolutely get the summary statements from all of the applications -- the scores and the summary statements from all of the applications, whether they're discussed or not. Thank you for asking that question so I could clarify that.

DR. CLANCY: Yeah, and for the record, I was being amusing a moment ago. I actually do think Sharon-Lise is right on this, and I'm not altogether sure that I'm that comfortable with

asking for public input on a decision that's about to be made in real time. For future solicitations, I think that's much, much cleaner. By definition, these Pilot Projects, we cast a very, very broad net by design and by intent, in part because it would inform future iterations of our priorities. And that fifth box, as it currently stands in the draft, is one area where we may want to boost the infrastructure in terms of human capital and what it's going to take to help people make the right connections to people -- between folks who know how to write grants and people who've got way out of the box ideas but don't actually have that skill set or experience.

CHAIRMAN WASHINGTON: We are pressed for time, but I -- if we're talking about the same question, then I want to challenge that idea that we wouldn't ask for stakeholder engagement or public input into developing criteria that have nothing to do with the scientific merit. We're talking about a totally different phase of decision-making and, you know, we're not behind the

eight ball here. As a Board, I have not had a conversation with anyone, certainly as a group, about what criteria are we going to use beyond scientific merit.

I mean, at one extreme the decision could be zero, where we're just going to take, you know, the top rated. I don't think that's going to be the case. And then somewhere on the other end is, okay, we've got some criteria that we think are important based on our perspective experiences, but I see no reason why we wouldn't want to, you know, at this very early stage, get some input about, you know, from other people who have experience, and not necessarily in research, about what's important for PCORI to be thinking about, why we wouldn't want their input. So --

DR. CLANCY: Well, let me just respond for one second and say I was sharing my own personal view on this, so I understand that others may differ on that. I think one likely source of feedback that wouldn't be necessarily all positive would be something like, gee, if say geographic

diversity is something we want in terms of patient population, I, Sharon-Lise Normand, would have found a rural co-investigator. So, I think that's one kind of feedback that we might get. So, that's a little bit of my concern. I'm, perhaps, a bit more risk averse than the rest of the group is comfortable with.

MS. NORMAND: So, can I also add? This is Sharon-Lise Normand, Methodology Committee. So, I'm not -- I think we should get the information, but I just don't think it should be used for this round. And, again, part of it is the example that Dr. Clancy gave, but here's another example. We get 17 applications all looking at hypertension, and we solicit feedback from whomever, it's wide open. And several people have put push grants on prostate cancer -- I'm making this up entirely -- then you could lobby and get a lot of people to email in comments to say, you know what, I think prostate cancer is the real one you want to get.

So, it's not clean at this point.

CHAIRMAN WASHINGTON: So, just to play

this out, Dr. Clancy, it's okay for a Board Member to make that same suggestion regarding geographic distribution. We shouldn't discount it if it comes from a Board Member versus if it comes from someone that's out -- a part of another constituency. What's the difference?

DR. CLANCY: Well, to some extent being a Board Member means that you have a fiduciary responsibility for how well this institute does. I'll take the point. I'll also say that we could certainly be transparent about the options that we considered in terms of a public report to a whole variety of stakeholders in terms of how we came to the final decision, and that was tricky.

My own thinking about this has been in terms of this small group teeing up options. For example, if we value geographic diversity, the list would look like this versus like that.

But not having sent any signals, I think, is a little bit tricky.

CHAIRMAN WASHINGTON: Thank you, Carolyn. I have Steve and then -- let's just work this way,

Steve, Freda, Rick, and Harlan.

VICE CHAIRMAN LIPSTEIN: So, again, I'll speak, I guess, my personal point of view is you don't get into condition-specific criteria if you say that of these first 40 or 50 grants that we're going to fund, no more than 25 percent would be associated with any single medical condition. That's a kind of a criteria that doesn't bring out the people who are in favor of prostate cancer or any other condition, so there's a way to make it neutral. That would be one observation.

The second is, if our first 40 grants all go to investigators from Massachusetts, you can bet that that will affect the second round submissions, and so I do think that we may want to say that, again, no more than 5 or 10 percent our submissions would go to investigators from any single state, and, again, it still makes it state neutral, but what it does is it reflects that America's a pretty diverse place and we want to make sure we cover the full spectrum of possibilities. But you can do it without being state specific or territory specific

or diagnosis specific.

DR. HALL: Freda Lewis-Hall. I was actually going down the same road as Steve, which is, I'm a little confused about what we would actually be asking for and from whom, but if we were asking general questions about how to diversify a portfolio, I'm not sure it matters because they wouldn't be privy to what we currently have on the table.

So, if that was the direction, I would be comfortable to go down Steve's road, you know, what are the most important criteria for differentiation, how would you want to see us stratify those, what is the rank order importance of those things to you, and you aggregate that and get a sense of, you know, how people would like to see us diversify this research portfolio.

If, on the other hand, what we're proposing is that we would actually talk about what we currently have and ask for comments on how to diversify that, that I would be very uncomfortable with.

DR. GOERTZ: Yeah, no, we would not do that.

CHAIRMAN WASHINGTON: We're definitely talking about the former, not the latter.

DR. HALL: Okay.

DR. KUNTZ: Rick Kuntz, Board Member. I was just going to say the same thing. I think this is a good discussion to have and we need to have more of these.

I want to emphasize as to what Sharon-Lise and Carolyn said because I think the appearance of bias is something we want to avoid early on. And I just wanted to remind everybody, there was so much of a pilot phase to this, I mean, we wanted to get in quickly, kind of, you know, grease the gears a little bit to understand how the core process works, to already discover that there's a lot of interest out there with 850 grants that we got formally submitted.

I don't know what the down side is for us to not, you know, utilize criteria from the public in this first round, if there's going to be an

appearance of bias, and we should just utilize more rigorous, formal criteria that we can come up with before we get public feedback.

CHAIRMAN WASHINGTON: Harlan.

DR. WEISMAN: Yeah, I'm assuming -- I just wanted to, and maybe this is what Freda said, but I would like to understand from either Sharon-Lise or from Carolyn, that if we, meaning your working group, Christine, came up with the factors that you think are important, and then we had to -- and you used the word balance, which means that we're going to put some weighting on each one of those in terms of its importance, and I agree with Steve, that can be done generally, like condition or geography or whatever it is you like, then by asking stakeholders not to get -- only to tell us what they think those proportions should be in what has already been determined as important -- you know, the factors we want to look at, is that -- I'm struggling with why that would introduce bias if there are these kind of general categories and we're only asking about weighting.

CHAIRMAN WASHINGTON: Could I just clarify one thing just before you respond? First of all, we, as a Board, have not made a decision that Christine's group is going to be the group to come up with the criteria.

DR. WEISMAN: Whatever the process is, PCORI could say, these are the factors we're going to judge these on, here's the weighting we're thinking about, tell us about what you think about our weighting, which is sort of a generic question about the importance of these things rather than getting very specific that involves the content of any specific grant.

MS. NORMAND: This is Sharon-Lise Normand, just because Harlan asked.

CHAIRMAN WASHINGTON: Please.

MS. NORMAND: So, I'll tell you, the issue is, I just want to be very clear at least in terms of the way I see it. One issue is asking the public, which may be stakeholders, whoever they are, to comment on something when the members of that public have submitted things. That's where I

see the problem because I could get a thousand of my friends to come and say, you know, you absolutely need to fund this. I realize this, you know, may not happen, but it could happen.

So, that's the concern I have about getting information to help us decide on something when I've got a horse in that race, and I can get all my friends -- that's the problem. I have no problem with, yes, we need to have a way to decide which are going to be funded, we obviously need some criteria. So, it's doing it after they've been submitted already to come up with those criteria. So, that's the first thing.

The second thing, and, again, it's -- you could have the general things that Steve said and that's fine, but it's a little unfair, I would say, and I think that was Dr. Clancy's example, to say, we're going to favor studies that have focus on rural populations. I would have submitted a different application then, and that's, sort of, you know, it's okay going forward but not going backwards in time, and so those were the -- the

main issue was having people from the outside who have a horse in the race to give your comments about things.

DR. CLANCY: Carolyn Clancy, Board Member. The one thing I would add is, it does create something of an appearance that we cut the data first, that is to say, we were looking at the list of grants and then concluded that we needed criteria to go with rather than having done it prospectively. Now, that may be a kind of researcher bias in terms of you state your hypotheses and criteria up front before you actually start cutting the data, but --

CHAIRMAN WASHINGTON: Again, this is very helpful because the proposal is, is that we do this long before we see any of the data that's coming from NIH.

DR. CLANCY: Well, to be clear, though, some of us have actually helped review the abstracts so that they could be double reviewed and so on and so forth.

CHAIRMAN WASHINGTON: Well, we'd make sure

you weren't involved, and, as I say, it's a good point. But that's why I'm saying this is helpful because most Board Members haven't seen it.

DR. GOERTZ: Christine Goertz. Just one thing is that we have stated, you know, at this meeting and publically a number of times that we would do this sort of analytic assessment and also have listed, you know, some of the criteria that we were likely to consider. So, it's not like it's coming completely, you know, out of the blue. We have already provided that information.

The second thing is, I mean, I'm actually not in disagreement with Sharon-Lise or Carolyn, but, I mean, an argument could be made that if I know the type of research I'm likely to be funding or be submitting in the future in the next round, I could introduce the same sort of bias even if, you know, the applications were to come in the future.

So, I just think this is an important issue, I think it's something that we need to think about very carefully, I think this was a great discussion, and I think we can move forward, you

know, thinking about this from a little bit broader perspective than we were before.

CHAIRMAN WASHINGTON: Sounds great. So, Leah and Rick, we're going to ask you if there's another part and to wrap up this discussion.

MS. HOLE-CURRY: I just had one other consideration as we're moving forward and that's just a very basic practical one in terms of timing and if we do open it up for comment we need to be responsive to it and I think that's appropriate, but that also takes a lot of resources to do that. So, I just ask that we keep that in mind as we try to design it.

DR. GOERTZ: Good point.

CHAIRMAN WASHINGTON: Rick.

DR. KRUMHOLZ: Thanks, Gene. This is Rick Kuntz, Board Member. First of all, just as a Board Member, it's really exciting to see these milestones being met and the progress we've made over the past year, and, again as a Board Member, I think we're really fortunate to have Carolyn and Arnie heading up the National Priorities and just

bringing their expertise to the table and getting the job done, same with Harlan and Leah. I mean, there's just been a lot of work done by this group and I also want to thank Joe and Anne in really mustering the staff, and this has really been changing the wheels on a moving car very quickly over the past three to six months.

And I am excited about this progress. And then in addition, I think the work that Christine and Gail have done in this and the Pilot Project grants, as we've just discussed here, is also a great milestone for us and I think it's very obvious to the public that we're very serious about what we're doing here, that this is a serious research institute that's addressing cutting edge and current issues and that we work by the primary principle of transparency as much as possible. So, again, I just want to thank my colleagues in closing and it's really exciting to establish these milestones and look down the river.

CHAIRMAN WASHINGTON: I want to join you in thanking our colleagues.

[Applause.]

CHAIRMAN WASHIGNTON: Dr. Gabriel, if you don't mind, we really do need a real break before we turn the floor over to you and your colleagues to get the report. So, ten minutes?

DR. GABRIEL: That would be great because I see a couple of my colleagues aren't here yet, so that's perfect.

CHAIRMAN WASHINGTON: Four o'clock? Ten minutes. And thanks again, everyone, for a very stimulating and informative discussion just now.

[Recess.]

CHAIRMAN WASHINGTON: So Sherine, do you have another colleague who is coming to join you?

DR. GABRIEL: Well, this is Christine's seat -- oh, she's moving over there. Jean, Jean Slutsky was going to sit here.

[Board Members reassembling.]

CHAIRMAN WASHINGTON: Okay. The next presentation will be a report from the Methodology Committee, which is chaired by Dr. Gabriel with a vice-chair as Dr. Normand and we have two -- three

Methodology Committee members here and Dr. Gabriel will make the introduction.

DR. GABRIEL: Okay, well, thank you very much. I'm actually going to ask Jean to come up here and sit with us for, you know, moral support here, but we'll be hearing from all three of these individuals and I think Dave Flum from the Methodology Committee will also be calling in.

Just by way of introduction, we're going to try something just a little bit different with respect to our progress report this time around. Rather than provide you details on where we've been, we're going to talk a little bit, share a little bit with you about what we're thinking about and where we're heading and how we're hoping to approach particularly the introduction of our methodology report, and you'll hear a little bit more about that later.

But I did want to bring your attention to the briefing materials that were sent ahead of time that do have all that detailed information about what we've done and where we've been. In

particular, if you haven't looked at them, you know, I'd encourage you to look at the detailed PowerPoints that really itemize the activities of each of the working groups, talk in more detail than I'm going to share, I'm going to share some highlights, but your briefing materials talk in more detail about who the contractors are, what their scope of work is, what their timelines are, et cetera.

I would also ask you to take a look at, if you haven't already, our first RFI that went out for information from the broader community regarding the translation table. As we hear at these meetings over and over and over again, it's really not about us, but we're -- all of us, but we're really a vehicle to pull in information and content and expertise from a much broader community and our RFI is a means to do that.

Also in your briefing materials is a template, a voting template, and it's a tool that we've developed that we'll use in April. Once we get all of the input from all of the contractors we

have to have some way of synthesizing it and putting it all together in a meaningful way for the report. And we're working with the contractors so that when they produce their work, they produce it in alignment with a template and then that will make it, hopefully, easier for us to assemble it all in our first report for May.

So, all of that is in your materials and I encourage you to take a look at it.

At the end of our comments today there will be a brief discussion about what's -- progress with respect to the definition. Dave Flum, who's led the definition workgroup, is going to lead that discussion and Jean will pipe in also.

But in your briefing materials, again, is the NORC report and summary and I'd encourage you to take a look at that because there's lots of useful information there.

So, that's just a preamble. Again, I'm going to share just highlights of the work that we've been doing and in particular today we're going to spend most of our time really previewing

for you a case study approach, it's an approach that's still in development, so the details are going to be increasingly worked out over time, but based on information that we learned in part from the definition working group, focus groups, and others, it became clear that it was important for us to draw a clear line of sight between methods -- having the right methods, using the right methods, and producing trusted, high-quality, useful information to help people make healthcare decisions, and that line of sight, based on the focus groups, isn't as clear as we might want it to be in the public's eye, and so we have developed -- we're in the process of developing this case study approach to help make that clearer, and Robin and Mark will share that with you in a couple of minutes.

Yes?

DR. WEISMAN: Just a quick question. Because you mentioned what was in our briefing package and the NORC report was excellent. Is that going to be on our website as a question or is that

for Board eyes only? Is that considered a public document?

DR. GABRIEL: That's a good question. I don't see why it should be for Board eyes only. What do you think, Joe?

DR. SELBY: No, I completely agree. There's a lot of interest in it and I think also it would be good to hear when we think our response to that material will be, what's the timeline for being able to post that.

DR. GABRIEL: And again we have --

DR. WEISMAN: I'm sorry, we've talked a lot about patient engagement and how we've been doing this along the way, and that's just a beautiful example of how that was conducted in a rigorous way under the supervision of the Methodology Committee, and I think it may give the public an understanding of some of the issues we've been talking about.

But I had another question about this public thing because one of the intents that you hit on some of the contracting that you've been

doing is publications as well, and are you worried at all about, you know, preempting publication, or do you think it's not a big deal to be doing this? That's sort of why I was asking this question, too.

DR. GABRIEL: Well, let me just speak to your first point. So, I think you're absolutely right with respect to the NORC report. It really is a prototype for, I think, how this can be done and there will be very clear examples of how input that comes from in from the broader community really changed our definition and changed our thinking.

But the publication question, yeah, that is an expected deliverable, so what the contracts show is that the work that's going to be done by the contractors is really owned by us because the contracts are very specific, they're not nearly as open-ended as the grants are or will be, they are helping us fulfill our charge. And to do that we're on the phone with them every week or every other week to make sure that what they're doing is aligned with what we need to produce.

And, so, the end product is owned by us but the publication is really an expected deliverable.

So, just in terms of what I'm going to share, a little bit of an overview of how we're structured, I know you all know that, highlights of what we've done with respect to contracts. I'll mention a little bit about our Methodology Committee Board engagement activities past and future, and then really I'll turn it over to Mark and Robin for this case study preview.

So, again, this is how we've organized ourselves for the first methodology report. So, this organization and these subgroups may very well change after May. We'll be reconsidering that in May and may very well change, but this is how we've organized ourselves, and I think you've seen this before, to produce the methodology report and the translation table.

Just in terms of a high level summary, there are a number of resources that helped us along the way since we came to the Board last.

Lori Frank has been an incredible asset and we're just delighted that she's joined the PCORI staff, but we've also brought on interim researchers, Tim Carey, Ed Reid, and interim contractor who have been very helpful. We've also brought on a medical writer whose name isn't on there but you probably could tell me.

We've awarded a number of contracts. Out of a total of 48 submissions to our calls we've awarded 15, as you can see there, in the three areas, and as I think I mentioned in my preamble, we hold bi-weekly check in calls with the contractors just to be sure that the work that they're doing aligns with our needs and to answer any questions they might have.

The RFI is posted for development of the translation table. We do have a couple of workshops planned that will be on March 6th and 7th right after the Board meeting in Baltimore, so we invite any interested Board Members to please join us for those.

And, again, those are for the contractors

to share what they've found with the larger group in order to facilitate writing our report.

And this last item is one that really grew out of -- I think it was something that Steve Lipstein suggested or at least it came out of discussion at this Board -- that we really ought to, sooner rather than later, this is something we were thinking of doing in the second round, talk about the electronic data systems and really gather information from a broader community about how these can be used for PCOR.

And, so, 34 interviews have been conducted and, again, if you look at your briefing document, there's a lot more detail as to who was interviewed and why including government, commercial, and academia, to really help us understand how the EHR and electronic data systems can be better leveraged for CER and CPOR and PCOR. Those are ongoing.

To give you a sense of where these awarded contracts are, we do have reasonable geographic diversity, and also, even though each of these points to a certain institution or group in a

certain location, I think without -- almost without exception, every one of these groups has reached out, at least locally, and sometimes regionally and nationally, to bring in others into the work.

So, again, 15 contractors from around the country and you can see the color code there with the green being evidence for eliciting the patient perspective, one of our work groups, methods for setting priorities in red, and the guidance documents for selected methods in blue.

So, a couple of slides on the Board of Governors' engagement and then we'll turn it over to the team here. We've worked to engage the Board in a number of ways to try and accomplish these and other objectives and we always look for better and more transparent ways to engage the Board in everything that we do.

So, we've established liaisons to the patient-centeredness working group, so thank you to Ellen and Gray for joining us on our telecons and participating in some of our other activities. Of course, we participate in at least five bi-monthly

Board meetings. She even has the number of direct hours of interaction, I like that. We're all counting hours -- provide import regarding the Research Agenda and Pilot Projects and, again, thanks to Harlan and Christine for providing us opportunities to do that.

We shared the highlights of the electronic data task and solicited about six candidates for interview, again, referred by Harlan Weisman, Rick Kuntz, Steve, and Harlan K.

At Mark's direction and with his leadership we've orchestrated two out of three -- the next one is going to be in about two weeks -- two out of three teleconferences to engage the Board in a discussion of what this report ought to look like. We did not want to be in a position where we're creating the report, we're providing guidance on methods, and you see it at the same time as it's put out for public comment, or almost at the same time, so we've orchestrated these calls to really give individuals an opportunity to dive a bit deeper into the outline and exactly how we're

building this out as we're doing it.

The third call, which will be in a couple of weeks, will really focus on helping flesh out this case study approach in more detail, so if you like the approach and you want to help us flesh it out in more detail and want to provide some more granular comments, please do join us in a couple of weeks.

Obviously, we submit briefings to you, submitted 11 to date, and we've invited Board Members to participate in reviews of the contractor proposals and are grateful to those of you who helped us with that, so, Leah, Harlan W. Debra Barksdale, Rick, and Steve Lipstein.

In terms of what we want to do next, again, continue these teleconferences. I think there will be a total of just three, so one more of those to go, to talk about the report. We have a communications plan that we're in the process of finalizing to share the methodology report in the period just prior to public comment. So, again, the work is mostly with the contractors at present.

We've developed a tool that will help us gather the product of their work in a systematic way. We're going to be meeting on April 2nd or 3rd or something like that synthesize all of that information, and then once we have it, we will look at it and share it with you at the same time and then, again, bring all those comments together. We will have a voting process on the submitted recommendations and then come up with a methodology report at the end of May that will be, at that point, ready for public comment.

And so, there's, again, more detail on that to come. We'll continue to provide opportunities for our liaisons in the patient-centeredness working group and, again, of course, continue to provide briefings and status updates and present during the Board meetings.

CHAIRMAN WASHINGTON: Sherine, could you go back to this slide, please, Dr. Gabriel, and just hold for 30 seconds. I want Board Members to absorb this slide and see if you have any questions, comments.

DR. GABRIEL: This one or the previous one?

CHAIRMAN WASHINGTON: This one. Yes, Gail, please.

MS. HUNT: Gail Hunt. Board Member. Are there opportunities besides the two Board Members that you mentioned for people to -- for Board Members to participate in the patient-centeredness working group or is it sort of limited to the two people you mentioned earlier? I think it's Gray and Allen.

So, when you're talking about that next slide.

DR. GABRIEL: Yeah, yeah, yeah. There are -- I mean, you are welcome to participate in virtually anything we're doing, and so there's nothing to preclude more participation. We want more rather than fewer voices, and so if there's interest in, you now, the patient-centeredness working group teleconference schedules, you're more than welcome to see that, I think it's public, and to join us.

I think we wanted to have two individuals who are sort of held accountable for making that connection and so we could bug them if they didn't call in, but that's not to preclude input from really anybody who sees something we're doing where some input or guidance from a Board Member would be helpful.

And that group, I don't think we have --

MS. HUNT: Thanks.

DR. GABRIEL: -- membership from that group here, but I'm sure they'd agree.

DR. NORQUIST: I just want to say that it's been a very good experience for me to interface with that group. I mean, it's a wonderful group -- I mean, not that we didn't know the Methodology Committee was not a wonderful group, but I just want to say that being involved and actually listening to their -- it's every Monday from noon to one, if you want to -- well, maybe that's central time, but --

DR. GABRIEL: Yeah and we can send --

DR. NORQUIST: -- so, I mean, there's

plenty of opportunity to do stuff but, to be honest, it's very helpful to come from the community outreach and engagement and be a part of this group because it really does help to see the interface there.

CHAIRMAN WASHINGTON: Please.

MS. NORMAND: Sharon-Lise Normand, Methodology Committee member. I just wanted to emphasize, Gail, with your question, that this March 6th and 7th -- 5th and 6th or -- 6th and 7th meeting, the patient centered working group is part of that and, so, in particular, if that's something that you're interested in attending --

MS. HUNT: I've already got it on my calendar.

DR. GABRIEL: That would be a perfect opportunity because you'll hear from all the contractors, you'll know what they're doing, and it will be an opportunity to distill that information.

Leah, did you have a comment?

MS. HOLE-CURRY: Leah Hole-Curry, Board Member. I was interested in the communications

plan that you mentioned and wondered if whether at a future Board -- I feel like we're all running -- sprinting in parallel. I know you all have felt that pressure for some time as well. But I think that I would like to see those arrows going both ways. I've really appreciated you and Sharon-Lise's participation on the Program Development Committee and the opportunity that I had to come present to the Methodology Committee about an update for the Research Agenda, for instance.

So, I'm wondering if the communications plan, at some point, could be a vehicle for us to discuss kind of those opportunities and ensure that we're maximizing them.

I know it's always going to be a tension between how much time each individual person has and where we want to spend it, but just having a little bit more of a strategic view of, here are kind of all the activities, if everyone could plug into two that cross over, or something, I think that would be really great. I'm not asking for it now or that you take responsibility. I'm just

saying, that communications plan triggered for me that that might be a place we can start.

DR. GABRIEL: Yeah. I agree, and actually our communication plan there, what we refer to refers to communication for the writing of the methodology report.

MS. HOLE-CURRY: Makes sense.

DR. GABRIEL: So, how do we make sure that before we get that first report even out for public comment, we have appropriate touch points, frequent touch points with the Board?

But I completely agree with the notion of generalizing that idea. And we've also been, kind of separate and aside from that, having discussions about what would be the best way to engage given everybody's time and we'll be coming back with some ideas there.

Okay, so now really we wanted to use most of our time today getting input from the Board about this process that we're, again, still a process in development, but a process that's hopefully going to lead us to draw some clear lines

of sight between the critical role of methods and producing trusted, high-quality, useful information that people can use for healthcare decision-making.

That connection isn't perhaps as clear as it ought to be even within the broader community and certainly in the public, and we see that as an important prerequisite, really, to appropriate use, appropriate adoption of what we're going to put forth in the first methodology report.

And, again, we're looking at this as an approach to perhaps communicate those messages a bit more effectively than, you know, in typical prose, and just wanted to share it with you while it's still in evolution to get your comments and input on the approach, not so much the detail, and hopefully to entice you to work with us on our next call to flesh this out further.

So, with that, I'll actually ask Mark, to introduce the topic in a bit more detail and then Mark and Robin -- Robin and Mark, will share two cases that they're working on.

MR. HELFAND: Well, I'm Mark Helfand and

I'm on the Methodology Committee. It's getting closer to the time that PCORI will start soliciting or choosing patient-centered questions to study in research studies, and as we approach that time, I think recognition will take shape that what's going to matter to patients and clinicians just as much is how good we can get at designing and carrying out studies that will answer those questions.

As a researcher, but even more as a physician, I can tell you that methodological decisions directly affect people. They affect what you're told or read about a condition, what you and your doctor can find out about different treatment options, and what your doctor believes and recommends. And when researchers make the wrong methodologic decisions in research studies, patients can be harmed.

So, today we're using a couple of case studies to illustrate how attention to the best practices in formulating better research questions and carrying out investigations will help PCORI achieve its greatest return on its investments.

And we've chosen four of the two dozen or so methodologic dilemmas, the Methodology Committee's identified as its priorities, for its first reports, which will come out later this year.

And this is called a preview because the right way to do a case study is interactive, discussion and so on, and that's what's going to happen in the call that we are holding, the third of the series of three calls, the next call in a couple weeks will be that opportunity to interact on a case study that way.

Today we're just going to kind of run through a couple and then afterwards, hopefully, there will be some questions and discussion, but it takes hours, not minutes, to really go through one of these right.

So, with that, I'll introduce Robin, who's going to talk about the first of the case studies. Can we have the next slide? These are, I mentioned, four of the areas, and these are the four, roughly speaking, that we'll touch on today.

MS. NEWHOUSE: Robin Newhouse, Methodology

Committee. And I'll start with a scenario about Ben. Ben is a 78-year-old Caucasian male. He lives about 30 miles from the nearest hospital. He has a diagnosis of heart failure, and like most heart failure patients, has a complex disease, so he also has diabetes and he also has renal failure and has to go to outpatient dialysis three times each week.

He lives in a home with his wife and they have a two-story home. Unfortunately, he can't go up the steps to his bedroom anymore so he's been sleeping downstairs.

Ben's been admitted to the hospital four times over the past year and four times he's received discharge instructions to help him take care of himself at home, and four times he's come back to the hospital.

The fact of the matter is, Ben has experienced symptoms in his home that were cues that should have indicated that he should call the doctor, but did not. So, he experienced symptoms like fatigue, weight gain, dyspnea, that would be

something to indicate that his condition is changing.

So, this was the second time he had been readmitted after missing a dialysis appointment because he was just too tired to go.

So, for Ben the question is, one of the PCOR questions is, how can the clinicians and healthcare system they work in improve chances of achieving the outcomes that Ben seeks? So, there are two methodological issues I'll focus on. The first is, we need to pick the right outcome. The second is we need to use the evidence to inform the studies, the study design, the study interventions, that we use to understand the experience of the people to improve their health.

So, the first issue is, getting the outcome right. So, anybody that's done work in heart failure probably has used readmission as an outcome at some point, and readmission is an important outcome for us, but usually it's regarded as a system outcome. We assume that a patient doesn't want to go back to the hospital and I think

we'd all agree that it certainly is an important outcome, but people that are being discharged are not too eager to come back to the hospital again, so they'd like to stay out of the hospital.

But maybe it is a surrogate outcome for things that people care about. But the truth of the matter is that what's important to Ben is that he can function, he can engage in social activity, and he can maintain his health as long as he possibly can, so readmission may not be the highest priority outcome for him.

So, when we think about what outcomes should we study for heart failure patients, we need to start thinking about how we can engage the patients and understand what are the outcomes that are most important to them. So, people will benefit most when we study the outcomes that we care about, so the methods that are associated with how do we engage the people that have heart failure, one of the ways the Methodology Committee is addressing some of those standards are through the two contracts that are doing some synthesis

about patient engagement and additional patient interviews about engagement, but I also want to mention too in terms of methodology, the outcomes for Ben might be different than outcomes that we study for patients in urban centers.

I mentioned Ben lives about 30 miles from the closest hospital in a rural setting. When we think about engaging patients in rural settings, we're talking about settings that are very remote, it's very difficult to recruit patients in these rural settings. Many -- 41 percent of our acute care hospitals are rural hospitals and, in fact, many of those rural hospitals don't have an affiliation with an institutional review board, which makes it very difficult to train the staff, physicians, nurses, and others that are engaged in the research study.

So, we need to address these methodology and ethical implications of how can we enroll these vulnerable populations to determine in fact what outcomes are most important to them?

The second thing about getting the outcome

right is, these key measures have to be of practical concern to clinicians. So, we want to study the right thing, but we -- clinicians want to study the right thing that are important to patients, but they have to be important outcomes to clinicians too.

We have lots of efficacy studies that don't necessarily tell us a lot about what we can implement and practice. So, we need to spend more time determining what we can implement and practice and the outcomes that are important to providers as well. There's little benefit to studying the same thing over again and the same outcomes, but the studies that are important to providers have to have a population that's similar and a setting that's similar to the patients that they see.

So, that's the first point in terms of getting the outcomes right. The second point is, the proposed study should take advantage of comprehensive reviews of the literature to inform the design, the interventions, and the outcomes of interest, and the methods as well.

Here's another example, a heart failure example. So, depression has a very high incidence in heart failure patients, very prevalent, and yet not many studies include depression screening as a measure, either to risk adjust or to determine the effect on the outcomes of care. So, the studies that have used depression do indicate that outcomes are worse for patients that are depressed. So, there's some effect of depression.

So, wouldn't that say, first of all, when the evidence is pretty clear that there is an effect with depression, that shouldn't there be variables that should always be included in these studies that are funded, number one, and when studies -- prior studies have flagged problems or problems with enrollment or treatment failures, we need to better understand the origin of those treatment failures to understand the interventions and design better implementation strategies, otherwise we're redoing the same studies over and over again.

The next point I'll make is just about

complex interventions. So, there have been synthesis to evaluate the literature related to care coordination and the best care coordination is around all of us working together -- nurses, physicians, social workers, nutritionists, psychologists -- really on a common patient-centered goal. And care coordination activities, there are a lot of different models out there, but the fact of the matter is that it works pretty well for heart failure patients, particularly if there's a direct intervention with the heart failure patient.

But the problem with these studies is they are complex interventions. We don't always know the exact part of the intervention, and there's not enough known about what the intervention actually consists of. For example, when we think about a team approach, we don't actually know what the relationships were on the team, the roles and functions that the people performed, how often that that care coordination occurred, sort of, what's the dose, what happened during that care

coordination.

So, there really is a need for some clear standards of reporting these complex interventions and need for studies to understand what pieces of this complex intervention are absolutely essential that can be replicated in other settings.

And then in terms of replicating in other settings, taking it to the rural example, when there are care coordination activities, if we have a bundle that can be identified and we know that it works in one setting, it has to be taken to the rural setting, which probably is going to require a fair amount of tailoring, so we're going to have to not only include efficacy studies and effectiveness studies, but really understand how these interventions can be implemented in these settings where we have very different kinds of providers and different kinds of patients.

So, the bottom line is, just two methodological issues, one, the outcomes, and the second, informing our interventions, our design, and our methods based on a comprehensive review of

the literature. And the likelihood of producing results that will help people will be enhanced.

VICE CHAIRMAN LIPSTEIN: Can we comment?

MR. HELFAND: No, we're going to wait. We're going to wait. Okay. And just on the time, we did start late. If we run out of time and you need flexibility, I don't know if you'll be here tomorrow, I'll be here tomorrow, we could schedule something for more questions.

DR. GABRIEL: Dave Flum is calling in in a little bit to talk about the definition, which is our next item --

MR. HELFAND: Well --

DR. GABRIEL: -- but I don't want to cut your time because this is important.

MR. HELFAND: Yeah, so we'll do what we can. I'm sorry, but, yeah, just to try to --

VICE CHAIRMAN LIPSTEIN: [Off microphone.]

[Laughter.]

MR. HELFAND: I'm not answering that question. So, anyway, the second case study concerns depression too, and yours did in a way,

and so we'll talk about Mary, for several months who's had feelings of unhappiness and pessimism and low self-esteem, loss of interest in activities that she usually enjoys, and she's tried to cope, but now after some more time she has insomnia, early morning awakening, oversleeping, difficulty concentrating and remembering, and she goes to her primary care doctor who prescribes an antidepressant.

After a week she switches to --

CHAIRMAN WASHINGTON: I'm sorry, Mark, but give us her age.

MR. HELFAND: I will in a minute, I will later.

CHAIRMAN WASHINGTON: Okay, I'm sorry.

MR. HELFAND: It's a great question, but it kind of ruins the punch line later on.

[Laughter.]

MR. HELFAND: What -- she's 45. After that she switched to another antidepressant after a week because of side effects and then after a couple more weeks she told her doctor she's not

feeling that much better and wants to know if there's something else she can try, and after discussing the situation, she decides to give it more time.

And over the next few weeks there's improvement in some of the problems she's had, less feelings of worthlessness and guilt, better energy, basically, a little more enjoyment of activities, but the insomnia is worse and she's anxious when she wakes up and as the day goes on, and eventually she and her doctor agree it's time to try something else.

We have four topics here. Since Robin talked about getting the patient's voice into selecting outcomes, I'm going to not say as much about it as I'd like to, but that point also says outcomes and comparisons, so I'll say a word about the comparisons.

The doctor takes the time in this situation, let's say, to discuss some of the choices -- switching to another SSRI antidepressant or another type of antidepressant or stopping the

medication and using a kind of talk therapy, a cognitive behavioral therapy, and there are several other options as well. But the doctor says, we don't really know much about the choices, the patient's questions, what's the best choice in the long run? We don't know much about that.

What about switching to a very different kind of antidepressant? We don't really know how that compares in the long run. How well does the cognitive therapy work compared with drugs at this point? We know something about it but not as much as people would like to.

And so, the doctor is giving very honest answers and the answers that Mary might get looking on the web and asking other people might be similar.

So, here's the thing, besides getting the outcomes right, to be patient-centered, research also needs to get the choices or alternatives to study right and this isn't easy when there's a lot of different alternative treatments, a lot of diversity among patients regarding what options

they're interested in, and new information and new treatments becoming available all the time.

So, choosing comparisons for study is also a methodologic challenge and a logistical one and the Methodology Committee hopes to contribute approaches to do it well and transparently.

So, I'll move on to existing evidence. Robin also spoke a lot about existing evidence but there's two more points that need to be mentioned, one in particular for treatment-resistant depression, and the first is one -- and these are -- the first is a problem of data we can't even get. And Robin emphasized the importance of a systematic review of previous studies, but if the previous studies aren't easily available, doctors and the public can have the wrong information. And usually your doctor's estimate of the chance of responding to something, whether it's a medication or a different kind of treatment, or of having a positive or negative -- false positive or negative test, depends on whether there's unpublished data that might help us fill out the chances correctly.

More importantly, when a review is based just on published studies, some harms might be unknown or not widely known enough and one of your members, Dr. Krumholz has asked in an article, do you really want your doctor to know only part of the story about a therapy that might be recommended for you, and called the problem of this kind of missing data, medicine's biggest threat.

There's another threat, too, I want to mention and that's when researchers don't pay enough attention to what we already know from studies. And when I say threat in this context all I mean is, hazard in trying to design a research program. And I'll use a surgical example because it was studied so well and not a depression example, so let me shift gears for a second. We're talking about a drug for controlling bleeding during surgery that was studied in 64 trials over five years. And the question those trials asked was, how well does it control bleeding, especially in cardiac surgery patients?

And that question was answered numerically

and definitively by the 12th study, but another 42 were done, more studies kept coming of the same question. So, think about this. Patients were enrolled in some of which were placebo controlled trials of a question that had already been answered definitively. And what's a more important question, how do the patients do in the long run, was neglected in most of those trials. Later the drug was found to have a down side when bleeding's less -- you know, when you stop bleeding you always tend to increase clotting and so you have a trade-off of the risk of stroke in these cases.

So, Ian Chalmers wrote an editorial about this phenomenon. The article is the most recent evidence of an ongoing scandal in which research funders, academia researchers, research ethic committees, and scientific journals are all complicit. In applied fields like healthcare, failure to prepare scientifically defensible reviews of relevant animal and human data results not only in wasted resources, but also in unnecessary suffering and premature death.

Now, this phenomenon -- this is a great case example. It's not that it -- we'll get back to depression in a minute, but the phenomenon of not citing previous research, in these studies the average was citing two previous trials even though they were accumulating by the dozen over five years.

It's not an uncommon phenomenon and in a study across a number of different areas, the phenomenon of this sort of under-citing or keep doing the research without really saying what's happened before was found to be pretty common. We have one more slide, which -- I won't -- if you've got the other slide -- I won't read it, I'll just give you a second to look at it.

The point I want to make, really, is not to get into threats and scandals and all of this language that you see in publications about this, but to say it's not patient-centered to neglect or misuse previous studies in designing the next research study, and it's a methodologic issue because finding and using all the data doesn't just

happen. Standards and methods for doing it are needed to make it happen. It's not just a methodologic issue, it's an ethical one, there's many other aspects to it, but it's an example of how important using existing evidence really is.

Now, the remaining issues, the choice of study design and determining how treatments affect people differently, are a bit more technical and so let's go back to our case for a minute, and Gene, this is what I was holding on the age -- new data were published not long ago, I think October, noting that -- from the NHANES survey -- saying that one in every ten Americans twelve years of age and older take an antidepressant. But only about one-third of persons with severe depressive symptoms take one.

And 23 percent of women in their 40s and 50s take antidepressants. And more than 60 percent of those taking antidepressant medications have taken it for two years or longer with 14 percent having the medication for 10 years or more.

So, it's somewhat amazing that Mary

doesn't have more to go on when choosing an option at this point. There's all that experience out there. But it is true, and I'm going to quote Dr. Krumholz again, he has a way with these issues, "I'd like to let you in on a little secret in medicine: we know a lot less than you might think about the effects of" -- he said medications, but I'd say all treatments, "and in particular how they compare with one another."

So, methodologically we have to say, how can this be when there are millions of patients out there with records and information that we seem to be under using? One would imagine that, over time, knowing what even 1 percent of people taking antidepressants and other treatments or not treatment, like many of the people with severe depression, over the long haul we could provide a lot more for Mary and her doctor to go on, but the methodologic challenges of using this kind of experience, making reliable conclusions from it, are great.

And so now we're talking about selecting

the right study designs. We're talking about much more than that, but that's really where we are now.

Now, the best known example of this study design issue, which I'm sure you're all familiar with, is the hormone replacement story where, based on observational studies, and doctors believed that hormone replacement prevented heart attacks, then a big randomized trial came out and said it's quite the opposite. But that came out many years later, after, I don't know how many hundreds of millions of days of treatment.

And so, what I want to get at in selecting the study design issue is that there's a lot of good news. First, there's better methods now. The observational studies of hormone replacement therapy on which those earlier conclusions were based, have been reanalyzed using better statistical methods and probably would have given the right answer early -- not just earlier, at the time that they were giving the wrong answers, if indeed they are wrong, using methods that weren't available at the time those studies were published.

And so that's one bit of good news.

And the Methodology Committee is looking carefully at these new options for analyzing data, observational data, because if they prove reliable and applicable, standards that incorporate them could make a huge difference.

And second, PCORI generally is trying to also look at the source. If the data can be better in the first place, they'll be more valid when they're analyzed, and the Board has had discussions of that and so have we.

And third, and, again, this is particularly with respect to treatment-resistant depression and our case study, we're in a great position now to get more from observational studies on this particular issue because we have something of a benchmark for what happens in everyday practice from a previously done large, and I will say, very expensive trial conducted by the NIH. And from it we have a pretty good idea of the effectiveness of some antidepressants and some other strategies, and so when we conduct -- if we

go forward with observational studies of, let's say, a new treatment or a different approach, if we do better than a 30 percent remission rate at the second stage that Mary's in, we're probably on to something and we can have more confidence in that from an observational study than we would have if, in this case, NIH hadn't pulled out the big guns and done the big trial.

So, one thing about this area of methodologic innovation and development in observational studies is, it should give us more confidence, too, not only about using observational data, but when it really is necessary to do a large, randomized trial, so it helps both ways. It's very tempting to take the question, but --

DR. WEISMAN: [Off microphone.] -- because I'd like you to consider it because everyone throws it away as useless data, and that's claims databases and other things.

MR. HELFAND: I know.

DR. WEISMAN: I have a small informatics group and this very question on depression, we just

ask very simple questions and duplicate it in several different large claims databases including one that's very good, the Thompson Financial one that the multinationals work at, use it for hypothesis testing and other things, not for publication, not for conclusions but, boy, you can get a tremendous amount of information on the amount of switches are done over what period of time, things like hospitalizations and, you know what, it's holes all over the place, all kinds of problems with it, but you're dealing with hundreds of thousands to tens of millions to hundreds of millions of lives, and when you're doing that, signal starts rising, noise starts going down, and I'm not saying it's great quality stuff, I know that you guys -- lots of people hate that kind of stuff -- but in terms of exploratory data analysis and looking for something, I think it's something you guys ought to think about as we try to refine those databases to have more data.

I don't know whether -- you talk to one of the people that was pulled was somebody who works

for me who does this work, but it's incredible what you can find out on this, just from a hypothesis standpoint.

MR. HELFAND: I'm going to hold off on responding because I did -- I'm being told that we do have to wrap up.

So, the last point about determining if -- how treatments affect people differently. One of -- another interesting thing about the NIH, the Star D Study that I referred to, is that it doesn't help a lot figuring out how to individualize decisions. It's still a big question, and a lot of methodologic research recently has attempted to look at this issue of how much -- would it be worthwhile to go further into these areas like diabetes treatment in the elderly and treatment-resistant depression, looking for how different people respond differently to different approaches, and there is a lot of potential value in that.

And so, I did want to say a word about the methods here. This is not a simple matter of there's no methods, this is an example of where

there are some methods. They're not as widely used and we, as a committee, as well -- and not everyone on the committee is that familiar with them -- and so we're looking at these methods for dealing with what's called heterogeneity of treatment effects.

But I'd say that the -- this is on the plus, the optimism side, it isn't that there's no ways to do this, it is right at the vanguard now to do it more not only in observational studies, but in trials as well.

So, just to wrap up, it's easier to see how methods to bring in the patient's voice or get all the data out in the open improve the lives of patients and the lines of communication among caregivers, patients, and doctors. It can be harder to make the connection with some of these more statistical or other methodologic issues, but the stakes for the patients on those issues are just as high and bad choices for methods such -- for these kinds of things, as well as issues such as handling the data from patients who leave studies early or methods for following them up or

for collecting information or for taking account of differences, those make a big difference in what options people are offered in real life and whether the information about those options is close to true or way off.

So, the good news, this Methodology Committee is taking a look at a lot of approaches, innovative and standard approaches, that were developed for other types of research, haven't maybe been used as widely in this area of patient-centered outcomes, and really trying to find the -- you know, the sort of fit for what PCORI is supposed to do.

And, last point to make, the methods we propose are probably going to be a mix of the proven, the promising, the innovative, and we're going to say, "we don't know, let's figure it out," when we don't know, and the methods are designed to work best in an environment of open-minded research that's not hidden, abandoned if we don't get the answer we want, and that open-mindedness of sort of the research program is probably the thing that

will make this work the best because that's really what, when you talk about all the compromises and decisions you make along the way, if they fall in the direction of open-mindedness and responsiveness to the needs of patients, caregivers, and clinicians, you're probably on the right track. Thanks.

DR. GABRIEL: So, thank you. Thanks very much to both of you. And again, for the Board, what we're trying to do is sort of invite you to join us on this path where we're trying to identify ways to communicate more effectively, perhaps, the importance of methods to improving -- helping people make better decisions and at the end of the day improving patient outcomes. And using examples like you've just heard of how choosing the wrong methods can actually lead to patient harm or at the very least keep people from getting treatments that are known to be effective, using that as a platform to explain how PCORI and the PCORI Methodology Committee is going to contribute by ensuring that the right methods are used in promulgating

methodologic standards that help us to ensure the right methods are used and therefore we get the right answers and, you know, prevent the harm that might result from using the wrong approaches.

So, again, input on what do you think about this as a way to communicate these messages in this report and perhaps in the future? And try and get you interested in joining us two weeks from now to really get the details of this sorted out.

I'll stop here. We'll talk about the definition maybe after we take a couple questions.

CHAIRMAN WASHINGTON: Why don't start with Steve, since you didn't get --

VICE CHAIRMAN LIPSTEIN: [Off microphone.] Harlan, they took it away from me. See that?

But the -- I think making methods patient-centered is very important and using examples like Ben -- and I forgot the name of --

DR. GABRIEL: Mary.

VICE CHAIRMAN LIPSTEIN: -- Ben and Mary -- I think is a great way to make this real and very -- because many people can identify with Ben and

Mary. I mean, I felt like Ben was a patient who lived in the boot hill of Missouri, and so I could relate right away. So, I thought it was very ingenious and creative and a great way to go about it.

CHAIRMAN WASHINGTON: Okay. Sharon, Carolyn, and the Larry and Harlan.

DR. LEVINE: As a non-methodologist, non-researcher, you wound or wove a wonderful narrative, it was actually a very compelling narrative. And very clear. I mean, I assume this is intended for a non-researcher audience, is that correct? Yeah, it's extremely effective.

DR. CLANCY: So, I would agree with that and I just made a notation in my calendar, so to the best of my ability I will be with you at the workshop because I think this is very compelling.

I wanted to raise two issues, not for you to respond to, but just to think about. One is the whole issue of, what if Ben really wants something that doesn't fit our model? So, it could be in your example that actually what he needs more than

anything is one of those lifts to get him upstairs, I mean, that that would actually do more for his outcomes, because I think that's an issue that we have to struggle with.

And Mark, I loved your examples, but I think there is an issue of needing to consider alternative explanations. It may be methodology challenges, it may also be weakness in how we have funded research in the past, so I had a political scientist ask me a couple of years ago how come we don't do enough follow on studies, and I said, well, what do you mean? He said, well, you have all these studies and you guys have supported a lot of them that say, you know, we've got disparities here, there, and everywhere. How come nobody follows up immediately with another study?

Now, part of the answer from our -- to that is kind of instability of funding. PCORI doesn't have that answer. Some of it may be just bias in terms of wanting to fund new stuff all the time, I don't know, but that may be as important as methodology.

So, I think it's just important to think through some of these issues, but I'm definitely looking forward to this. I think it makes it real for people why this is all so important.

CHAIRMAN WASHINGTON: Larry and then Harlan.

MR. BECKER: So, the danger of not knowing anything about what I'm about to say -- here's what I heard. I heard you say that there are few, if any, robust study registries, so that people don't build on other studies in many cases. And so, I go back to sort of the analogy of PCORI setting some kind of a platform and the Methodology Committee being in the analogy, the source code.

So, the way things are done -- and one of those certification source code things are requiring that studies be registered so that people can access those things and have a place where the next researcher along the way can look at those 40 studies that were done that you referenced.

And it seems to me that it's a powerful opportunity to take all of the work that's being

done and -- now, maybe I don't know what I'm talking about, but that's what I kind of heard as a possibility

CHAIRMAN WASHINGTON: Harlan.

DR. WEISMAN: I agree with those who said that they really like what you did. I mean, as a physician I could clearly -- that's what you face when you're treating patients as a patient, that's what you face when you're looking for good treatment, and it could be any number of things, and I think the fact that some of our stakeholders will clearly show that you empathize with what it is that they're experiencing, whether they're physicians or other clinical caregivers or they're patients or personal caregivers, people will get this, that we, you know, as -- that we feel their pain and we're trying to address it.

I don't know the answer to what Carolyn said in terms of how much of this is methodological challenge, although I know that there is tremendous methodological challenge to this. I mean, there's all the ones you mentioned plus dozens of other

complications, but in terms -- we've been talking as a Board about this whole role of could we -- and I know, Mark, you and I have talked about the idea -- could we start setting standards for longitudinal data collection? Because it's not worthless, it tells you what happens. It may not be a randomized trial, but it does provide useful information, and one of the problems is that none of these datasets are compatible with each other.

And Carolyn mentioned earlier about a conversation she had about orthopedic implant registries. I know four of them right now and I've talked to some of the investigators and they don't really care what anybody else is doing. So, if we could do what Larry's saying and set standards, it would be wonderful.

But I think you guys are onto something, bottom line. I liked it.

CHAIRMAN WASHINGTON: We have Bob and then Debra and Gray.

DR. ZWOLAK: Bob Zwolak, Board Member. That was a very nice publication -- comments, but

Robin, when you started out talking about Ben and looking for outcomes that may well be patient-specific, I think that's a fabulous concept and I started to wonder -- then Carolyn mentioned, you know, the chair that goes up and down that they can get up to the second floor -- how likely is it, do you think, we'll come up with testable outcomes?

Are you going to have to make some kind of reporting standards? If you do this and find an outcome, will you have to develop and test reporting standards to see how reportable it is? One of the reasons that people like readmissions is it's a nice, clean, binary, hard data bit. Do you think we'll find good outcomes that we can test and do you have some examples, some real life examples where people may have been successful at that to date?

MS. NEWHOUSE: For heart failure there is a state of the science paper published by American Heart Association and there are many, many interventions that help people take care of themselves at home and manage better. And I think

Carolyn's example was a great example and I didn't go -- this was a teaser, there's lots -- lots of exciting things when you talk about complex interventions and implementation. But that is part of, you mentioned, the reporting standard. We have to be better at understanding what exactly happens. So, you read a -- it could be an efficacy or an effectiveness trial, but you don't actually know what happened, you don't know what the interactions were.

So, we do need some reporting standards to help understand exactly what happened and what were the individual kinds of things that -- what patients did better under this care coordination standard?

So, a lift -- under a care coordination model of any type, perhaps the team would have somebody visit the patient's home or the person's home, and they may recognize a lift, but as a researcher, we have to collect all that material. There are probably some number data and there are some language data that we have to collect to

understand exactly what happened.

So, yes, we need to do a better job, but that is how we will have reproducible results that can be used in multiple settings and help people understand what they have to do and what they can tailor to make it work for their individual setting, and I do think that's a primary goal to help us have data that the people can use and providers can use as well.

CHAIRMAN WASHINGTON: Okay, Debra, please. Then Rick.

MS. BARKSDALE: You know, stories and cases are very powerful and I think you both did a great job in identifying a lot of the issues around use or lack of use of data. I'm trying to understand a little bit about, in your approach, how you use this kind of method to come up with methods, you know, for example in the 65 trials and after 12 the answer was known but they kept doing it, one reason not reporting that these other studies had been done.

So, what would be the role of the

Methodology Committee in addressing that? Would you make recommendations for standards or --

MR. HELFAND: So, I'll take that. I want to say that, you know, there's been comments and questions and I haven't said anything because on almost all of them I know there's several people on the Methodology Committee who know a lot more about it and if I say anything I'll probably be not as right as they would be, but this one's kind of closer to my expertise.

So, you know, people have already tried things and various, you know -- in little ways, either in journals, for instance *The Lancet* says -- if you're going to put a paper in *The Lancet* you're supposed to really summarize the previous literature accurately or use a systematic review that does to avoid that problem that each of these trials came out and only cited a couple others.

From a funding organization's viewpoint, I'd say funding organizations are much, much more interested now than they ever have been and PCORI, in particular, to see that the previous existing

evidence is known well and used well, and AHRQ, in particular has done a lot of work because they commission a lot of systematic reviews, right, to see how can you turn that into something that really gives a picture of the existing evidence so that people will be guided in their future research to not repeat things.

So, from the viewpoint of standards, you know, you could imagine methodologic standards that apply to the researcher that says, show us that the outcomes you're looking at or the comparisons you're looking at, that you're doing something different, that you're not doing -- or we could imagine that there is a set of systematic reviews that PCORI relies on or commissions -- there's a lot out there, they don't have to commission them all -- and sort of see if there's a consistency between what they say is known and unknown and what the research is going for. But I do think it can be handled, at least in part, with standards for conducting research or recommendations to support those standards. I think it falls within that, you

know, doable category.

DR. GABRIEL: And if I could just add a small point. Months ago, I think, Dr. Washington said that the standards that come out of PCORI and the PCORI Methodology Committee ought to be standards for the nation. So, you could imagine, if we were successful in that goal, we could really fix this problem that -- or at least go a long way towards fixing the problem that Mark just highlighted, if we not only put the standards out there but were successful in having them adopted very widely.

DR. NORQUIST: So, what I would say is two things, definitely do the case studies because -- if it makes you feel better, when I was at NIMH we tested this out about human studies because now if you look at most NIMH reports they have a human story in them, so it does make a difference, it gets people's attention. It also explains it to the general public in a better way.

The other thing I would say is that we have -- the thing about making standards is that

you can also help people who think there's no way of doing this kind of study. So, we learned a lot about just studies when I was at NIMH and the problem was, Carolyn, is that -- I don't know if Fran is still on the phone -- but nobody would continue to fund this to really follow, so there were very important things you learned by doing a study.

So, Mark and I talked about this, that one of the things we had, what do you do in a study when people say, I don't want to be randomized to all these different arms, so I'm not going to play the game. So, then you lose a huge group of the population.

So, I always feel good when I can mention this thing, equipoise stratified randomization. I feel like I'm a methodologist. So, you know, they came up with this idea of how you could actually allow people to make a few choices and not all of them, and that solved a problem for us because we had a huge issue in mental health about some people, they don't want CPT, they just want the

drugs or they want whatever.

So, for me there are like all these kinds of issues plus you don't have to have endpoints that you think that are a symptom in point -- you know, our outcome in the CATIE trial, which was antipsychotics, was not an endpoint of whether somebody's psychotic symptoms went down, it was all-cause dropout. And that was a huge issue in our field as to whether people stay on medications or not, but we had to deal with that from a methodological issue too.

So, there's a huge help with this interface when you look at that, but I would hope that whatever studies we do or what we put out to the field is that we should do some work when we do these trials about what happens. There's so much to learn from trials about what happens, and then you go back to the drawing board on it.

CHAIRMAN WASHINGTON: Okay, a couple more focused comments. Rick and then -- okay, and then Arnie.

DR. KUNTZ: Rick Kuntz, I'll try to make

this quick. This is really exciting stuff and you've got such a talented group to look at this and I just want to ask a little question about scope. There are three dimensions that you talked about, one is the patient-centeredness part, the other is control of confounding control of observational improvements and statistics, and the other is this infrastructure of longitudinal follow up.

The first part, in and of itself, is very complicated, obviously, you know, how much does patient-centeredness outcome relate to the path of physiology of disease and when do they start to separate and not separate. The confounding control of observational studies is a big question a lot of people are trying to ask and they're trying to satisfy this with improvements in registries and surveillance databases and so on. And then the other part about the infrastructure of doing longitudinal research is really the connection of HIT and other stuff like that.

Do we -- it's hard to continue to think

about the patient-centered part as we track each of those different dimensions because I think you can get lost in the methods to some degree and I just wondered what your feeling was about that, about keeping scope with the patient-centered side, because you can spend a lot of time on those other issues, especially the observational confounding control part, unless we can all agree that that's a really critical part for patient-centered research.

MS. NORMAND: Sharon-Lise. So, obviously Mark and Robin should respond, but I'm going to say that we put the patient-centeredness focus on every piece of this, so even when we get to the confounding issues -- and it's always placed -- we always have this lens to say, okay, when we go through these case studies we think about the design, is this really a good patient-centered design, so we think about the equipoise preference-based randomized trials, things such as that, treatment heterogeneity, again, what are we looking at?

And so, Rick, I don't think we see them as

two separate things. I think we think of the first one as looking at outcomes and looking at evidence and then looking at the right design and then looking at other things, but at each one of those it's like, okay, what makes this patient-centered? What makes adjusting confounding patient-centered?

I don't know if Mark or Robin would like to add.

DR. GABRIEL: I think you saw that even in the charge of the patient-centeredness group to really look at every step of the research process and put a patient centeredness lens on it.

CHAIRMAN WASHINGTON: Okay, we have Arnie and Christine and Steve.

DR. EPSTEIN: Am I on?

CHAIRMAN WASHINGTON: Name.

DR. EPSTEIN: Oh. Arnie Epstein, Board of Governors. I was going to say a name and then I realized you wanted my name.

[Laughter.]

DR. EPSTEIN: I thought, wow, that was so much harder. Yeah. So, I have some reflections

which -- really for you, but also for the Board. This is -- I have the same feeling as everybody else, this is just sort of a, wow. It made me start to reflect on what do we think we want methods to do. And I can think of three quick things off the top of my head. One is that we wanted to take the opportunity to sensitize patients and providers, broadly defined, including some who run plans, to the fact that methods are variable. Lots of lousy methods out there, and they're part of the problem that Harlan has raised for us, again, about us not having enough information is due, in some part, to methodology, other things too. And this kind of stuff really brings it home really nicely. So, that was really good.

And then I thought of two other tasks, one of which is more grandiose, it's how do we increase or improve the methods in the field more generally? And the second, more close to home -- or the third, second of that line -- is, we're PCORI, what do we do differently because of this?

The first one is an invitation for you to say a little bit about, this is going to really speak to patients and doctors, what are you going to do to speak to the people who are already okay, the Rick Kuntz's the Harlan Krumholz's, who are using methods and make it better? And then the second is really a question for us. At the moment, what we have is a very traditional NIH methods review, nothing better than that. And I wonder whether the opportunities you're laying for us with the kind of expertise you bring should make us think about whether we want to do something more energetic than that.

You're on for at least what you're doing.

UNIDENTIFIED SPEAKER: [Off microphone.]

DR. EPSTEIN: Yeah, in other words, there are three focuses for how methods interact. One is to engage patients and doctors across the country to the fact that methods really matter, and what I just heard from you is a wonderful, wonderful vehicle. Second is how do you produce better methods more generally? It would be easy if you

said you're about to produce the definitive textbook in methods which will show you the 14 commonly -- here's chapter one, 14 commonly used methods we should never use again, or something of that version.

And then the last one is a challenge that I'm trying to weigh, which is, what am I doing differently? Because at the moment we're doing same old, same old.

MS. SLUTSKY: That's a great question and I'm really sorry that Brian Mittman isn't here because he actually gave a fabulous -- led a fabulous discussion early this morning about, okay, now we've got this, how do we disseminate it to funders, researchers, patients. And so, we've just begun to talk about that and it has to be in collaboration with the Board. So, we are thinking about it and it's an important issue.

DR. EPSTEIN: If you could just disseminate it to us. I'm serious.

CHAIRMAN WASHINGTON: That's what I picked up.

DR. EPSTEIN: It might have primary and secondary gain.

MS. SLUTSKY: Yeah, I mean, we're just really at the first stage of that, but he laid out a really elegant paper that we all just saw ourselves this morning, so definitely we're thinking about it, but in the throes of trying to get this draft report out, it's lagged behind. But you've really hit the nail on the head.

DR. GABRIEL: And, in fact, we're obligated by statute to incorporate the methods and the standards that we develop into our own grant review process. So, we're going to have to figure this out -- I mean, it wouldn't make any sense for PCORI to put out, these are the methods that need to be used under these circumstances, and then review grants over here and not apply that lens.

So, we've got to bring those activities together and it's going to be a non-trivial task. But if I can --

DR. EPSTEIN: Well, the "we" has got to be the Board and the --

DR. GABRIEL: Yes, all of us.

CHAIRMAN WASHINGTON: It's going to come to you. Steve. Christine has taken a pass.

VICE CHAIRMAN LIPSTEIN: Last comment is that I think you've heard from a number of Board Members that the use of case studies is very powerful. Under the overall heading of rigorous methods, it's really important that if we're going to use Ben or Mary that the methods recommendations you come forth with, tell us what we must know about Ben and Mary in order to do rigorous research on their outcomes. And that includes the use of surrogates, so we came up with, you know, Ben has a rural address or Mary has something else that you may be able to get from demographic data, but is that an appropriate surrogate for other attributes of Ben or Mary?

And so, I guess, what I would encourage you to do as you use these powerful case studies is to make sure that we define standards for what you have to know about Ben and Mary in order to do research, comparative research, on their clinical

or patient-centered outcomes.

CHAIRMAN WASHINGTON: That's a great point.

DR. GABRIEL: So, thank you for those comments. We've captured them all. Again, we'll invite you to participate in the call that moves this further, but just the one comment that I wanted to make is to thank my colleagues. I mean, everybody here talked about how powerful it is to use case studies like these, but the other side of the coin is that they could have given a little talk on heterogeneity of treatment effects or evidence synthesis in their sleep, and this effort, even though it's not complete, took hours and hours and hours and hours, and so thank you very much for that effort.

DR. EPSTEIN: Time well spent.

DR. GABRIEL: Yes. Time well spent. We do have one -- if you could just either give me the clicker or just move it forward -- we did want to share with you a brief update on what's happening with the definition. Moving forward, the

definition that I alluded to. Now, Dave Flum was going to call in since he leads this workgroup.

MR. FLUM: I'm on.

DR. GABRIEL: Are you on, Dave?

MR. FLUM: Yes, I'm on the line.

DR. GABRIEL: Terrific. Thank you. Sorry that we're running a little bit behind. So, we have the slide up and I'll turn it over to you.

MR. FLUM: Thanks, and I'll be brief. This is Dave Flum on the Methodology Committee. I'm sorry I can't be there in sunny Florida, I'm actually sitting in a blizzard in Seattle, which his ironic. We handle rain in many ways but not in the solid form, very well.

I'm representing a workgroup that involves many Methodology Committee members and Board Members as well. The creation of the definition of patient-centered outcomes research has been a very deliberative process aiming at really establishing clarity and precision in the terms, and also reflecting what exactly we mean when we are soliciting patient-centered outcomes research and

talking about what PCORI will be all about.

The process that occurred over the last seven months was both internal, carefully going through all the different options for how to frame the wording of patient-centered outcomes research, and then engage the public in several ways. The first was considered Phase 1 where the definition was posted on the website with lots of public feedback. It was synthesized by NORC over the course of the last few months. And then a follow up of six focus groups that NORC conducted. I believe you have work product from NORC on both phase one and phase two.

The focus groups were -- sought the input of the general public and specific patient groups, including those with chronic illness, caregivers, and other populations of patients, and they included both an assessment of what the definition that we had proposed was meaning to them, wording selections that we had specific questions about, and solicited feedback on better ways to communicate the intention of the definition.

Those themes that were derived from the focus groups have been evaluated by the working group members as was the public input, and the working group, to date, has met several times to consider both the public comment, to create a set of responses to the public commentary, and then to evaluate each of the themes that emerged from the focus groups, to identify areas where there's opportunity to change the definition and to modify the rationale for the definition based on those themes.

We've met several times in the last month and we will meet one or two more times with a voting process to determine whether or not revisions to the definition or the draft definition are being recommended. We hope to bring that back to you at the next formal meeting of the PCORI Board.

And that is really an update of the definition working group. Things are moving along quite nicely, we should have a final draft for your review by the next meeting and I'm open to

questions.

DR. GABRIEL: So, again, we're not bringing the recommendations today, just giving the Board an update of the process, and I do think, as was mentioned earlier, the processes relevant here might be a prototype for how we bring in comments from the public in a systematic way.

So, questions on that for David?

DR. CLANCY: I guess I would ask the Arnie question on this --

MR. HELFAND: Say who you are.

DR. CLANCY: I'm sorry, Carolyn Clancy. Thank you, Mark. Board Member. I would build on Arnie's previous questions about the case studies to say, will there be recommendations about what we could be doing differently? I mean, I've found reading the focus groups and the responses to public comment, which I still have right on my desk, highly informative, but the question is, sort of, how do we get past this? Not how do we get past it, but how do we get sort of smarter about it? How does that get folded into actually what

we do, and so forth?

DR. GABRIEL: And I'll let Dave comment, but the first step, obviously, is how we're using the input to change the definition, but you're saying, what can we learn from the process and how can we use those lessons to improve what we do going forward?

So, Dave, do you have any comments? I think that's as much a kind of piece of advice as a question.

MR. FLUM: Well, I think this also can bear some elaboration. I mean, I think the first thing we did was really, really listen. You know, that public input involved hundreds and hundreds of comments, often split on opposite ends of the same question. And so recognizing what is an opinion that's shared by many, versus an opinion that's split evenly is the first part, really listening carefully and creating alternative vehicles to engage voices, not just those who respond to a web posting, but also reaching out to the public through focus groups. I think it's an excellent

lesson in how to engage those voices.

I personally think engaging them earlier is always better, but also listening to the focus groups with the eye of offering meaningful revisions, or at least being able to explain more clearly the rationale as an opportunity to both listen and then to respond meaningfully.

And so, I think that this was a very deliberative process that intentionally engaged the public with a lot of transparency, which I think is a model for how we can move forward with other PCORI activities.

DR. GABRIEL: And I think, you know, just to sort of underscore Carolyn's comments, perhaps when the group comes back with recommendations on what ought to be changed in the definition based on what we learned from this process, we can also have kind of a lessons learned. Are there some things that might be generalizable to other PCORI activities? So, we'll do both of those things.

DR. CLANCY: Well, and also -- Carolyn Clancy again -- to a broader audience. I mean, I'm

not sure we need to keep relearning the same points. It would be nice to imagine that we only had to maybe relearn half.

CHAIRMAN WASHINGTON: [Off microphone.] -- because we have 5:30 and we have a group out -- if you could send a note otherwise. Sherine, any concluding --

DR. GABRIEL: Yeah, and just to share with you our next steps and the green spots are the interactions with the Board to get us to submitting the report. So, I won't go through those, I'll just have you take a look at that. And, thank you.

CHAIRMAN WASHINGTON: Okay. You should have this -- and, since I know Joe is out with our panelists, I'm going to ask that we send this as a separate specific attachment to Board Members --

DR. CLANCY: Yes.

CHAIRMAN WASHINGTON: -- just to remind us that these are critical points at which we can, should, in fact, the expectation is that we will be involved in terms of providing input.

DR. CLANCY: I assume you mean the whole

slide deck?

CHAIRMAN WASHINGTON: [Off microphone.] That's an important point too, but I want to make sure that everyone gets this particular --

DR. CLANCY: Okay, because I hadn't seen these before.

DR. GABRIEL: We can send the more detailed one that has specific dates and what not.

CHAIRMAN WASHINGTON: Okay, but would you send that as a separate one in addition to the deck. Okay.

DR. GABRIEL: I think Raneisha [phonetic] has that.

CHAIRMAN WASHINGTON: Sherine, is that it? Again, I didn't get to comment about the case reports because others echoed my sentiment. Thank you. I think it's terrific. My larger thought is, I hope that we will adopt the case report in other venues across PCORI. In fact, you know, we're going to tag PCORI case reports and use it in multiple different settings because that was very powerful what you just -- so, thank you very much.

[Applause.]

[Whereupon, at 5:35 p.m., the PCORI Board of Governors meeting was recessed, to reconvene at 8:00 a.m. on Thursday, January 19, 2012.]